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# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

# FORM 10-K

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

**For the Fiscal Year Ended December 31, 2022**

**or**

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

**Commission File Number: 001-31918**

# Aceragen, Inc.

**(Exact name of Registrant as specified in its charter)**

| **Delaware** | **04-3072298** |
|---|---|
| (State or other jurisdiction of incorporation or organization) | (I.R.S. Employer Identification No.) |
| **505 Eagleview Blvd., Suite 212**<br>**Exton, Pennsylvania**<br>(Address of principal executive offices) | **19341**<br>(Zip Code) |

**(484) 348-1600**

**(Registrant's telephone number, including area code)**

**Securities registered pursuant to Section 12(b) of the Act**

| Title of Each Class: | Trading Symbol | Name of Each Exchange on Which Registered |
|---|---|---|
| **Common Stock, $0.001 par value** | **ACGN** | **Nasdaq Capital Market** |

**Securities registered pursuant to Section 12(g) of the Act: None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Securities Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

| | | | |
|---|---|---|---|
| Large accelerated filer | ☐ | Accelerated filer | ☐ |
| Non-accelerated filer | ☒ | Smaller reporting company | ☒ |
| | | Emerging growth company | ☐ |

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act.) Yes ☐ No ☒

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant was $24.0 million based on the last sale price of the registrant's common stock as reported on the Nasdaq Capital Market on June 30, 2022 (the last business day of the registrant's most recently completed second fiscal quarter).

As of April 13, 2023, the registrant had 8,423,504 shares of common stock outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

None.

# ACERAGEN, INC.
# FORM 10-K

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Unless otherwise stated or the context requires otherwise, references in this Annual Report on Form 10-K to "Aceragen," the "company," the "Company," "we," "us," or "our" refer to Aceragen, Inc. (formerly known as Idera Pharmaceuticals, Inc.) and its subsidiaries, taken together. The Aceragen logo and other trademarks or service marks of the Company appearing in this Annual Report on Form 10-K are the property of Aceragen, Inc. All other brand names or trademarks are the property of their respective owners.

Website addresses referenced in this Annual Report on Form 10-K are provided for convenience only, and the content on the referenced websites does not constitute a part of, and are specifically not incorporated by reference into, this Annual Report on Form 10-K.

All share and per share amounts, including the exercise or conversion price of any of our securities, reflect, as applicable, the occurrence of a 1-for-17 reverse split of our common stock that occurred on January 17, 2023.

## NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K ("Form 10-K") and the documents we incorporate by reference contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements, other than statements of historical fact, included or incorporated in this report regarding, among other things, our cash resources and projected cash runway, financial position, our strategy, strategic alternatives, future operations, clinical trials (including, without limitation, the anticipated timing enrollment, and results thereof), collaborations, intellectual property, future revenues, projected costs, fundraising and/or financing plans, prospects, the ongoing impacts of the coronavirus ("COVID-19") pandemic, the benefits related to the Aceragen Acquisition (as defined below), the benefits related to the Reverse Stock Split (as defined below) and the plans and objectives of management are forward-looking statements. The words "believes," "anticipates," "estimates," "plans," "expects," "intends," "may," "could," "should," "potential," "likely," "projects," "continue," "will," "schedule," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We cannot guarantee that we will actually achieve the plans, intentions, or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. These forward-looking statements involve known and unknown risks, uncertainties, and other factors, which may be beyond our control, and which may cause the actual results, performance, or achievements of the Company to be materially different from future results, performance, or achievements expressed or implied by such forward-looking statements.

There are a number of important factors that could cause our actual results to differ materially from those indicated or implied by forward-looking statements. These important factors include those set forth below under Part I, Item 1A "Risk Factors" and in our other disclosures and filings with the Securities and Exchange Commission ("SEC"). These factors and the other cautionary statements made in this Form 10-K and the documents we incorporate by reference should be read as being applicable to all related forward-looking statements whenever they appear in this Form 10-K and the documents we incorporate by reference.

In addition, any forward-looking statements represent our estimates only as of the date that this Form 10-K is filed with the SEC and should not be relied upon as representing our estimates as of any subsequent date. All forward-looking statements included in this Form 10-K are made as of the date hereof and are expressly qualified in their entirety by this cautionary notice. We disclaim any intention or obligation to update or revise any forward-looking statement, whether as a result of new information, future events, or otherwise, except as may be required by law.

# PART I.

## Item 1. Business.

*Prior to January 17, 2023, we were known as Idera Pharmaceuticals, Inc. On September 28, 2022 we completed the Aceragen Acquisition, whereby we acquired all of the outstanding equity interests in Aceragen, Inc., a private company, as further described below. In connection with the Aceragen Acquisition and related transactions, we changed our name to Aceragen, Inc. Unless the context indicates otherwise, references in this section to the "Company," "Aceragen," "Idera," "we," "us," "our" and similar terms refer to Aceragen, Inc. (f/k/a Idera Pharmaceuticals, Inc.) and our consolidated subsidiaries. References to "Legacy Aceragen" refer to Aceragen, Inc. and its subsidiaries prior to the consummation of the Aceragen Acquisition.*

*All share and per share amounts, including the exercise or conversion price of any of our securities, reflect, as applicable, the occurrence of a 1-for-17 reverse split of our common stock that occurred on January 17, 2023.*

### Overview

We are a clinical-stage biopharmaceutical company with a business strategy focused on the clinical development, and ultimately the commercialization, of drug candidates for rare disease indications characterized by small, well-defined patient populations with serious unmet medical needs. Our current focus is to develop and optimize commercial value of ACG-701 (patented formulation of sodium fusidate) and ACG-801 (recombinant human acid ceramidase (rhAC)) for appropriate patients. We have in the past and may in the future explore collaborative alliances to support development and commercialization of any of our drug candidates. We may also seek to identify and potentially acquire rights to novel development and commercial-stage rare disease programs through new business development opportunities, including additional strategic alternatives.

As discussed in more detail below, in September 2022, we acquired Legacy Aceragen, a privately-held biotechnology company focused on addressing rare, orphan pulmonary, and rheumatic diseases for which there are limited or no available treatments. Legacy Aceragen owned or controlled the intellectual property related to ACG-701 (patented formulation of sodium fusidate) and ACG-801 (recombinant human acid ceramidase (rhAC)). Following the Aceragen Acquisition, our business strategy is to develop and optimize commercial value of ACG-701 and ACG-801 for appropriate patients. Accordingly, we are developing ACG-701 to treat cystic fibrosis ("CF") pulmonary exacerbations ("PEx") and melioidosis, a severe, life-threatening infection, and ACG-801 to treat patients suffering from a genetic mutation in the ASAH 1 gene, also known as Farber disease.

Until December 2021, we were developing tilsotolimod, via intratumoral injection, for the treatment of solid tumors in combination with nivolumab, an anti-PD1 antibody marketed as Opdivo® by Bristol Myers Squibb Company ("BMS"), and/or ipilimumab, an anti-CTLA4 antibody marketed as Yervoy® by BMS. Due to Phase 3 results in anti-PD-1 refractory advanced melanoma (ILLUMINATE-301), reported in March 2021, which showed the study failed to meet its primary endpoint, as well as a decision in December 2021 to discontinue enrollment in ILLUMINATE-206, our Phase 2 study in solid tumors, Company-sponsored development of tilsotolimod in oncology was discontinued. Although clinical trials with tilsotolimod have not yet translated into a new treatment alternative for patients, we believe that data supporting tilsotolimod's mechanism of action and encouraging safety profile from across the array of pre-clinical and clinical work to date, together with its intellectual property protection, are noteworthy.

### Business Acquisition

On September 28, 2022 ("Acquisition Date"), in accordance with the terms of an Agreement and Plan of Merger (the "Merger Agreement"), we acquired 100% of the outstanding security interests of Legacy Aceragen in a "stock-for-stock" transaction, whereby all Legacy Aceragen outstanding equity interests were exchanged for a combination of shares of our common stock, shares of Series Z preferred stock, par value $0.01 per share ("Series Z Preferred Stock"), and shares of the newly-designated Series X non-voting preferred stock, par value $0.01 per share ("Series X Preferred Stock") (such acquisition, the "Aceragen Acquisition"). Under the terms of the Merger Agreement, Legacy Aceragen stockholders received (i) 451,608 shares of our common stock (inclusive of shares subject to repurchase), (ii) 80,656 shares of Series Z Preferred Stock (inclusive of shares subject to repurchase), and (iii) five shares of Series X Preferred Stock. In addition, all outstanding options and warrants to purchase

Legacy Aceragen common stock were converted into stock options and warrants to purchase shares of our common stock and Series Z Preferred Stock on terms substantially identical to those in effect prior to the Aceragen Acquisition, except for adjustments to the underlying number of shares and the exercise price based on the Merger Agreement exchange ratio.

Pursuant to the Merger Agreement, we held a Special Meeting of Stockholders' on January 12, 2023 (the "Special Meeting") at which our stockholders approved, among other matters: (i) the conversion of Series Z Preferred Stock into shares of common stock in accordance with Nasdaq Listing Rule 5635(a) (the "Conversion Proposal") and (ii) a proposal to amend our Charter to effect a reverse stock split of all of the Company's issued and outstanding shares of common stock (the "Reverse Stock Split Proposal" and, together with the Conversion Proposal, the "Merger Agreement Meeting Proposals").

See "Item 7—Management's Discussion and Analysis of Financial Condition and Results of Operations" included elsewhere in this Form 10-K for further discussion of the Aceragen Acquisition and related transactions.

## Recent Developments

On April 13, 2023, the Board approved certain cost-cutting measures with a view to preserving capital to support our continuing operations. As part of this plan, we have commenced the furlough of 12 employees, representing approximately 46% of our workforce. Additionally, certain of our employees and executive officers will defer portions of their respective base salaries in amounts that exceed $200,000, with such deferrals having a retroactive effective date of April 5, 2023. We will continue to review operations for other opportunities to reduce costs and pursue financing opportunities. For more information, please see Item 9B of this Form 10-K.

## Clinical Development

### Our Pipeline

We are developing ACG-701 to treat CF PEx and melioidosis, and ACG-801 to treat Farber disease, each of which is discussed in greater detail below.

### ACG-701 *(sodium fusidate)*

***ACG-701 for Cystic Fibrosis Pulmonary Exacerbations***

ACG-701 is a proprietary oral, loading dose formulation of sodium fusidate being developed as a potential treatment for acute PEx associated with CF, a major factor driving lung function decline in people living with CF.

CF is a progressive, genetic disease hallmarked by inflammatory and infectious pulmonary exacerbations that are the primary cause of morbidity and mortality for CF patients. PEx are a sudden worsening of respiratory symptoms caused by lung infections that often require intravenous ("IV") antibiotic treatment. Current standard of care for acute PEx in CF is multifaceted, involving antibiotics, chest physiotherapy, inhaled medications to promote secretion clearance, and anti-inflammatory agents.

Sodium fusidate has an established clinical efficacy and safety profile from more than 50 years of use in other countries, including as part of CF PEx treatment guidelines in the United Kingdom and Australia. Despite this, the compound has never been approved by the U.S. Food and Drug Administration ("FDA") and represents a new and potentially powerful approach in the United States to address the infection, inflammation, and enhanced mucin expression that are hallmark features of CF PEx. According to the Cystic Fibrosis Foundation, in 2022, it was estimated that 105,000 patients have been diagnosed and were living with CF globally, with approximately 40,000 patients in the United States. If approved, ACG-701 would represent the first oral product in the United States indicated for the treatment of CF PEx.

In December 2022, we initiated the REPRIEVE study (NCT05641298), a Phase 2 randomized, double-blinded, placebo-controlled study evaluating ACG-701 for the treatment of CF PEx (the "REPRIEVE Study"), which is being funded in part by $3.5 million in support from the Cystic Fibrosis Foundation. The REPRIEVE Study, which is

expected to enroll 80 patients, will evaluate the efficacy and safety of ACG-701 plus optimized background therapy (OBT) compared to oral placebo plus OBT and capture multiple clinical events inclusive of patient-reported outcomes, FEV1, and antimicrobial regimen changes through day 14. The study opened for patient enrollment in March 2023 at clinical sites in the United States. Patients enrolled in the REPRIEVE Study will either be administered oral ACG-701 tablets twice a day for 14 days plus OBT or placebo tablets twice a day for 14 days plus OBT. The REPRIEVE Study will apply a desirability of outcome ranking (DOOR) analysis to the results. Data from the REPRIEVE Study is expected in the first half of 2024.

ACG-701 has received Orphan Drug designations for the treatment of CF patients from the FDA. In addition, we have also received Fast Track and Qualified Infectious Disease Product ("QIDP") designation for ACG-701 for the treatment of CF PEx. If approved, QIDP will provide an additional five-year extension of regulatory exclusivity to any exclusivities that the product may otherwise qualify.

***ACG-701 for Melioidosis***

ACG-701 is also being developed for the treatment of melioidosis, a life-threatening infection that can affect numerous organ systems, including the lungs. The pathogen that causes melioidosis, B. pseudomallei, is endemic in Southeast Asia and is classified as a Tier 1 biothreat agent by the U.S. government. The U.S. Department of Defense's Defense Threat Reduction Agency ("DTRA") is supporting the development of ACG-701 as a potential medical countermeasure against this pathogen and has awarded us funding of up to $49.7 million, of which $30.0 million remains available as of December 31, 2022.

In May 2022, we initiated the TERRA study (NCT05105035), a Phase 2 randomized, double-blind, placebo-controlled study evaluating ACG-701 for the treatment of melioidosis in hospitalized patients (the "TERRA Study"). The TERRA Study, which is actively recruiting and expected to enroll 125 patients, will evaluate the efficacy, safety and tolerability of ACG-701 given in combination with IV ceftazidime or IV meropenem and capture multiple clinical events inclusive of mortality, organ failure, sepsis, and treatment modifications through day 14. Patients enrolled in the study will be administered either IV ceftazidime or IV meropenem per standard of care and either placebo or ACG-701 every 12 hours for days 1-14. Day 1 dosing consists of two doses of 1500mg ACG-701 administered 12 hours apart, and day 2-14 dosing consists of 600mg of ACG-701 administered every 12 hours.

An independent data monitoring committee ("iDMC") is responsible for overseeing the safety and efficacy data. In February 2023, we announced that the iDMC for the TERRA Study met and recommended that the TERRA Study continue without modification. A data read-out from the TERRA Study is expected in the fourth quarter of 2023.

## ACG-801 *(acid ceramidase)*

***ACG-801 for Farber Disease***

ACG-801, recombinant human acid ceramidase, is an investigational biological enzyme replacement therapy being developed for the treatment of Farber disease. Farber disease is a severe, progressive monogenic lysosomal storage disorder, involving mutations in the acid ceramidase gene that lead to toxic levels of ceramide accumulation. Acid ceramidase acts in the lysosome to metabolize ceramide, a pro-inflammatory lipid. Loss of acid ceramidase function leads to abnormal accumulation of ceramide, causing macrophage-driven inflammation and multi-organ disease affecting bone, cartilage, the immune system, central nervous system, and the lungs. Patients with the most severe phenotype of Farber disease die early in life, most commonly from respiratory failure. As of December 31, 2022, we estimated that worldwide prevalence of Farber disease exceeded 1,000 patients. We are not aware of any competitive development programs seeking to treat Farber disease and there are no Farber disease-specific treatments currently approved by the FDA.

We are planning a single, harmonized trial for regulatory submission for both FDA and European Medicines Agency ("EMA") approval, known as the ADVANCE Study, which, as discussed in greater detail below, has been partially funded by NovaQuest (as defined below). A randomized, double-blind, placebo-controlled study of Farber disease patients, the ADVANCE study will measure nodule changes and capture patient-specific disease burden improvement through week 28. We have had regular interactions with the FDA, including a clinical-

focused Type C meeting in January 2023 to discuss trial design. Following resolution of the clinical hold placed on ACG-801 pertaining to manufacturing and quality issues, which we filed a response to in March 2023, we expect to initiate ADVANCE Study clinical activities in the first quarter of 2024.

The FDA has granted Orphan and Fast Track designations for ACG-801. The FDA has also designated ACG-801 as a product for rare pediatric diseases. If the ACG-801 marketing application is ultimately determined to meet the FDA's criteria to be a rare pediatric disease application, ACG-801 may be eligible for a Rare Pediatric Disease priority review voucher provided that the marketing application is approved before the program sunsets. Additionally, ACG-801 was granted Orphan Drug Designation by the EMA for Farber disease.

### Discontinued Programs

#### *Tilsotolimod (IMO-2125)*

Tilsotolimod is a synthetic phosphorothioate oligonucleotide that acts as a direct agonist of TLR9 to stimulate the innate and adaptive immune systems. It was developed for administration via intratumoral injection in combination with systemically administered checkpoint inhibitors and costimulation therapies for the treatment of various solid tumors. We referred to our tilsotolimod development program as the ILLUMINATE development program.

In the first quarter of 2018, we initiated a Phase 3 trial of intratumoral tilsotolimod in combination with ipilimumab in patients with anti-PD-1 refractory melanoma, which we referred to as ILLUMINATE-301. This trial compared the results of the tilsotolimod–ipilimumab combination to those of ipilimumab alone in a 1:1 randomization. The family of primary endpoints of the trial consisted of Objective Response Rate ("ORR") by blinded independent central review RECIST v1.1 and median Overall Survival ("OS"). In March 2021, we reported that ILLUMINATE-301 did not meet its primary endpoint of ORR. In May 2021, following evaluation of the full data set, we announced we would not continue ILLUMINATE-301 to its OS primary endpoint.

In September 2019, we initiated a Phase 2, open-label, global, multicohort study to evaluate the safety and effectiveness of tilsotolimod administered intratumorally in combination with nivolumab and ipilimumab for the treatment of solid tumors, which we referred to as ILLUMINATE-206. The first solid tumor investigated under ILLUMINATE-206 was relapsed/refractory Microsatellite-Stable Colorectal Cancer ("MSS-CRC") in immunotherapy-naïve patients (the "MSS-CRC Study"). In December 2021, based on an analysis of preliminary data from the safety cohort of ILLUMINATE-206, we announced that further enrollment in the trial had been discontinued. The decision to discontinue ILLUMINATE-206 was not related to safety concerns.

Following the announcement that all Company-sponsored development of tilsotolimod was discontinued in December 2021, all study-related activities concluded.

## Collaborative Alliances and Clinical Funding Arrangements

Our current collaborative alliances and clinical funding arrangements include agreements with the Cystic Fibrosis Foundation, DTRA (as defined below), and Scriptr Global, Inc. ("Scriptr"), each discussed further below. We may seek to enter into new funding arrangements or collaborative alliances to support development and commercialization of additional drug candidates.

#### *Cystic Fibrosis Foundation*

On December 13, 2021, Legacy Aceragen entered into a therapeutic development and award agreement (the "CFF Agreement") with The Cystic Fibrosis Foundation ("CFF"), pursuant to which CFF agreed to fund the REPRIEVE Study, as described above under the heading "Clinical Development," in an amount up to $3.5 million (the "CFF Award"), of which $1.0 million had been received as of December 31, 2022. In consideration for the CFF Award and CFF's license of CFF Know-How, Legacy Aceragen agreed to pay CFF royalties of up to $21.0 million as follows: (a) one-time royalty equal to 3x the CFF Award if a product resulting from the REPRIEVE Study is approved for commercial sale; (b) an amount equal to 1x the CFF Award in the event of a license transaction or change in control, as defined in the CFF Agreement; and (c) a one-time royalty equal to 1x the CFF

Award upon (i) attainment of reaching net sales of $250 million and (ii) upon attainment of reaching net sales of $500 million.

***Department of Defense's Defense Threat Reduction Agency ("DTRA")***

In connection with the Aceragen Acquisition, we assumed development contracts awarded to Legacy Aceragen in June 2020 and August 2021 which provide for funding of up to $49.7 million in the aggregate by the Department of Defense's DTRA for the clinical and regulatory development of ACG-701 as a potential medical countermeasure against the pathogen that causes melioidosis, B. Pseudomallei, as further discussed above. As of December 31, 2022, $19.7 million had been used and $30.0 million remains available for use through December 2026.

***Collaboration with Scriptr***

In February 2021, we entered into a collaboration and option agreement with Scriptr, pursuant to which (i) Scriptr and Idera will conduct a research collaboration utilizing Scriptr Platform Technology ("SPT") to identify, research, and develop gene therapy candidates (each, a "Collaboration Candidate") for the treatment, palliation, diagnosis, or prevention of (a) myotonic dystrophy type 1 ("DM1 Field") and (b) Friedreich's Ataxia ("FA Field") on a Research Program-by-Research Program basis, as applicable, and (ii) we were granted an exclusive option, in our sole discretion, to make effective the Scriptr License Agreement (as defined below) for a given Research Program (as defined below) to make use of Collaboration Candidates and related intellectual property (collectively, the "Scriptr Agreement").

Pursuant to the Scriptr Agreement, Scriptr will use commercially reasonable efforts to carry out research activities set forth in accordance with the applicable DM1 Field and FA Field research plans, including certain pre-clinical proof of concept studies, to identify research Collaboration Candidates utilizing SPT (each, a "Research Program"). Following the completion of activities under a given Research Program, Scriptr will prepare and submit to us a comprehensive data package (each, a "Data Package") that summarizes, on a Research Program-by-Research Program basis, any Collaboration Candidates researched under the Research Program, including any data and results. Upon receipt of a Data Package, we have, in our sole discretion, up to two-hundred seventy (270) calendar days to make effective the exclusive license agreement entered into by and between Scriptr and us, pursuant to which, among other things, Scriptr grants us exclusive rights and licenses with respect to the development, manufacture and commercialization of licensed candidates and products, subject to certain conditions and limitations (the "Scriptr License Agreement"), for a given Research Program (each licensed Research Program, a "Licensed Program"). The Scriptr License Agreement provides for customary development milestones on candidates developed under a Licensed Program and royalties on licensed products, if any.

In partial consideration of the rights granted by Scriptr to us under the Scriptr Agreement, we made a one-time, non-creditable, and non-refundable payment to Scriptr during the first quarter of 2021. In order to fund the Research Programs, we reimburse Scriptr for costs incurred by or on behalf of Scriptr in connection with the conduct of each Research Program during the research term in accordance with the applicable Research Program budget and payment schedule. We incurred $0.5 million and $2.1 million in research and development expenses under the Scriptr Agreement during the years ended December 31, 2022 and 2021, respectively.

## Academic and Research Collaborations

We have entered into research collaborations with scientists at leading academic research institutions. These research collaborations allow us to augment our internal research capabilities and obtain access to specialized knowledge and expertise. In general, our research collaborations may require us to supply compounds and pay various amounts to support the research. Under these research agreements, if a collaborator, solely or jointly with us, creates any invention, we may own exclusively such invention, have an automatic paid-up, royalty-free, non-exclusive license, or have an option to negotiate an exclusive, worldwide, royalty-bearing license to such invention. Inventions developed solely by our scientists in connection with research collaborations are owned exclusively by us. These collaborative agreements are non-exclusive and may be terminated with limited notice.

## Other Material Contracts

***NovaQuest Agreements***

*NovaQuest Side Letter*

At closing of the Aceragen Acquisition, we entered into a side letter agreement (the "NovaQuest Side Letter") with NovaQuest Co-Investment Fund XV, L.P. ("NovaQuest"), pursuant to which: (i) NovaQuest was granted customary observer rights to have a managing director or investment professional of NovaQuest Capital Management, L.L.C. attend all meetings of our board of directors and all meetings of the Audit Committee, Compensation Committee, and Nominating and Corporate Governance Committee thereof in a non-voting capacity until such time as NovaQuest no longer beneficially owns shares of Series X Preferred Stock (as defined below); (ii) we agreed to grant NovaQuest customary registration rights in the event we in the future grant registration rights to any investor or equity holder with respect to shares of our capital stock; and (iii) we acknowledged and agreed that pursuant to the terms of the Series X Preferred Stock Certificate of Designation, we shall not authorize or issue shares of its capital stock unless the same ranks junior to the Series X Preferred Stock, or increase the authorized number of shares of Series X Preferred Stock or any additional class of capital stock unless the same ranks junior to the Series X Preferred Stock, in each case until such time as NovaQuest no longer beneficially owns shares of Series X Preferred Stock.

*NovaQuest Purchase Agreement (Related to ACG-801)*

Pursuant to the Stock and Warrant Purchase Agreement, dated as of March 24, 2021, by and between Legacy Aceragen and NovaQuest, as amended by that Amendment, dated October 25, 2021, and as such agreement may be amended from time to time (the "Purchase Agreement"), NovaQuest agreed to provide up to $35.0 million in product-based financing to support the development of ACG-801 for Farber disease. The financing was to be provided through (i) $15.0 million in proceeds from the sale of Legacy Aceragen capital stock and warrants to purchase shares of Legacy Aceragen capital stock, and (ii) up to $20.0 million in capital contributions for development funding relating to the treatment of Farber disease ("Capital Contributions"). The Capital Contributions were to be paid by NovaQuest in quarterly installments for Legacy Aceragen's eligible expenses associated with the development of ACG-801 for Farber disease ("ACG-801 Product"). Prior to the Aceragen Acquisition, Legacy Aceragen received $20.0 million in Capital Contributions, representing the total eligible Capital Contributions provided for under the Purchase Agreement.

NovaQuest, holders of Series X Preferred Stock (as defined below), are entitled to receive distributions on shares of Series X Preferred Stock as set forth in (a) the Purchase Agreement, and (b) that certain Sales Distribution and PRV Agreement, dated as of October 25, 2021, by and between Legacy Aceragen and NovaQuest, as such agreement may be amended from time to time (the "PRV Agreement"), prior and in preference to any declaration or payment of any other distribution or dividend (other than dividends on shares of Common Stock payable in shares of Common Stock).

Pursuant to the terms and conditions of the Purchase Agreement, we must make a distribution to NovaQuest of 35% of the net proceeds we receive in connection with a transaction (a "PRV Sale Transaction"), pursuant to which we sell any of our right, title, and interest in and to a priority review voucher ("PRV") granted by the FDA in connection with regulatory approval of ACG-801 Product. Further, in the event that we do not receive a PRV in connection with regulatory approval of ACG-801 Product by the FDA, or do not complete a PRV Sale Transaction within 12 months after our receipt of a PRV, then we shall make a distribution to NovaQuest equal to $35 million in two equal installments, the first of which shall be effected within 45 days after the date we receive regulatory approval of ACG-801 Product from the FDA (the "U.S. Approval Date") and the second of which shall be effected within one year after the U.S. Approval Date.

In addition, pursuant to the terms and conditions of the Purchase Agreement, after first commercial sale of ACG-801 Product and continuing for each fiscal quarter until the Distribution End Date (as defined therein), we shall make a distribution to NovaQuest (the "Required Net Sales Distribution") equal to the product of the Required Net Sales Distribution Rate (as defined below) multiplied by the aggregate total of the net sales of ACG-801 Product for such fiscal quarter. The Required Net Sales Distribution Rate shall initially be 15%; provided that once the aggregate of all distributions paid to NovaQuest equals $140 million, the Required Net Sales

Distribution Rate shall decrease to 5%. The Required Net Sales Distribution shall end after the date on which the last of the following has occurred: (i) we pay $140 million to NovaQuest; (ii) the last valid patent covering ACG-801 Product in the United States and European Union has expired; and (iii) regulatory exclusivity for ACG-801 Product in both the United States and the European Union has expired.

Pursuant to the terms and conditions of the Purchase Agreement, we must use commercially reasonable efforts to (i) develop ACG-801 in a manner that ensures that Aceragen is reasonably likely to obtain regulatory approval from the FDA for Farber disease no later than September 1, 2024 and to obtain regulatory approval from the European Medicines Agency (EMA) no later than October 1, 2024; (ii) identify a third-party purchaser of a PRV, if we receive a PRV, and consummate a PRV Sales Transaction (as defined therein) within 12 months of receipt of the PRV; and (iii) commercialize ACG-801 Product in each jurisdiction in which regulatory approval is received.

*NovaQuest PRV Agreement (Related to the Arrevus Acquisition)*

Pursuant to the terms and conditions of the PRV Agreement related to Legacy Aceragen's acquisition of Arrevus, Inc., we must make a distribution to NovaQuest of 10% of the net proceeds we receive in connection with a PRV Sales Transaction pursuant to which we sell any of our right, title, and interest in and to a PRV that we receive in connection with regulatory approval of ACG-701. Further, in the event that we do not receive a PRV in connection with regulatory approval of ACG-701 by the FDA or do not complete a PRV Sales Transaction within 12 months after our receipt of such PRV, then we must make certain satisfaction milestone payments in favor of NovaQuest.

Pursuant to the terms and conditions of the PRV Agreement, we must use commercially reasonable efforts to (i) develop ACG-701 in a manner that ensures that we are reasonably likely to obtain regulatory approval from the FDA for ACG-701 by no later than December 31, 2024, (ii) if we receive a PRV in connection with regulatory approval of ACG-701, identify a third-party purchaser of such PRV and consummate a PRV Sales Transaction within 12 months of receipt of such PRV, and (iii) commercialize ACG-701 in each jurisdiction for which regulatory approval is received. After first commercial sale of ACG-701 and continuing for each fiscal quarter until the Distribution End Date (as defined therein), we shall make the Required Net Sales Distribution equal to product of 5% multiplied by the aggregate total of net sales of ACG-701 for such fiscal quarter. The Required Net Sales Distribution shall end after we pay $50 million to NovaQuest.

## Customers

The U.S. Government accounted for all of our revenues for the year ended December 31, 2022.

## Intellectual Property

Our success depends in part on our ability to obtain and maintain proprietary protection for our product candidates, technology and know-how, to operate without infringing the proprietary rights of others and to prevent others from infringing our proprietary rights. We use a variety of methods to seek to protect our proprietary position, including filing U.S. and foreign patent applications related to our proprietary technology, inventions and improvements that are important to the development of our business. We also rely on trade secrets, know-how, continuing technological innovation, and in-licensing opportunities to develop and maintain our proprietary position. We also intend to rely on regulatory exclusivities, when available, to protect the market for any of our product candidates that receive regulatory approval.

Our intellectual property is critical to our business and we strive to protect it, including by seeking to obtain and maintain patent protection in the United States and internationally to cover our product candidates, their methods of use, and processes for their manufacture and any other inventions that are commercially important to the development of our business. Our patent portfolio includes patent and patent applications that we own or exclusively license with composition of matter and method of use claims with respect to our product candidates, ACG-701 and ACG-801. Our policy is to pursue, maintain and defend patent rights whether developed internally or licensed from third parties and to protect the technology, inventions and improvements that are commercially important to the development of our business.

Our patent portfolios for our ACG-701 ad ACG-801 candidates are summarized below.

***ACG-801***

We exclusively license from Mt. Sinai 4 patent families that claim certain compositions, method of making and methods of use with respect to acid ceramidase and ACG-801. One of the patent families includes granted patents in the United States, Europe, Japan, Europe, Israel, Mexico, and Hong-Kong, and pending applications in Brazil, Canada, Europe, Hong-Kong, Japan, Israel, Mexico, Saudi Arabia, and United States. The granted and pending claims in this patent family are directed to certain compositions of acid ceramidase and methods of producing the compositions. The 20-year term for patents in this family runs through 2034, absent any available patent term adjustments or extension. A second patent family currently includes pending patent applications in the United States, Australia, Canada, Europe, Hong-Kong, Japan, Mexico, Jordan, and New Zealand that claim certain methods of treating Farber Disease using acid ceramidase and ACG-801. A third patent family currently includes granted patents in the United States, Europe, Hong-Kong, and Japan, and pending applications in the United States, Canada, China, Europe, Hong-Kong, and Japan. The granted and pending claims in this family are directed to certain methods of treating infections in patients with cystic fibrosis, chronic obstructive pulmonary disease, or an open wound. The 20-year term for patents in this family runs through 2033, absent any available patent term adjustments or extension. A fourth patent family currently includes granted patents in the United States and Europe, and pending applications in each of these jurisdictions with claims directed to certain methods of using acid ceramidase to produce chondrocytes. The 20-year term for patents in this family runs through 2032, absent any available patent term adjustments or extensions.

We own 3 patent families directed to certain therapeutic uses of acid ceramidase and ACG-801, including for treating Farber Disease. One of the patent families includes pending applications in United States, Australia, Brazil, Canada, Columbia, China, Europe, Hong Kong, Israel, Japan, South Korea, Mexico, Russia, New Zealand, Philippines, and Thailand that claim certain methods of treating Farber Disease using acid ceramidase and ACG-801. The 20-year term for patents in this family runs through 2039, absent any available patent term adjustments or extensions. A second patent family currently includes pending patent applications in United States, Australia, Brazil, Canada, China, Columbia, Europe, Hong Kong, Israel, Japan, South Korea, Mexico, Russia, South Africa, New Zealand, and Philippines that claim certain biomarkers of and methods of assessing Farber Disease. The 20-year term for patents in this family runs through 2039, absent any available patent term adjustments or extensions. A third patent family includes pending patent applications in United States and Argentina with claims directed to treating certain inflammatory disorders associated with Farber Disease. The 20-year term for patents in this family runs through 2041.

***ACG-701***

We own 2 patent families directed to ACG-701 and certain therapeutic uses thereof. One patent family includes patents issued in the U.S. and Canada that claim certain therapeutic uses of fusidic acid for treating bacterial infections. The 20-year term for patents in this family runs through 2030, absent any availably patent term adjustments or extensions. A second patent family includes a patent issued in Japan and a pending application in the U.S. that claims compositions of fusidic acid. The 20-year term for patents in this family runs through 2032, absent any available patent term adjustments.

The status of our patent portfolios changes frequently in the ordinary course of our business. As of March 13, 2023, we owned or exclusively licensed approximately 10 U.S. patents and patent applications and about 111 patents and patent applications throughout the rest of the world that relate to our ACG-701 ad ACG-801 candidates.

***TLR-targeted immune modulation technologies***

Prior to the Aceragen Acquisition, we devoted a substantial amount of our resources into establishing intellectual property protection for:

- novel chemical entities that function as agonists of TLR3, TLR7, TLR8 or TLR9;
- novel chemical entities that function as antagonists of TLR7, TLR8 or TLR9; and
- composition and use of our nucleic acid chemistry compounds to treat and prevent a variety of diseases.

As of March 15, 2023, we owned approximately 44 U.S. patents and patent applications and about 177 patents and patent applications throughout the rest of the world for our TLR-targeted immune modulation technologies. These patents and patent applications include claims covering the chemical compositions of matter and methods of use of our IMO compounds, such as IMO-8400, IMO-9200 and tilsotolimod (IMO-2125), as well as other compounds. These patents and patent applications (if granted) expire at various dates ranging from 2023 to 2041. With respect to IMO-8400, we have ten issued U.S. patents that cover the chemical composition of matter of IMO-8400 and certain methods of its use, the latest of which expires in 2031. With respect to IMO-9200, we also have ten issued U.S. patents that cover the chemical composition for IMO-9200 and methods of its use, the latest of which expires in 2034. With respect to tilsotolimod, we have issued U.S. patents that cover the chemical composition of matter of tilsotolimod that will expire between 2023 and 2026, and we have additional U.S. patents that cover methods of its use, the latest of which will expire in 2037 assuming payment of all maintenance fees.

Because patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind actual discoveries, neither we nor our licensors can be certain that we or they were the first to make the inventions claimed in issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications.

Litigation may be necessary to defend against or assert claims of infringement, to enforce patents issued to us, to protect trade secrets or know-how owned by us, or to determine the scope and validity of the proprietary rights of others or to determine the appropriate term for an issued patent. In addition, USPTO may declare interference proceedings to determine the priority of inventions with respect to our patent applications or reexamination or reissue proceedings to determine if the scope of a patent should be narrowed. Litigation or any of these other proceedings could result in substantial costs to and diversion of effort by us, even if the eventual outcome is favorable to us, and could have a material adverse effect on our business, financial condition and results of operations. These efforts by us may not be successful.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent. A patent's term may also be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a patent that covers a drug, biological product, or medical device approved pursuant to a pre-market approval may also be eligible for patent term extension when U.S. Food and Drug Administration ("FDA") approval is granted, provided statutory and regulatory requirements are met. The length of the patent term extension is related to the length of time the drug is under regulatory review and development. The Drug Price Competition and Patent Term Restoration Act of 1984 (the "Hatch-Waxman Act") permits a patent term extension of up to five years beyond the expiration date set for the patent. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be granted an extension and only those claims reading on the approved drug are extended. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug.

We may rely, in some circumstances, on trade secrets and confidentiality agreements to protect our technology. Although trade secrets are difficult to protect, wherever possible, we use confidentiality agreements to protect the proprietary nature of our technology. We regularly implement confidentiality agreements with our employees, consultants, scientific advisors, and other contractors and collaborators. However, there can be no assurance that these agreements will not be breached, that we will have adequate remedies for any breach, or that our trade secrets and/or proprietary information will not otherwise become known or be independently discovered by

competitors. To the extent that our employees, consultants or contractors use intellectual property owned by others in their work for us, disputes may also arise as to the rights in related or resulting know-how and inventions.

In addition to patents, we rely upon unpatented trade secrets, know-how and continuing technological innovation to develop and maintain a competitive position. We seek to protect our proprietary information, in part, through confidentiality agreements with our employees, collaborators, contractors and consultants, and invention assignment agreements with our employees and some of our collaborators. The confidentiality agreements are designed to protect our proprietary information and, in the case of agreements or clauses requiring invention assignment, to grant us ownership of technologies that are developed through a relationship with a third party.

## Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We are currently developing ACG-701, a patented formulation of sodium fusidate, to treat CF PEx and melioidosis, and ACG-801, recombinant human acid ceramidase (rhAC), to treat patients suffering from Farber disease. We recognize that other companies, including large pharmaceutical companies, may be developing or have plans to develop products and technologies that may compete with our drug candidates, ACG-701 and ACG-801, or any other drug candidates we may develop or acquire in the future. Our products, if approved, will compete with novel therapies developed by biopharmaceutical companies, academic research institutions, governmental agencies, and public and private research institutions, in addition to standard of care treatments. Our competitors compete with us on the level of the technologies employed, or on the level of development of product candidates. In addition, many small biotechnology companies have formed collaborations with large, established companies to obtain support for their research, development, and commercialization of products that may potentially directly compete with our current or future product candidates. We anticipate that we will continue to face increasing competition as new therapies and combinations thereof, technologies, and data emerge for rare diseases which we are pursuing.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical, and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments, and the commercialization of those treatments. Accordingly, our competitors may be more successful than us in obtaining approval for treatments and achieving widespread market acceptance.

ACG-701 is currently being developed to treat patient suffering from CF PEx and melioidosis. We are aware of several companies that may compete with ACG-701 for the treatment of melioidosis, including, AN2 Therapeutics, Inc., Bugworks Research, Inc., Entasis Therapeutics, Inc., Soligenix, Inc., and Venatorx Pharmaceuticals, Inc. While there are currently no FDA-approved drugs specifically for the treatment of melioidosis, there are several antimicrobial drugs used off-label for the treatment of melioidosis, such as beta-lactams (e.g., ceftazidime, certain beta-lactam-beta-lactamase inhibitor combinations), carbapenems, trimethoprim-sulfamethoxazole (TMP-SMX), and doxycycline, depending on the phase of treatment, with which our drug candidate may compete. While there are FDA-approved products for the treatment of CF and several companies developing therapies to address the underlying cause of CF, there are currently no FDA-approved drugs specifically for the treatment of CF PEx and, to the best of our knowledge, no company’s programs are currently aimed directly at addressing CF PEx. However, there may also be unexpected or unknown competitors that we are not presently aware of.

ACG-801 is being initially developed for the treatment of patients suffering from Farber disease, for which there is currently no approved therapies. While we are aware of several companies developing enzyme replacement therapies for other lysosomal storage diseases, to the best of our knowledge, no company’s programs are currently aimed directly at addressing Farber disease. However, there may be unexpected or unknown competitors of which we are not presently aware.

Our commercial potential for our current drug candidates, ACG-701 and ACG-801, or any other drug candidates or technologies we may acquire, could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than products that we may develop. Our competitors also may obtain FDA, EMA, or other regulatory approval for their products more rapidly than we may obtain approval for our products, which could result in our competitors establishing a strong market position before we are able to enter the market or make our development

more complicated. The key competitive factors affecting the success of all of our programs are likely to be efficacy, safety, and convenience.

These competitors may also vie for a similar pool of qualified scientific and management talent, sites, and patient populations for clinical trials, as well as for technologies complementary to, or necessary for, our products. Further, our competitive position will also depend upon our ability to obtain patent protection or otherwise develop proprietary products or processes, protect our intellectual property, and to secure sufficient capital resources for the period between technological conception and commercial sales.

## Manufacturing

We do not currently own or operate, and currently have no plans to establish, any manufacturing facilities for the production of preclinical, clinical, or commercial quantities of ACG-701 or ACG-801, or any other product candidate. We currently rely and expect to continue to rely on third-party contract development and manufacturing organizations ("CDMOs") for all of our requirements of raw materials, drug substance, and drug product. We are not currently party to any long-term agreements with our current CDMOs. We intend to rely on CDMOs for clinical and commercial manufacturing of ACG-701 and ACG-801, as well as the clinical and commercial manufacturing of our other product candidates and any other product candidates that we may identify in the future. Although we rely on CDMOs, we have employees and third-party consultants with extensive manufacturing experience to oversee the relationships with our contract manufacturers.

## Government Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing and sales, pricing, post-approval monitoring and reporting, and import and export of pharmaceutical products. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources. Regulatory requirements are also continually evolving. By example, the FDA and the EU regulatory authorities have also issued a growing body of guidance documents that provide the agency's interpretation of regulatory requirements.

***Review and Approval of Drugs and Biologics in the United States***

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act ("FDCA") and associated implementing regulations and guidance. In the United States, biologics are regulated under both the FDCA and the Public Health Services Act ("PHSA"). The failure to comply with the FDCA and other applicable U.S. requirements at any time during the product development process, approval process or after approval may subject an applicant and/or sponsor to a variety of administrative or judicial sanctions, including refusal by the FDA to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and other types of enforcement letters, product recalls, product seizures, operating restrictions such as the total or partial suspension of production or distribution, import bans, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, or civil or criminal investigations and penalties brought by the FDA and the Department of Justice ("DOJ") or other governmental entities, including state agencies.

An applicant seeking approval to market and distribute a new drug or biologic product in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice ("GLP") regulations and other applicable laws or regulations;
- submission to the FDA of an investigational new drug ("IND") application, which must take effect before human clinical trials may begin in the United States;
- initial and continuing approval by an independent institutional review board ("IRB"), representing each clinical site before each clinical trial may be initiated;

- performance of adequate and well-controlled human clinical trials in accordance with good clinical practices ("GCP") to establish the safety and efficacy of the proposed product for each indication;
- preparation and submission to the FDA of a new drug application ("NDA") for drug products or biologic license application ("BLA") for biologic products;
- review of the product candidate by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of one or more FDA inspections or remote regulatory assessments of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data;
- payment of user fees and securing FDA approval of the marketing application; and
- compliance with any post-approval requirements, including Risk Evaluation and Mitigation Strategies ("REMS") where applicable, and post-approval studies required by the FDA.

***Preclinical Studies and Submission of an IND***

Before an applicant begins testing a product candidate in humans, the product candidate enters the preclinical testing stage. Preclinical studies include laboratory evaluation of the chemistry, pharmacology, toxicity, purity, and stability, among other attributes, of the manufactured drug or biologic substance or active pharmaceutical ingredient and the formulated product, as well as in vitro and animal studies to assess the safety and activity of the drug or biologic for initial testing in humans and to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations. The results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical studies, among other things, are submitted to the FDA as part of an IND. Additional preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted.

***Human Clinical Studies in Support of a Marketing Application***

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Special clinical trial ethical considerations also must be taken into account if a study involves children. Clinical trials are conducted under written study protocols detailing, among other things, the objectives of the study, inclusion and exclusion criteria, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated, and a statistical analysis plan.

A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. Under newly enacted legislation, diversity plans will also be required. Once an IND is in effect, unapproved product candidates may be shipped in interstate commerce for use in an investigational clinical trial and the investigational product may be administered to humans as part of a clinical trial. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to a proposed clinical trial and places the trial on clinical hold or partial clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Clinical holds also may be imposed by the FDA at any time before or during trials due to safety concerns or non-compliance. Depending on the conditions under development, multiple INDs may be required for the same product.

In addition to the foregoing IND requirements, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review and reapprove the study at least annually. The IRB must review and approve, among other things, the study protocol, protocol amendments, communications to study subjects and informed consent information to be provided to study subjects. An IRB must operate in compliance with FDA regulations. Information

about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health ("NIH") for public dissemination on their ClinicalTrials.gov website. Sponsors or distributors of investigational products for the diagnosis, monitoring, or treatment of one or more serious disease or conditions must also have a publicly available policy on evaluating and responding to requests for expanded access. Investigators must also provide certain information to clinical trial sponsors to allow the sponsors to make certain financial disclosures to the FDA.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data monitoring committee ("DMC"). This group provides recommendations as to whether a trial should move forward at designated check points based on access that only the group maintains to available data from the study. Suspension or termination of development during any phase of clinical trials can occur if it is determined that the participants or patients are being exposed to an unacceptable health risk. Other reasons for suspension or termination may be made by us based on evolving business objectives and/or competitive climate.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

***Phase 1****:* The product is initially introduced into healthy human subjects or patients with the target disease (e.g. cancer) or condition and tested for safety, dosage tolerance, structure-activity relationships, mechanism of action, absorption, metabolism, distribution, excretion, and pharmacokinetics and, if possible, to gain an early indication of its effectiveness and to determine optimal dosage.

***Phase 2****:* The product is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

***Phase 3****:* The product is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

In some cases, the FDA may condition approval of an NDA or BLA on the applicant's agreement to conduct additional clinical trials to further assess the product's safety and effectiveness after NDA or BLA approval. Such post-approval trials are typically referred to as Phase 4 studies. The results of Phase 4 studies can confirm or refute the effectiveness of a product candidate, and can provide important safety information.

The manufacture of investigational products for the conduct of human clinical trials is subject to cGMP requirements. Investigational products and active ingredients imported into the United States are also subject to regulation by the FDA. Further, the export of investigational products outside the United States is subject to regulatory requirements of the receiving country as well as U.S. export requirements under the FDCA.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and to the IRB and more frequently if serious adverse events occur. In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or in vitro testing that suggest a significant risk in humans exposed to the product; and any clinically important increase in the case of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA, the sponsor or the data monitoring committee for a clinical trial may suspend or terminate the clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product has been associated with unexpected serious harm to patients. The FDA will typically inspect or conduct a remote regulatory assessment of one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

***Section 505(b)(2) NDAs***

NDAs for most new drug products are based on clinical studies conducted by or for the product sponsor. These applications are submitted under Section 505(b)(1) of the FDCA. The FDA is, however, authorized to approve an

alternative type of NDA under Section 505(b)(2) of the FDCA. This type of application allows the applicant to rely, in part, on the FDA's previous findings of safety and efficacy for a similar product or published literature. Specifically, Section 505(b)(2) applies to NDAs for a drug for which the investigations made to show whether or not the drug is safe for use and effective in use and relied upon by the applicant for approval of the application "were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted."

Thus, Section 505(b)(2) authorizes the FDA to approve an NDA based on safety and effectiveness data that was not developed by the applicant. NDAs filed under Section 505(b)(2) may provide an alternate and potentially more expeditious pathway to FDA approval for new or improved formulations or new uses of previously approved products. If the Section 505(b)(2) applicant can establish that reliance on the FDA's previous approval or a similar product or that the applicant's reliance on published data is scientifically appropriate, such as through bridging studies, the applicant may eliminate the need to conduct certain preclinical or clinical studies of the new product. Companies using this pathway, however, must conduct studies to support any differences from an approved product. The FDA may then approve the new drug candidate for all or some of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

***Submission of a Marketing Application to the FDA***

Assuming successful completion of required clinical testing and other requirements, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA, for drug products, or BLA for biologic products, requesting approval to market the product for one or more indications. Under federal law, the submission of most NDAs and BLAs is subject to a substantial application user fee. The sponsor of an approved NDA or BLA is also subject to an annual program fee. Depending on the product and fee, a company may qualify for fee exceptions or waivers.

The FDA conducts a preliminary review of an NDA or BLA within 60 calendar days of its receipt and strives to inform the sponsor by the 74$^{th}$ day after the FDA's receipt of the submission of any filing review issues. The FDA may request additional information rather than accept an application for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review.

The FDA has agreed to specified performance goals under the Prescription Drug User Fee Act ("PDUFA") guidelines in the review process of NDAs and BLAs. Under PDUFA, 90% of applications seeking approval of New Molecular Entities ("NMEs"), are meant to be reviewed within ten months from the date on which the FDA accepts the NDA or BLA for filing, and 90% of applications for NMEs that have been designated for "priority review" are meant to be reviewed within six months of the filing date. For applications seeking approval of products that are not NMEs, the ten-month and six-month review periods run from the date that the FDA receives the application. The review process may be extended by the FDA for three additional months to consider new information or clarification provided by the applicant to address an outstanding deficiency identified by the FDA following the original submission.

Before approving a marketing application, the FDA typically will inspect or conduct a remote regulatory assessment of the facility or facilities where the product is or will be manufactured. These pre-approval inspections cover all facilities associated with a marketing application submission, including component manufacturing (such as active pharmaceutical ingredients), finished drug product manufacturing, and control testing laboratories. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA or BLA,, the FDA will typically inspect or conduct a remote regulatory assessment of one or more clinical sites to assure compliance with GCP.

In addition, as a condition of approval, the FDA may require an applicant to develop a REMS. REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events, and whether the product is a new molecular entity. REMS can

include medication guides, physician communication plans for healthcare professionals, and elements to assure safe use ("ETASU"). ETASU may include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The FDA may require a REMS before approval or post-approval if it becomes aware of a serious risk associated with use of the product. The requirement for a REMS can materially affect the potential market and profitability of a product. The REMS strategy must be approved by the FDA. In addition, the REMS must include a timetable to assess the strategy at 18 months, three years, and seven years after the strategy's approval.

The FDA is required to refer an application for a novel drug to an advisory committee or explain why such referral was not made. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

***Fast Track, Breakthrough Therapy and Priority Review Designations***

The FDA is authorized to designate certain products for expedited review or increased regulatory guidance if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. These programs are fast track designation, breakthrough therapy designation and priority review designation.

Specifically, the FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a fast track product's marketing application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the marketing application is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

In 2012, Congress enacted the Food and Drug Administration Safety and Innovation Act ("FDASIA"). This law established a new regulatory scheme allowing for expedited review of products designated as "breakthrough therapies." A product may be designated as a breakthrough therapy if it is intended, either alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking other steps to design the clinical trials in an efficient manner. Like fast track designation, breakthrough designation may be rescinded if the product no longer meets the qualifying criteria.

Third, the FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA determines, on a case-by-case basis, whether the proposed product represents a significant improvement when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting drug reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, and evidence of safety and effectiveness in a new subpopulation. A priority designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from ten months to six months.

***Accelerated Approval Pathway***

The FDA may grant accelerated approval to a drug or biologic for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality ("IMM"), and that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Products granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a product, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a product.

The accelerated approval pathway is most often used in settings in which the course of a disease is long, and an extended period of time is required to measure the intended clinical benefit of a product, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used extensively in the development and approval of products for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the product's clinical benefit. As a result, a product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. By the date of approval of an accelerated approval product, FDA must specify the conditions for the required post approval studies, including enrollment targets, the study protocol, milestones, and target completion dates. The FDA may also require that the confirmatory Phase 4 studies be commenced prior to the FDA granting a product accelerate approval. Reports on the progress of the required Phase 4 confirmatory studies must be submitted to the FDA every 180 days after approval. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the product from the market on an expedited basis through a statutorily defined streamlined process. All promotional materials for product candidates approved under accelerated regulations are subject to prior review by the FDA.

***The FDA's Decision on Marketing Application***

On the basis of the FDA's evaluation of the NDA or BLA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the marketing application, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

If the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess the product's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including

distribution restrictions or other risk management mechanisms, including REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

***Post-Approval Requirements***

Products manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences and production issues with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data. Many states also regulate the distribution of drug product samples and commercial product.

In addition, product manufacturers and other entities involved in the manufacture and distribution of approved drugs and biologics are required to register their establishments with the FDA and state agencies, are required to list their distributed products, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. The information that must be submitted to FDA regarding manufactured products was expanded through the Coronavirus Aid, Relief, and Economic Security Act ("CARES Act") to include the volume of products produced during the prior year. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, suspension of the approval, complete withdrawal of the product from the market or product recalls;
- warning letters, untitled letters, cyber letters, or holds or termination of post-approval clinical trials;
- refusal of the FDA to approve pending marketing applications or supplements to approved marketing applications, or suspension or revocation of product license approvals;
- product seizure or detention, product recalls, or refusal to permit the import or export of products; or
- FDA debarment, injunctions, fines, consent decrees, corporate integrity agreements, suspension and debarment from government contracts, refusal of orders under existing government contracts, exclusion from participation in federal and state healthcare programs, restitution, disgorgement, or the imposition of civil or criminal penalties, including fines and imprisonment, and adverse publicity.

Sponsors are further subject to various requirements related to FDA product shortage and manufacturing volume reporting, supply chain security, such as risk management plan requirements, and the promotion of supply chain redundancy. FDA post-approval requirements are further continually evolving. For example, in March 2020, the U.S. Congress passed the CARES Act, which includes various provisions regarding FDA product shortage and manufacturing volume reporting requirements, as well as provisions regarding supply chain security, such as risk management plan requirements, and the promotion of supply chain redundancy and domestic manufacturing. The executive branch has also taken steps to promote domestic manufacturing, and the Consolidated Appropriations

Act of 2023, and the reauthorization of the Prescription Drug User Fee Act, which were passed in 2022, included several changes to the FDC Act.

The FDA strictly regulates the marketing, labeling, advertising and promotion of prescription products placed on the market. This regulation includes, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the Internet and social media. Promotional claims about a drug's safety or effectiveness are prohibited before the product is approved. After approval, a product may not be promoted for uses that are not approved by the FDA, as reflected in the product's prescribing information. All statements must be consistent with the FDA-approved label, truthful, and non-misleading. Additionally, claims about product benefits and risks must be substantiated adequately while maintaining a fair balance between the two. For instance, it is impermissible to make claims about the superiority of our products over other treatments without sufficient evidence, which often requires head-to-head clinical studies for substantiation. In the United States, health care professionals are generally permitted to prescribe products for such uses not described in the product's labeling, known as off-label uses, because the FDA does not regulate the practice of medicine. However, FDA regulations impose rigorous restrictions on manufacturers' communications, prohibiting the promotion of off-label uses. It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information.

If a company is found to have promoted off-label uses, it may become subject to adverse public relations and administrative and judicial enforcement by the FDA, the DOJ, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes products. The federal government has levied large civil penalties and criminal fines against companies for alleged improper promotion and has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act ("PDMA"), and its implementing regulations, as well as the Drug Supply Chain Security Act ("DSCSA"). The PDMA regulate and limit the distribution and tracing of prescription drug samples at the federal level. The DSCSA imposes requirements to ensure accountability in distribution, which requires certain licensing and licensing standards, and to identify and remove counterfeit and other illegitimate products from the market.

***Additional controls for biologics***

In addition to the above requirements, biologic products are subject to additional controls and requirements. Biologic products are subject, not only to drug cGMPs, but also to a special set of manufacturing controls that are intended to ensure product quality. Due to risks involved with biologic products, FDA can immediately suspend licenses in situations where there is a danger to public health. FDA can also prepare or procure biologic products in the event of shortages and critical public health needs. After a BLA is approved, the product may also be subject to official lot release as a condition of approval, meaning that the manufacturer must submit samples of each lot of product to the FDA together with a release protocol showing the results of all of the manufacturer's tests. Manufacturers subject to lot release may not release a biologic product for distribution until authorized by FDA. The FDA may also perform certain confirmatory tests on lots of some products before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

***Abbreviated New Drug Applications for Generic Drugs***

In 1984, with passage of the Hatch-Waxman Amendments to the FDCA, Congress authorized the FDA to approve generic drugs under abbreviated approval requirements. To obtain approval of a generic drug, an applicant must submit an Abbreviated New Drug Application ("ANDA") to the agency. In support of such applications, a generic manufacturer may rely on the preclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the Reference Listed Drug ("RLD").

Specifically, in order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, conditions of use, and the strength of the drug, among other requirements. Certain differences, however, between the reference listed drug and ANDA product may be permitted pursuant to a suitability petition. Certain labeling differences may also be permitted if information in the reference listed drug's label is protected by patent or exclusivities. At the same time, the FDA must also determine that the generic drug is "bioequivalent" to the innovator drug. Under the statute, a generic drug is bioequivalent to a RLD if "the rate and extent of absorption of the drug do not show a significant difference from the rate and extent of absorption of the listed drug."

Upon approval of an ANDA, the FDA indicates whether the generic product is "therapeutically equivalent" to the RLD in its publication "Approved Drug Products with Therapeutic Equivalence Evaluations," also referred to as the "Orange Book." Physicians and pharmacists consider a therapeutic equivalent generic drug to be fully substitutable for the RLD, subject to state law requirements. In addition, by operation of certain state laws and numerous health insurance programs, the FDA's designation of therapeutic equivalence often results in automatic substitution of the generic drug at the pharmacy.

***Hatch-Waxman Exclusivities***

Under the Hatch-Waxman Amendments, the FDA may not approve an ANDA or 505(b)(2) application until any applicable period of non-patent exclusivity for the RLD has expired. The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity. For the purposes of this provision, a new chemical entity ("NCE"), is a drug that contains no active moiety that has previously been approved by the FDA in any other NDA. An active moiety is the molecule or ion, excluding those appended portions of the molecule that cause the drug to be an ester, salt, or other noncovalent derivative, responsible for the physiological or pharmacological action of the drug substance. In cases where such exclusivity has been granted, an ANDA or 505(b)(2) application may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification, in which case the applicant may submit its application four years following the original product approval. The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period prevents FDA from making a drug approval effective for the same changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication, as the product that holds the exclusivity. The three-year and five-year exclusivities, however, do not prevent the filing or approval of full NDAs.

***Hatch-Waxman Patent Certification and the 30-Month Stay***

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or an approved method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA or 505(b)(2) applicant files its application with the FDA, the applicant is required to provide a certification to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval.

Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;
- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or
- the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicates that it is not seeking approval of a patented method of use, the application will not be approved until all the listed patents claiming the referenced product have expired (other than method of use

patents involving indications for which the ANDA applicant is not seeking approval). The applicant may also elect to submit a “Section VIII” statement certifying that its proposed label does not contain (or carves out) any language regarding the patented method-of-use rather than certifying to a listed method-of-use patent.

If the ANDA or 505(b)(2) applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders within certain specified timeframes. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA or 505(b)(2) application until the earlier of 30 months after the receipt of the Paragraph IV notice, expiration of the patent, or a decision in the infringement case that is favorable to the ANDA applicant.

***Biologic Exclusivity and Biosimilars***

Under the Biologic Product Competition and Innovation Act (BPCIA), a product sponsor may apply to FDA for approval of a biologic product that is “biosimilar to” or “interchangeable with” a previously approved biological product. For the FDA to approve a biosimilar product, it must find that there is a high degree of similarity to the reference product, notwithstanding minor differences in clinically inactive components, and that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. Biosimilarity must be shown through analytical studies, animal studies, and at least one clinical trial, absent a waiver by the FDA. There must be no difference between the reference product and a biosimilar in mechanism of action, conditions of use, route of administration, dosage form, and strength. For the FDA to approve a biosimilar product as interchangeable with a reference product, the FDA must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

If a biosimilar application is submitted to FDA, the biosimilar product sponsor and reference product sponsor may exchange certain patent and product information for the purpose of determining whether there should be a legal patent challenge. Based on the outcome of negotiations surrounding the exchanged information, the reference product sponsor may bring a patent infringement suit and injunction proceedings against the biosimilar product sponsor. The biosimilar applicant may also be able to bring an action for declaratory judgment concerning the patent.

Like the Hatch Waxman Act for small molecule drugs, the BPCIA provides for certain periods of regulatory exclusivity for biologic products. Specifically, a biosimilar product application may not be submitted to the FDA until four years following the date of approval of the reference product and the FDA may not approve a biosimilar product until 12 years from the date of approval of the reference product. Not all biologic products, however, will qualify for exclusivity. For instance, certain changes and supplements to an approved BLA, and subsequent applications filed by the same sponsor, manufacturer, licensor, predecessor in interest, or other related entity do not qualify for the 12 year exclusivity period. Biologic product exclusivity also does not block FDA approval of a full BLA for a competing product.

The FDA maintains a publicly-available online database of licensed biological products, which is commonly referred to as the “Purple Book.” The Purple Book lists product names, dates of licensure, and applicable periods of exclusivity. Further, the reference product sponsor must provide patent information and patent expiry dates to FDA following the exchange of patent information between biosimilar and reference product sponsors. This information is then published in the Purple Book.

***FDA Efforts to Increase Competition***

In an effort to increase competition in the drug and biologic product marketplace, Congress, the executive branch, and FDA have taken certain legislative and regulatory steps. For example, measures have been proposed and implemented to facilitate product importation. Moreover, the 2020 Further Consolidated Appropriations Act included provisions requiring that sponsors of approved drug products, including those subject to REMS, provide samples of the approved products to persons developing 505(b)(2) NDA or ANDA drug products within specified timeframes, in sufficient quantities, and on commercially reasonable market-based terms. Failure to do so can

subject the approved product sponsor to civil actions, penalties, and responsibility for attorney's fees and costs of the civil action. This same bill also includes provisions with respect to shared and separate REMS programs for reference and generic drug products.

***Pediatric Studies, Exclusivity, and Vouchers***

Under the Pediatric Research Equity Act ("PREA") of 2003, an NDA, BLA, or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. With enactment of the FDASIA in 2012, sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in the FFDCA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity and on listed patents. This six-month exclusivity may be granted if a sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the applicable time limits, whatever statutory or regulatory periods of exclusivity or orange book listed patents that cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application.

As a further incentive to develop products for pediatric diseases, the FDCA established a program under which the FDA awards priority review vouchers to sponsors of rare pediatric disease products that meet certain criteria. The rare disease must be serious or life-threatening in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. The product must contain no active ingredient (including any ester or salt of the active ingredient) that has been previously approved in any other application and the application must meet certain additional qualifying criteria, including eligibility for FDA priority review. If FDA determines that a product is for a rare pediatric disease and the qualifying application criteria are met, FDA may award a priority review voucher. This voucher may be redeemed to receive priority review of a subsequent marketing application for a different product. Use of a priority review voucher is subject to an FDA user fee. These vouchers are also transferable and may be bought and sold. Vouchers may also be revoked by FDA under certain circumstances and sponsors of approved rare pediatric disease products must submit certain reports to FDA. Changes to the FDCA, however, have limited future pediatric priority review vouchers. Under the law's sunset provision, the drug or biologic must be designated by FDA for a rare pediatric disease no later than September 30, 2024, and approved no later than September 30, 2026, unless the law is reauthorized by Congress.

***Orphan Drug Designation and Exclusivity***

Under the Orphan Drug Act, the FDA may designate a drug or biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a product available in the United States for treatment of the disease or condition will be recovered from sales of the product in the United States). Additionally, sponsors must present a plausible hypothesis for clinical superiority to obtain orphan designation if there is a product already approved by the FDA that that is considered by the FDA to be the same as the already approved product and is intended for the same indication. This hypothesis must be demonstrated to obtain orphan exclusivity. A company must request orphan product designation before submitting

a marketing application. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process, although it does convey certain advantages such as tax benefits and exemption from Prescription Drug User Fee Act fees.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will receive orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances. Competitors may receive approval of different products for the indication for which the orphan product has exclusivity and may obtain approval for the same product but for a different indication. If a product designated as an orphan product ultimately receives marketing approval for an indication broader than what was designated in its orphan product application, it may not be entitled to exclusivity.

Orphan drug exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product is deemed to be the same as the product with exclusivity and for the same condition is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand.

Presently, the exact scope of orphan drug exclusivity is currently in flux. In 2021, the scope of orphan product exclusivity for the drug Firdapse was the subject of a legal challenge and reversal in the Catalyst Pharms., Inc. v. Becerra judicial decision. The court ruled that the orphan drug exclusivity for Firdapse prevented another company's application for the same drug from being approved for the entire disease or condition for which orphan drug designation was granted, despite the fact that the approved product indication was narrower. This decision differed from the FDA's interpretation of the FDCA, which held that orphan drug exclusivity only protected a product's approved indication. In January 2023, the FDA published a notice in the Federal Register indicating that it interprets the Catalyst Pharms. decision narrowly and plans to continue limiting the scope of orphan drug exclusivity to the uses or indications for which a drug is approved. The scope of orphan drug exclusivity will likely be an evolving area.

***Qualified Infectious Disease Products***

For certain infectious disease products, the FDCA provides further development incentives through additional periods of regulatory exclusivity. Specifically, qualified infectious disease products (QIDPs) are eligible for the extension of existing periods of regulatory exclusivities by an additional five years. This exclusivity extension is subject to certain statutory limits. A QIDP is an antibacterial or antifungal drug for human use intended to treat serious or life-threatening infections, including those caused by an antibacterial or antifungal resistant pathogen, including novel or emerging infectious pathogens; or qualifying pathogens designated by the FDA. Sponsors must request, any time before submitting an NDA, that the FDA designate the product as a QIDP. QIDP designation may be withdrawn if FDA finds that the request for designation contained an untrue statement of material fact.

***Patent Term Restoration and Extension***

A patent claiming a new drug or biologic product may be eligible for a limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, which permits a patent restoration of up to five years for patent term lost during product development and the FDA regulatory review. The restoration period granted is typically one-half the time between the effective date of an IND and the submission date of a marketing application, plus the time between the submission date of a marketing application and the ultimate approval date. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date. Only one patent applicable to an approved product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent in question. A patent that covers multiple drugs or biologics for which approval is sought can only be extended in connection with one of the approvals. The USPTO reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

***Review and Approval of Drug Products in the European Union***

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of drug products. Whether or not it obtains FDA approval for a product, the company would need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. The approval process ultimately varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

***Clinical Trial Approval in the EU***

Pursuant to the previous European Clinical Trials Directive, a system for the approval of clinical trials in the European Union ("EU") has been implemented through national legislation of the member states. Under this system, an applicant obtained approval from the competent national authority of a EU member state in which the clinical trial was conducted. In April 2014, the EU adopted a new Clinical Trials Regulation (which came fully into force from January 2022), which will be directly applicable to and binding without the need for any national implementing legislation. Under the new coordinated procedure for the approval of clinical trials, the sponsor of a multi-state clinical trial will be required to submit a single application for approval of a clinical trial to a reporting EU Member State (RMS) through an EU Portal. The submission procedure will be the same irrespective of whether the clinical trial is to be conducted in a single EU member state or in more than one EU member state. The Clinical Trials Regulation also aims to streamline and simplify the rules on safety reporting for clinical trials. The applicant may only start a clinical trial after a competent ethics committee has issued a favorable opinion. Clinical trial applications must be accompanied by an investigational medicinal product dossier with supporting information prescribed by the applicable EU legislation and corresponding national laws of the member states and further detailed in applicable guidance documents.

***Orphan Drug Designation and Exclusivity in the European Union***

Regulation 141/2000 provides that a drug shall be designated as an orphan drug if its sponsor can establish: that the product is intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the EU when the application is made, or that the product is intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating, or serious and chronic condition in the EU and that without incentives it is unlikely that the marketing of the drug in the EU would generate sufficient return to justify the necessary investment. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention, or treatment of the condition in question that has been authorized in the EU or, if such method exists, the drug will be of significant benefit to those affected by that condition.

Regulation 847/2000 sets out criteria and procedures governing designation of orphan drugs in the EU. Specifically, an application for designation as an orphan product can be made any time prior to the filing of an application for approval to market the product. Marketing authorization for an orphan drug leads to a ten-year period of market exclusivity. This period may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation, for example because the product is sufficiently profitable not to justify market exclusivity. Market exclusivity can be revoked only in very selected cases, such as consent from the marketing authorization holder, inability to supply sufficient quantities of the product, demonstration of "clinically relevant superiority" by a similar medicinal product, or, after a review by the Committee for Orphan Medicinal Products, requested by a member state in the fifth year of the marketing exclusivity period (if the designation criteria are believed to no longer apply). Medicinal products designated as orphan drugs pursuant to Regulation 141/2000 shall be eligible for other incentives made available by the EU and by the EU member states to support research into, and the development and availability of, orphan drugs.

***Pharmaceutical Coverage, Pricing and Reimbursement***

Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Sales of products will depend, in part, on the extent to which the costs of the products will be covered by third-party payors, including government healthcare programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors are increasingly challenging the prices charged, examining the medical necessity, reviewing the cost-effectiveness of medical products and services, and imposing controls to manage costs. Our rebate payments may increase, or our prices be adjusted under value-based purchasing arrangements based on evidence-based measures or outcomes-based measures for a patient or beneficiary based on use of our drug. Third-party payors may also limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication or may impose other market access or utilization management controls.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable regulatory approvals. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Third-party reimbursement may not be sufficient to maintain price levels high enough to realize an appropriate return on investment in product development.

The containment of healthcare costs also has become a priority of federal, state and foreign governments and the prices of drugs have been a focus in this effort. Governments have shown significant interest in implementing cost-containment programs, including price controls, increases in rebates paid, restrictions on reimbursement, and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit a company's revenue generated from the sale of any approved products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which a company or its collaborators receive marketing approval, less favorable coverage policies and reimbursement rates may be implemented in the future. In the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that drug products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular drug candidate to currently available therapies, or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, the EU provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU member states may approve a specific price for a drug product or may instead adopt a system of direct or indirect controls on the profitability of the company placing the drug product on the market. Other EU member states allow companies to fix their own prices for drug products but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations for drug products may not allow favorable reimbursement and pricing arrangements.

***Healthcare Law and Regulation***

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations. Such restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;

- the federal False Claims Act imposes civil penalties, and provides for civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim, or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government, with potential substantial liability including mandatory treble damages at trial and significant per-claim penalties;

- the Affordable Care Act included a provision requiring certain providers and suppliers of items and services to federal healthcare programs to report and return overpayments within sixty days after they are "identified" (the "Overpayment Statute"), after which the recipient of the overpayment incurs federal civil False Claims Act liability;

- the Affordable Care Act authorized the imposition of civil monetary penalties on manufactures participating in the 340B program for failure to charge the statutory ceiling price, and required HHS to promulgate regulations establishing the standards for implementing this Civil Monetary Penalty, or CMP, authority. CMS' final CMP rule went into effect January 1, 2019;

- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and their respective implementing regulations, including the Final Omnibus Rule published in January 2013, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information and sanctions for failing to meet those obligations;

- HIPAA imposes specific privacy rules with respect to disclosure of protected health information from covered entities for research purposes, such as clinical trials;

- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;

- the federal transparency requirements under the Health Care Reform Law, known as the federal Physician Payments Sunshine Act, require manufacturers of covered drugs, devices, biologics and medical supplies to report to the Centers for Medicare & Medicaid Services ("CMS") within the Department of Health and Human Services information related to certain payments and other transfers of value to US-licensed physicians, physician assistants, nurse practitioners, certified registered nurse anesthetists and certified nurse midwifes and US teaching hospitals and to physician ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

***Healthcare Reform in the United States***

A primary trend in the United States healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last several years regarding the pricing of pharmaceutical and

biopharmaceutical products, limiting coverage and reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the Patient Protection and Affordable Care Act (the "PPACA") which, among other things, includes changes to the coverage and payment for products under government health care programs. Moreover, in 2017, the U.S. Congress modified and amended certain provisions of the PPACA, which could have an impact on coverage and reimbursement for healthcare items and services covered by the federal and state healthcare programs as well as plans in the private health insurance market. The so-called "individual mandate" was repealed as part of tax reform legislation adopted in December 2017. Legal challenges to the PPACA may continue to arise and there may continue to be future efforts to modify, repeal, or otherwise invalidate all, or certain provisions of the Affordable Care Act. The Biden administration is expected to continue to take measures to further facilitate the implementation of the PPACA. Among the provisions of the PPACA of importance to potential drug candidates are:

- an annual, nondeductible fee on any entity that manufactures, or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- expanded manufacturers' rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate for both branded and generic drugs and revising the definition of "average manufacturer price" ("AMP") for calculating and reporting Medicaid drug rebates on outpatient prescription drug prices and extending rebate liability to prescriptions for individuals enrolled in Medicare Advantage plans;
- addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expanded the types of entities eligible for the 340B drug discount program;
- established the Medicare Part D coverage gap discount program by requiring manufacturers to provide a point-of-sale-discount off the negotiated price of applicable brand drugs to eligible beneficiaries during their coverage gap period as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;
- established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- established the Independent Payment Advisory Board ("IPAB") which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs, subsequently repealed through the Bipartisan Budget Act of 2018; and
- established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Other legislative changes have been proposed and adopted since the PPACA was enacted. These changes include the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year that started in 2013 , were paused from May 1, 2020 through December 31, 2021, and will continue through 2031 unless additional Congressional action is taken, and the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and

otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

More recently, Congress amended the Medicaid statute, effective October 1, 2019, to exclude prices paid by secondary manufacturers for an authorized generic drug (but not a product approved under the BLA process) from the NDA holder's AMP for the brand, thereby increasing the rebate amount and the 340B price for the brand. This was implemented by CMS in a final rule issued December 31, 2020. The rule also expanded the definition of products identified as "line extensions" and, in certain circumstances, required inclusion of patient copay assistance in Medicaid best price (effective January 1, 2023), thereby potentially increasing Medicaid rebates paid by manufacturers for such drugs. 340B program guidance regulations on civil monetary penalties for statutory violations, which had been finalized in early 2017 but deferred, also recently went into effect. On November 27, 2020, CMS issued an interim final rule implementing a Most Favored Nation payment model under which reimbursement for certain Medicare Part B drugs and biologicals will be based on a price that reflects the lowest per capita Gross Domestic Product-adjusted (GDP-adjusted) price of any non-U.S. member country of the Organization for Economic Co-operation and Development (OECD) with a GDP per capita that is at least sixty percent of the U.S. GDP per capita. This rule now has been rescinded, but similar programs have been described in recent legislative proposals.

These healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, payment of increased rebates, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price for any approved product and/or the level of reimbursement physicians receive for administering any approved product. Reductions in reimbursement levels may negatively impact the prices or the frequency with which products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. Since enactment of the PPACA, there have been numerous legal challenges and congressional actions to repeal and replace provisions of the law.

Further, there have been several recent U.S. congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, Congress and the recent administrations have indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing.

## Human Capital Resources

Our vision is to translate scientific breakthroughs into important new medicine. We have a culture where patients are at the center of all we do, with core values that connect us to each other and our stakeholders and define who we are, what we stand for, and how we work.

At March 31, 2023, our total workforce consisted of 26 full-time employees, of whom 17 are engaged in research and development. However, on April 13, 2023, the Board approved certain cost-cutting measures with a view to preserving capital to support our continuing operations. As part of this plan, we have commenced the furlough of 12 employees, representing approximately 46% of our workforce. For more information, see Item 9B of this Form 10-K. None of our employees are represented by labor unions or covered by collective bargaining agreements, and we consider our relationship with employees to be good. We also utilize the services of several independent consultants to support our research and development and general and administrative operations.

We are focused on effective identification, recruitment, development, and retention of, and compensation and benefits to, human resource talent, including workforce and management development, diversity, equity, and inclusion initiatives, succession planning, and corporate culture and leadership quality, which are vital to our success. Attracting, developing and retaining talented employees to support the growth of our business is an integral part of our human capital strategy and critical to our long-term success. The principal purposes of our incentive plans are to attract, retain and motivate selected employees, consultants and directors. Accordingly, these individuals are eligible to receive stock-based compensation awards and cash-based performance bonus awards. Additionally, we offer a package of competitive employee-specific benefits, including 401(k) plan matching contributions.

## Corporate Information

We were incorporated in Delaware in 1989 and our office headquarters is located at 505 Eagleview Boulevard, Suite 212, Exton, Pennsylvania 19341. Our internet address is www.aceragen.com. The information contained on, or that can be accessed through, our website is not part of this Form 10-K. We have included our website in this Form 10-K solely as an inactive textual reference.

This Form 10-K contains references to our trademarks and trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this Form 10-K, including logos, artwork, and other visual displays, may appear without the ® or TM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other company.

## Available Information

We make available free of charge through our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to these reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after we electronically file or furnish such materials to the SEC. The SEC maintains an internet site at www.sec.gov containing reports, proxies and information statements and other information regarding issuers that file electronically with the SEC.

## Item 1A. Risk Factors.

*Investing in our securities involves a high degree of risk. You should carefully consider the risks and uncertainties described below in addition to the other information included or incorporated by reference in this Form 10-K before purchasing our common stock. Our business, financial condition and results of operations could be materially and adversely affected by any of these and currently unknown risks or uncertainties. In that case, the market price of our common stock could decline, and you may lose all or part of your investment in our securities.*

**Summary of Principal Risk Factors**

- We will need to raise additional capital, and if we are unable to do so, we will not be able to continue as a going concern.
- Adverse developments affecting financial institutions, companies in the financial services industry or the financial services industry generally, such as actual events or concerns involving liquidity, defaults or non-performance, could adversely affect our operations and liquidity.
- We expect that we will continue to incur net losses in the foreseeable future.
- Our stock price has been and may continue to be volatile, and the value of an investment in our common stock may decline.
- We may not be able to comply with Nasdaq's continued listing standards.
- Our recent organizational changes, including the Aceragen Acquisition, and any acquisitions or business development opportunities we pursue may not be successful.
- As a small biopharmaceutical-focused company with limited resources, we may be unable to attract and retain qualified personnel.
- We depend on our senior management team, and the loss of one or more of our executive officers or key employees or an inability to attract and retain highly skilled employees could compromise our ability to pursue our growth strategy and grow our business.
- We are depending heavily on the development, regulatory approval, and commercialization of product candidates. If we are unable to successfully develop and commercialize product candidates, or experience significant delays in doing so, our business may be materially harmed.
- If our clinical trials are unsuccessful, delayed or terminated, we may not be able to develop and commercialize our product candidates.
- The technologies on which we rely are unproven and may not result in any approved and marketable products.
- We face substantial competition, which may result in others discovering, developing, or commercializing products before or more successfully than us.
- Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming, and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates.
- We may not be able to obtain or maintain anticipated periods of regulatory exclusivity for our product candidates.
- A breakthrough therapy, fast track, or other expedited designation for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that those product candidates will receive marketing approval.
- We have only limited experience in regulatory affairs and our product candidates are based on new technologies; these factors may affect our ability or the time we require to obtain necessary regulatory approvals.
- We are subject to extensive and costly governmental regulation, the violation of which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, and diminished profits and future earnings.
- We depend on information technology, infrastructure, and data to conduct our business. Any significant disruption, or cyber-attacks, could have a material adverse effect on our business.
- Climate change, environmental, social and governance and sustainability initiatives may result in regulatory or structural industry changes that could require significant operational changes and expenditures, reduce demand for the Company's products and adversely affect our business.

- Our existing collaborations, clinical funding arrangements, and any collaborations or arrangements we enter into in the future may not be successful.
- If we are unable to establish additional collaborative alliances, our business may be materially harmed.
- We are subject to substantial customer concentration. If we fail to retain our revenues from the U.S. Government consistent with historical performance or acquire new customers cost-effectively, our business could be adversely affected.
- If we are unable to obtain and maintain patent protection for our discoveries, the value of our technology and any product candidates could be adversely affected.
- We do not own or license patents or patent applications that claim the active pharmaceutical ingredient in our ACG-701 and ACG-801 product candidates.
- Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Our rights to develop and commercialize ACG-801 and future product candidates may be subject to terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under our current or future license agreements, we could lose important intellectual property rights and the ability to continue to develop and commercialize products.
- We may not be successful in obtaining all rights necessary to develop and commercialize our current or future product candidates.
- We may become involved in expensive patent litigation or other proceedings, which could result in our incurring substantial costs and expenses or substantial liability for damages, require us to stop our development and commercialization efforts or result in our patents being invalidated, interpreted narrowly or limited.
- Our intellectual property may be infringed by a third party.
- If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.
- We may be subject to claims by third parties asserting that we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.
- We rely on government funding for certain aspects of our research and development activities and we may develop intellectual property through such activities and therefore may be subject to federal regulations. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.
- Even if the compounds we may develop are successful in clinical trials and receive regulatory approvals, we or our collaboration partners may not be able to successfully commercialize them.
- Because we have limited manufacturing experience, and no manufacturing facilities or infrastructure, we are dependent on third-party manufacturers to manufacture product candidates for us.
- We have no experience selling, marketing or distributing potential products and no internal capability to do so.
- If third parties on whom we rely for clinical and preclinical trials do not perform as contractually required or as we expect, we may not be able to obtain regulatory approval for or commercialize our drug candidates and our business may suffer.
- We face a risk of product liability claims and may not be able to obtain insurance.
- Provisions in our restated certificate of incorporation (“Charter”) and second amended and restated bylaws (“Bylaws”) may prevent a change in control that stockholders may consider desirable.
- Six stockholders beneficially own approximately 69% of our outstanding common stock. If these significant stockholders choose to act together, they could exert substantial influence over our business, and the interests of these stockholders may conflict with those of other stockholders.
- The issuance or sale of shares of our common stock could depress the trading price of our common stock.
- Because we do not intend to pay dividends on our common stock, investor returns will be limited to any increase in the value of our stock.
- Our Series X Preferred Stock have rights, preferences and privileges that are not held by, and are preferential to, the rights of our common stock, which could adversely affect our liquidity and financial condition, and may result in the interests of Series X Preferred Stock holders differing from those of the holders of common stock.

## Risks Relating to Our Financial Position and Need for Additional Capital

***Despite recent cost-saving measures, we will need to raise additional capital, and if we are unable to do so, we will not be able to continue as a going concern.***

As of December 31, 2022, we had $12.0 million in cash and cash equivalents. We believe based on our current operating plan, our existing cash and cash equivalents will enable us to fund our operations into May 2023. Accordingly, we need to raise additional capital to continue to fund our operations and service our obligations in the future. If we are unable to raise additional capital when needed, we will not be able to continue as a going concern. We do not currently have any products approved for sale and do not generate any revenue from product sales. Accordingly, we expect to rely primarily on equity and/or debt financings and our clinical funding arrangements (e.g., contract with Department of Defense's DTRA) to fund our continued operations.

We are seeking and expect to continue to seek additional funding through financings of equity and/or debt securities, strategic research and development collaborations, clinical funding arrangements, or the sale or license of technology assets. We may also engage in strategic alternative conversations of mergers, acquisition, or sale of the company or assets. We believe the key factors that will affect our ability to obtain funding are: (i) the results of our clinical development activities in our product candidates we develop on the timelines anticipated; (ii) the time and expense required to submit a marketing application for our product candidates; (iii) the cost, timing, and outcome of regulatory reviews; (iv) the receptivity of the capital markets to financings by biotechnology companies generally and companies with product candidates and technologies similar to ours specifically; (v) receptivity of the capital markets to any in-licensing, product acquisition or other transaction we may enter into; and (vi) ability to enter into additional collaborations and the success of such collaborations.

Financing may not be available to us when we need it, or on favorable or acceptable terms, or at all. We could be required to seek funds through collaborative alliances or through other means that may require us to relinquish rights to some of our technologies, product candidates or products that we would otherwise pursue on our own. In addition, if we raise additional funds by issuing equity securities, our existing stockholders may experience dilution, or an equity financing that involves existing stockholders may cause a concentration of ownership. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, and are likely to include rights that are senior to the holders of our common stock. Any additional debt or equity financing may contain terms which are not favorable to us or to our stockholders, such as liquidation and other preferences, or liens or other restrictions on our assets. As discussed in Note 16 to the consolidated financial statements appearing elsewhere in this Form 10-K, additional equity financings may also result in cumulative changes in ownership over a three-year period in excess of 50% which would limit the amount of net operating loss and tax credit carryforwards that we may utilize in any one year. If we are unable to obtain adequate funding on a timely basis or at all, we will be required to terminate, modify or delay clinical trials of our product candidates, or relinquish rights to portions of our technology, product candidates and/or products.

In addition, we recently undertook staff furloughs and salary reductions for certain of our employees, and we may undertake other cost-saving initiatives in the future. The actions we announced on April 13, 2023, as well as future restructuring or cost-saving initiatives, may not achieve our goal of preserving capital or raising additional capital.

If we are unable to raise additional capital when required or on acceptable terms, or we are unsuccessful in our cost-saving measures, we may be required to:

- significantly delay, scale back, or discontinue the development or commercialization of our product candidates;
- seek strategic alliances, or amend existing alliances, for research and development programs at an earlier stage than otherwise would be desirable or that we otherwise would have sought to develop independently, or on terms that are less favorable than might otherwise be available in the future;
- dispose of technology assets, or relinquish or license on unfavorable terms, our rights to technologies or any of our product candidates that we otherwise would seek to develop or commercialize ourselves;
- pursue the sale of our company to a third party at a price that may result in a loss on investment for our stockholders; or

- file for bankruptcy or cease operations altogether.

Any of these events could have a material adverse effect on our business, operating results, and prospects.

***Adverse developments affecting financial institutions, companies in the financial services industry or the financial services industry generally, such as actual events or concerns involving liquidity, defaults or non-performance by financial institutions, could adversely affect our current financial condition, operations and liquidity.***

Actual events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10, 2023, one of our banking partners, Silicon Valley Bank, ("SVB"), was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation, or the Federal Deposit Insurance Corporation ("FDIC"), as receiver. As more fully described in Note 19, "Subsequent Events," as of March 10, 2023, we had approximately 56% of our cash and cash equivalent balances in segregated custodial accounts held by a third-party custodian for which SVB was our agent and/or SVB Asset Management, an affiliate of SVB, was the advisor at the time SVB was closed.

On March 12, 2023, the Department of the Treasury, the Federal Reserve, and the FDIC jointly released a statement that depositors at SVB would have access to their funds, even those funds in excess of the standard FDIC insurance limits, under a systemic risk exception. As of March 13, 2023, we had access to our cash and cash equivalents at SVB; however, there is uncertainty in the markets regarding the stability of regional banks and the safety of deposits in excess of the FDIC insured deposit limits. There is no guarantee that the U.S. Department of Treasury, FDIC and Federal Reserve Board will provide access to uninsured funds in the future in the event of the closure of other banks or financial institutions in a timely fashion or at all. Furthermore, we may be impacted by other disruptions to the U.S. banking system caused by the recent developments involving SVB, including potential delays in our ability to transfer funds, make payments, or receive funds whether held with SVB or other banks.

***We expect that we will continue to incur net losses in the foreseeable future.***

During the year ended December 31, 2022, we incurred a net loss of $23.4 million, as compared to net income of $98.1 million for the year ended December 31, 2021. As of December 31, 2022, we had an accumulated deficit of $758.8 million and a cash and cash equivalents balance of $12.0 million. We expect to incur substantial operating losses in future periods and will require additional capital as we seek to advance any future product candidates through development to commercialization. We do not expect to generate product revenue, sales-based milestones or royalties until we successfully complete development of and obtain marketing approval for our product candidates, either alone or in collaboration with third parties, which may not occur or may take a number of years. To commercialize any future product candidates, we need to complete clinical development and comply with comprehensive regulatory requirements. We are subject to numerous risks and uncertainties similar to those of other companies of the same size within the biotechnology industry, such as uncertainty of clinical trial outcomes, uncertainty of additional funding and history of operating losses.

Even if we succeed in receiving marketing approval for and commercializing any product candidate, we will continue to incur substantial research and development and other expenditures to develop and market additional potential indications or products. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

***Our stock price has been and may continue to be volatile, and the value of an investment in our common stock may decline.***

We historically have experienced significant volatility in our stock price. Since December 31, 2022, our common stock has traded as high as 12.07 and as low as 2.14 per share. The realization of any of the risks described in these risk factors or other unforeseen risks could have an adverse effect on the market price of our common stock. The trading price of our common stock is likely to continue to be highly volatile and could be subject to declines in response to numerous factors, including disappointing results in a clinical program, as was the case following the announcement of topline results for ILLUMINATE-301. Other risk factors include results from clinical trials; FDA

regulatory actions; announcements by us or our competitors of acquisitions, regulatory approvals, clinical milestones, new products, significant contracts, commercial relationships, or capital commitments; additions or departures of key personnel; commencement of, or our involvement in, litigation; and any major change in our Board of Directors or management.

From time to time, we estimate the timing of the potential accomplishment of clinical and other development goals or milestones. These estimated milestones may include the commencement or completion of clinical trials. Also, from time to time, we expect that we will publicly announce the anticipated timing of some of these milestones. All estimated milestones are based on numerous assumptions. These milestones may change and the actual timing of meeting these milestones may vary dramatically from our estimates, in some cases for reasons beyond our control. If we do not meet these estimated milestones, or the anticipated timing thereof, as publicly announced, our stock price may decline.

***We may not be able to comply with Nasdaq's continued listing standards.***

Our Common Stock trades on The Nasdaq Capital Market ("Nasdaq") under the symbol "ACGN." We cannot assure you that our securities will continue to be listed on Nasdaq.

As previously reported, on November 26, 2021, we received a deficiency letter (the "First Nasdaq Letter") from the Nasdaq Listing Qualifications Department (the "Staff"), notifying us that we were not in compliance with Nasdaq Listing Rule 5550(a)(2), which requires us to maintain a minimum bid price of at least $1 per share for continued listing (the "Minimum Bid Requirement"). Our failure to comply with the Minimum Bid Requirement was based on the Common Stock per share price being below the $1.00 threshold for a period of 30 consecutive business days. Pursuant to the First Nasdaq Letter, we had 180 calendar days from November 26, 2021 to regain compliance with the Minimum Bid Requirement.

Also as previously reported, on May 26, 2022, we received a second notice (the "Second Nasdaq Letter") from the Staff indicating that, while we had not regained compliance with the Minimum Bid Requirement, the Staff had determined that we were eligible for an additional 180-day period, or until November 21, 2022, to regain compliance with the Minimum Bid Requirement.

On November 22, 2022, the Company was notified by the Staff that, based upon the Company's continued non-compliance with the Minimum Bid Requirement, the Company's securities were subject to delisting unless the Company timely requested a hearing before the Nasdaq Hearings Panel (the "Panel").

The Company timely requested a hearing before the Panel, which request stayed any further delisting action by the Staff at least pending the conclusion of the Company's hearing before the Panel and the expiration of any extension period that may be granted by the Panel to the Company following the hearing. The Company had its Panel hearing on January 5, 2023. On January 10, 2023, the Company received a letter on behalf of the Panel granting an extension until January 20, 2023, for the Company to regain compliance with the continued listing standards.

Following the successful completion of the Company's 1-to-17 reverse stock split, on January 25, 2023, the Company received a written notice from the Panel that the Company satisfied all initial listing requirements of Nasdaq, as required by the Panel.

While we regained compliance and are currently in compliance with Nasdaq continued listing requirements, there is no guarantee that we will be able to perpetually satisfy Nasdaq's continued listing requirements to maintain our listing on Nasdaq for any periods of time. Our failure to continue to meet these requirements may result in our securities being delisted from Nasdaq.

As of December 31, 2022, we had an accumulated deficit of $758.8 million and total stockholders' equity of $11.8 million. We expect to incur substantial operating losses in future periods and will require additional capital as we seek to advance any future product candidates through development to commercialization. Additionally, since December 31, 2022, our common stock has traded as low as 2.14 per share. If we fail to comply with Nasdaq rules and requirements, including, without limitation, with (i) the Minimum Bid Requirement, or (ii) Nasdaq Listing Rule 5550(b)(1), which requires the Company to maintain a minimum of $2.5 million in stockholders' equity (the "Minimum Equity Requirement"), our stock may be delisted. In addition, even if we demonstrate compliance

with the Minimum Bid Requirement and Minimum Equity Requirement, we will have to continue to meet other objective and subjective listing requirements to continue to be listed on Nasdaq. Delisting from Nasdaq could make trading our common stock more difficult for investors, potentially leading to declines in our share price and liquidity. Without a Nasdaq listing, stockholders may have a difficult time getting a quote for the sale or purchase of our common stock, the sale or purchase of our common stock would likely be made more difficult, and the trading volume and liquidity of our common stock could decline. Delisting from Nasdaq could also result in negative publicity and could also make it more difficult for us to raise additional capital. The absence of such a listing may adversely affect the acceptance of our common stock as currency or the value accorded by other parties. Further, if we are delisted, we would also incur additional costs under state blue sky laws in connection with any sales of our securities. These requirements could severely limit the market liquidity of our common stock and the ability of our stockholders to sell our common stock in the secondary market. If our common stock is delisted by Nasdaq, our common stock may be eligible to trade on an over-the-counter quotation system, such as the OTCQB Market, where an investor may find it more difficult to sell our stock or obtain accurate quotations as to the market value of our common stock. In the event our common stock is delisted from Nasdaq, we may not be able to list our common stock on another national securities exchange or obtain quotation on an over-the counter quotation system.

**Risks Relating to Our Business, Strategy, and Industry**

***Our recent organizational changes, including the Aceragen Acquisition, and any acquisitions or business development opportunities we pursue may not be successful.***

As part of our business strategy, we review and intend to continue to review acquisition opportunities that we believe would be advantageous or complementary to the development of our business. During the third quarter of 2022, we acquired Legacy Aceragen, and we may acquire additional businesses, assets, or technologies in the future. If we make any acquisitions, we could take any or all of the following actions, any one of which could adversely affect our business, financial condition, results of operations or share price:

- use a significant portion of our available cash, if any;
- require a significant devotion of management's time and resources in the pursuit or consummation of any acquisition;
- incur debt, which may not be available to us on favorable terms and may adversely affect our liquidity;
- issue equity or equity-based securities that would dilute existing stockholders' ownership percentage;
- assume contingent and other liabilities; and
- take charges in connection with such acquisitions.

For example, prior to the Aceragen Acquisition, Legacy Aceragen was obligated to pay an aggregate amount of $8.0 million to certain former stockholders of Arrevus, Inc. (the "Former Stockholders"). As a result of this obligation and subsequent to the Aceragen Acquisition, on January 31, 2023 the Company issued 12% convertible unsecured promissory notes to certain of the Former Stockholders in an aggregate amount of approximately $5.9 million, which convertible notes bear annual interest at 12%. Under the terms of the convertible notes, at the holder's election, any or all of the then outstanding principal and accrued interest may be converted into shares of Company's common stock. The terms of the convertible notes provide the Former Stockholders with customary registration rights covering the common stock issued following any conversion of the convertible notes.

Acquisitions also entail numerous other risks, including, without limitation: difficulties in assimilating acquired operations, products, technologies and personnel; unanticipated costs; diversion of management's attention from existing operations; risks of entering markets in which we have limited or no prior experience; risk relating to obtaining regulatory approvals; unanticipated costs or liabilities; and potential loss of key employees from either our existing business or the acquired organization. Acquisitions may result in accounting charges for restructuring and other expenses, amortization of purchased technology and intangible assets and stock-based compensation expense, any of which could materially and adversely affect our operating results. We may not be able to realize the anticipated synergies, innovation, operational efficiencies, benefits of or successfully integrate with our existing business the businesses, products, technologies or personnel that we acquire, and our failure to do so could harm our business, operating results, and potentially our share price.

***As a small biopharmaceutical-focused company with limited resources, we may be unable to attract and retain qualified personnel.***

As of March 31, 2023, we had 26 full-time employees. We are a small company and any future growth will require hiring additional qualified personnel. Also, because of the specialized scientific nature of our business, we face intense competition for qualified employees and consultants from biopharmaceutical companies, research organizations and academic institutions. Failure to attract and retain qualified personnel would materially harm our ability to compete effectively and grow our business.

***We depend on our senior management team, and the loss of one or more of our executive officers or key employees or an inability to attract and retain highly skilled employees could compromise our ability to pursue our growth strategy and grow our business.***

Our success depends largely upon the continued services of our executive officers and other key employees. We do not maintain "key person" insurance for our executive officers. From time to time there may be changes in our senior management team resulting from the hiring or departure of executives, which could disrupt our business. For example, in 2022, in connection with the Aceragen Acquisition, Vincent Milano resigned as Chief Executive Officer of the Company. Mr. Milano was replaced by John Taylor, the Chief Executive Officer of Legacy Aceragen. All of our employees' employment is at-will, including the employment of our Chief Executive Officer and our Chief Financial Officer, which means that any of these employees could leave our employment at any time. The replacement of one or more of our executive officers or other key employees would likely involve significant time and costs and may significantly delay or prevent the achievement of our business objectives.

***We are depending heavily on the development, regulatory approval, and commercialization of product candidates. If we are unable to successfully develop and commercialize product candidates, or experience significant delays in doing so, our business may be materially harmed.***

We have made and intend to continue to make a significant investment of our time and financial resources in the development and commercialization of our product candidates. Our ability to generate product revenues will depend heavily on our ability to successfully develop, obtain regulatory approval for, and commercialize our product candidates. If we fail to obtain regulatory approval and successfully commercialize our product candidates, our business would be materially and adversely impacted. Even if our product candidates receive regulatory approval, we will incur significant expenses to support its commercialization and launch, which investment may never be realized if sales are insufficient.

***If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.***

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the U.S. Enrollment may be particularly difficult for our product candidates that are intended for rare and orphan diseases.

In addition, some of our competitors have ongoing clinical trials for product candidates that treat the same indications as our product candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates. Our inability to enroll a sufficient number of patients for our clinical trials could also require us to abandon one or more clinical trials altogether. Enrollment delays may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

***If our clinical trials are unsuccessful, delayed or terminated, we may not be able to develop and commercialize our product candidates.***

We have not yet been able to commence our ADVANCE study for ACG-801 due to an FDA clinical hold. While we expect to be able to resolve the clinical hold and commence the ADVANCE study in the second half of 2023, there is no guarantee that FDA will agree and will permit us to begin the study. Additionally, there is a risk that we may experience additional clinical holds and will not be able to obtain timely or any approval for our product candidates, and we may need to carry out additional clinical trials, non-clinical studies, CMC assessments, and /or remediation. Clinical trials are lengthy, complex, and expensive processes with uncertain results. We may not be able to

complete any clinical trial of an investigational product within any specified time period or at all. Moreover, clinical trials may not show our investigational products to have an acceptable safety and efficacy profile. The FDA, IRBs, Drug Safety Monitoring Boards, or other equivalent foreign regulatory agencies may not allow us to complete these trials or commence and complete any other clinical trials.

Numerous unforeseen events may occur during, or as a result of, preclinical testing, nonclinical testing or the clinical trial process that could delay or inhibit the ability to receive regulatory approval or to commercialize products. For example, setbacks in clinical trials may result in enhanced scrutiny by regulators or IRBs of clinical trials of our product candidates. Regulators or IRBs may also prohibit the commencement or continuation of clinical trials, data monitoring committees may recommend that studies be modified or discontinued, regulatory authorities may require additional nonclinical studies as a precondition to commencing clinical trials or imposing restrictions on the design or scope of clinical trials that could slow enrollment of trials, increase the costs of trials or limit the significance of the results of trials. Such setbacks could also adversely impact the desire of investigators to enroll patients in, and the desire of patients to enroll in, clinical trials of our product candidates.

Other events that could delay or inhibit conduct of our clinical trials, or prevent receipt of product approval, include: (i) nonclinical or clinical data may not be readily interpreted, which may lead to delays and/or misinterpretation; (ii) our nonclinical tests, including toxicology studies, or clinical trials may produce negative or inconclusive results; (iii) we might have to suspend or terminate our clinical trials if the participating subjects experience serious adverse events or undesirable side effects or are exposed to unacceptable health risks; (iv) regulators or IRBs may hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements, issues identified through inspections of manufacturing or clinical trial operations or clinical trial sites; (v) we, along with our collaborators and subcontractors, may not employ, in any capacity, persons who have been debarred under by FDA or similar foreign regulatory authorities; (vi) we or our contract manufacturers may be unable to manufacture sufficient quantities of our product candidates for use in clinical trials meeting the applicable quality standards or regulatory authorities may disagree with our manufacturing and control methods; (vii) the cost of our clinical trials may be greater than we currently anticipate making continuation and/or completion improbable; (viii) our investigators and contract research organizations may not follow the applicable regulatory requirements requiring that studies be terminated or repeated; (ix) FDA and regulatory authorities may not agree with the design or analysis of the results from our clinical studies; (x) there may be changes in approval requirements that may necessitate that we conduct additional development work; and (ix) our product candidates may not cause the desired effects or may cause undesirable side effects or our product candidates may have other unexpected characteristics. These risks may be increased for product candidates intended for the treatment of diseases or conditions where there may be limited clinical experience; where there may not be validated or established measures to show improvement of a subject's condition; where we are using new, unvalidated, or unestablished, endpoints, tests or methodologies; or where the product candidates are new or novel.

In conducting clinical trials, we cannot be certain that any planned clinical trial will begin on time, if at all. Delays in commencing clinical trials of potential products could increase our product candidate development costs, delay any potential revenues, reduce the potential length of patent exclusivity and reduce the probability that a potential product will receive regulatory approval. Significant clinical trial delays also could allow our competitors to bring products to market before we do and impair our ability to commercialize our product candidates.

***The technologies on which we rely are unproven and may not result in any approved and marketable products.***

Our technologies or therapeutic approaches are relatively new and unproven. Further, the chemical and pharmacological properties of our product candidates may not be fully recognized in preclinical studies and small-scale clinical trials, and such compounds may interact with human biological systems in unforeseen, ineffective or harmful ways that we have not yet identified. Pre-clinical trials and early-stage clinical trials may not be indicative of results that may be obtained in later stage trials. As a result of these factors, we may never succeed in obtaining regulatory approval to market any product.

***We face substantial competition, which may result in others discovering, developing, or commercializing products before or more successfully than us.***

The development and commercialization of new products is highly competitive. There are many other companies, public and private, actively engaged in discovery, development, and commercializing products and technologies that may compete with our product candidates and rare disease program. Some potentially competitive products have been in development or commercialized for years. Many of the marketed products have been accepted by the

medical community, patients, and third-party payors. Our ability to compete may be affected by the previous adoption of such products by the medical community, patients, and third-party payors.

We recognize that other companies, including large pharmaceutical companies, may be developing or have plans to develop products and technologies that may compete with ours. Many of our competitors have substantially greater financial, technical, and human resources than we have and/or may have significantly greater experience than we have in undertaking preclinical studies and human clinical trials of new pharmaceutical products, obtaining FDA and other regulatory approvals of products for use in health care and manufacturing, and marketing and selling approved products. We anticipate that the competition with our product candidates and technologies will be based on a number of factors including product efficacy, safety, availability, reimbursement, insurance coverage, and price. The timing of market introduction of our product candidates and competitive products will also affect competition among products. We expect the relative speed with which we can develop products, complete the clinical trials and approval processes, and supply commercial quantities of the products to the market to be important competitive factors.

Information regarding our competitors is discussed in further detail in the section entitled “Business” of this Form 10-K.

***Our business could be adversely affected by the effects of health epidemics, such as the ongoing COVID-19 global pandemic, including disruptions to our clinical trials or the delay of regulatory approvals.***

Our business may be adversely affected by the effects of health epidemics, including the ongoing worldwide COVID-19 pandemic. The COVID-19 pandemic has caused significant volatility and uncertainty globally. This has resulted in an economic downturn and may disrupt our business and delay our clinical trials and regulatory approvals. This may also result in an interruption or issues with respect to the manufacture and supply of our product candidates. The COVID-19 pandemic may also require that changes be made to any clinical trials or product manufacturing that may ultimately have an adverse impact.

The COVID-19 pandemic continues to evolve. The ultimate impact of the COVID-19 pandemic is highly uncertain and subject to change. We do not yet know the full extent of potential delays or impacts on our business, our clinical trials, healthcare systems or the global economy. However, any one or a combination of these events could have an adverse effect on the operation of and results from our clinical trials, which could prevent or delay us from obtaining approval for our product candidates, or on our employee resources.

**Risks Relating to Regulatory Approval and Marketing and Other Compliance Matters**

***Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming, and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates.***

We are not permitted to market our product candidates in the U.S. or in other countries until we, or any future collaborators, receive marketing approval from the FDA or marketing approval from applicable regulatory authorities outside of the U.S. The approval process is lengthy, often taking a number of years, is uncertain, and is expensive. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate’s safety and efficacy. Information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities is also required. Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability or that of any collaborators we may have to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

***Our failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad which subjects us to additional business risks that could adversely affect our operations.***

We, and any future collaborators, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements in foreign jurisdictions. The approval procedure varies among countries and can involve additional studies. The time required to obtain approval may differ substantially from that required to obtain FDA approval. In addition, in many countries outside of the U.S., it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We, and any future collaborators,

may not obtain approvals from regulatory authorities outside of the U.S. on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in foreign jurisdictions, and approval by one regulatory authority outside of the U.S. does not ensure approval by regulatory authorities in other jurisdictions or by the FDA.

***Even if we, or any future collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.***

We, and any future collaborators, must comply with requirements concerning advertising and promotion for any of our product candidates for which we or they obtain marketing approval. Such promotional communications are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we, and any future collaborators, will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to Good Manufacturing Practices ("cGMPs"), which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, our future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA, and other regulatory authorities to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or our future collaborators, receive marketing approval for one or more of our product candidates, we, and our future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and our future collaborators, are not able to comply with post-approval regulatory requirements, we, and our future collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or our future collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Moreover, legislative and regulatory proposals have been made to expand post-approval requirements and restrict promotional activities relating to our drug and biologic products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and any collaborators to more stringent product labeling and post-marketing testing and other requirements.

***Any of our product candidates for which we, or our future collaborators, obtain marketing approval in the future could be subject to post-approval restrictions or withdrawal from the market and we, and our future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.***

Any of our product candidates for which we, or our future collaborators, obtain marketing approval in the future, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a Risk Evaluation and Mitigation Strategy, which could include requirements for a restricted distribution system.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the DOJ, closely regulate and

monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed, and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or our future collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label promotion.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements both before and after product approval, may yield various results, including: (i) litigation involving patients taking our product; (ii) restrictions on such products, manufacturers or manufacturing processes; (iii) restrictions on the labeling or marketing of a product; (iv) restrictions on product distribution or use; (v) requirements to conduct post-marketing studies or clinical trials; (vi) warning letters or untitled letters, as well as other enforcement and adverse actions; (vii) withdrawal of the products from the market; (viii) refusal to approve pending applications or supplements to approved applications that we submit; (ix) recall of products; (x) fines, restitution or disgorgement of profits or revenues; (xi) suspension or withdrawal of marketing approvals; (xii) damage to relationships with any potential collaborators; (xiii) unfavorable press coverage and damage to our reputation; (xiv) refusal to permit the import or export of products; (xv) product seizure; or (xvi) injunctions or the imposition of civil or criminal penalties.

***We may not be able to obtain or maintain anticipated periods of regulatory exclusivity for our product candidates.***

We currently anticipate that our product candidates may be eligible for certain periods of regulatory exclusivity. By example, the FDA has granted us orphan drug designation for ACG-701 (sodium fusidate) for the treatment of CF PEx patients and for ACG-801 (recombinant human acid ceramidase) for the treatment of Farber disease. Additionally, ACG-801 was granted orphan drug designation by the EMA for Farber disease. We also have received QIDP designation for ACG-701 for the treatment of CF PEx and we anticipate that, depending on the product candidate, we may be eligible for certain periods of exclusivity under the Hatch Waxman Act and the Biologic Price Competition and Innovation Act. There can be no assurance that we will obtain any anticipated periods of regulatory exclusivity or that we will be able to maintain the designations that are necessary for such exclusivities. We also may not be able to obtain additional necessary designations in the future. Moreover, even if we initially receive periods of regulatory exclusivity following product approval, such exclusivities may be lost or overcome under certain circumstances, and the scope of such exclusivities may not provide sufficient protection from competition.

***A breakthrough therapy, fast track, or other expedited designation for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that those product candidates will receive marketing approval.***

The FDA has granted us Fast Track designation for ACG-701 (sodium fusidate) for the treatment of CF PEx patients and for ACG-801 (recombinant human acid ceramidase) for the treatment of Farber disease.

We may also seek a breakthrough therapy, fast track, or other designation for appropriate product candidates. Designations such as these are within the discretion of the FDA, or other comparable regulatory authorities. The receipt of a designation for a product candidate may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify under one of FDA's designation programs, the FDA may later decide that the products no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

***We have only limited experience in regulatory affairs and our product candidates are based on new technologies; these factors may affect our ability or the time we require to obtain necessary regulatory approvals.***

We have never obtained regulatory approval for, or commercialized, a product. It is possible that the FDA may refuse to accept any or all of our planned marketing applications for substantive review or may conclude, after review of our data, that our applications are insufficient to obtain regulatory approval of any of our product candidates. The FDA may also require that we conduct additional clinical or manufacturing validation studies, which may be costly and time-consuming, and submit that data before it will reconsider our applications. Depending on the extent of these or any other FDA required studies, approval of any marketing application that we

submit may be significantly delayed, possibly for years, or may require us to expend more resources than we have available or can secure.

***A variety of factors, including inadequate funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel, accept payments of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result of these and other factors. In addition, government funding of government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new therapeutic candidates to be reviewed and/or approved by necessary government agencies, which could adversely affect our business. For example, over the last several years, the U.S. government has shut down several times. If a prolonged government shutdown occurs, or if global health concerns (such as those relating to the COVID-19 pandemic) continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

***We are subject to extensive and costly governmental regulation, the violation of which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, and diminished profits and future earnings.***

Our product candidates are subject, and any future commercial products will be subject to costly, extensive and rigorous domestic and foreign government regulation, as discussed under the caption “Government Regulation” within Item 1 of this Form 10-K. These requirements are continually evolving, which will require us to adapt our practices and processes, which we may not be able to do.

In addition, our future arrangements with third party payors, healthcare providers and physicians may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. These include, but are not limited to, the following: the Anti-Kickback Statute; the Foreign Corrupt Practices Act; the False Claims Act; privacy laws such as HIPAA; transparency requirements; and analogous state and foreign laws. Additionally, some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require manufacturers to report information related to drug pricing and to certain payments and other transfers of value to physicians, other healthcare providers, and healthcare entities, or marketing expenditures.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, suspension and debarment from procurement and non-procurement transactions, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil, or administrative sanctions, including exclusions from government funded healthcare programs.

***We depend on information technology, infrastructure, and data to conduct our business. Any significant disruption, or cyber-attacks, could have a material adverse effect on our business.***

We are dependent upon information technology, infrastructure, and data. Computer systems, including ours and those of our suppliers, partners, and service providers, contain sensitive confidential information or intellectual property, and are vulnerable to service interruption or destruction, cyber-attacks (both malicious and random) and other natural or man-made incidents or disasters, which may be prolonged or go undetected. Such events are increasing in their frequency, sophistication, and intensity, and have become increasingly difficult to detect. A significant interruption of our information technology could adversely affect our ability to manage and keep our operations running efficiently and effectively. An incident that results in a wider or sustained disruption to our business or products could have a material adverse effect on our business, financial condition and results of operations.

Likewise, data privacy or security breaches by employees or others may pose a risk that sensitive data, including our intellectual property, trade secrets or personal information of our employees, patients, or other business partners may be exposed to unauthorized persons or to the public. There can be no assurance that our efforts, or the efforts of our partners and vendors, will prevent service interruptions, or identify breaches in our systems, that could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business, or reputational harm to us. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyberattacks, and other related breaches.

***Climate change, environmental, social and governance and sustainability initiatives may result in regulatory or structural industry changes that could require significant operational changes and expenditures, reduce demand for the Company's products and adversely affect our business, financial condition, and results of operations.***

Climate change, environmental, social and governance ("ESG") and sustainability are a growing global movement. These matters have garnered continuous political and social attention leading to the introduction of national, regional, and local legislation, regulatory requirements, reporting obligations, and policy changes. Further, there is a growing societal demand to limit greenhouse gas emissions and support global initiatives. Compliance with these international agreements and measures may necessitate operational changes, impose taxes or require purchases of emission credits, leading to significant capital expenditures. Furthermore, future legislative, regulatory, or policy changes may emerge that require additional modifications to reduce greenhouse gas emissions from our operations, which may result in substantial capital expenditures.

The growing focus on climate change, ESG, and sustainability has led to government investigations and litigation, both public and private, which may result in increased costs or adverse effects on our business or financial results. Furthermore, entities that offer investors information regarding corporate governance and similar issues have developed rating systems for assessing companies on their ESG approach. Some investors use these ratings to inform their investment and voting decisions. If we receive unfavorable ESG ratings, it may lead to a rise in negative investor sentiment towards us, which could harm our securities' price and our ability to access capital at reasonable costs.

Any or all of these ESG and sustainability initiatives may result in significant operational changes and expenditures, cause us reputational harm, and could materially adversely affect our business, financial condition, and results of operations.

**Risks Relating to Collaborators**

***Our existing collaborations, clinical funding arrangements, and any collaborations or arrangements we enter into in the future may not be successful.***

Our current collaboration and clinical funding agreements, as more fully described within Item 1 of this Form 10-K, or any collaborations we may enter into in the future, may not be successful. The success of our collaborative alliances, if any, will depend heavily on the efforts and activities of our collaborators. Our existing collaborations and any potential future collaborations have risks, including the following: (i) our collaborators may control the development (and timing thereof) of the product candidates being developed with our technologies and compounds; (ii) our collaborators may control the public release of information regarding the developments; (iii) disputes may arise in the future with respect to the ownership of or right to use technology and intellectual property

developed with our collaborators; (iv) disagreements with our collaborators could delay or terminate the development of our products, or result in litigation or arbitration; (v) we may have difficulty enforcing the contracts if any of our collaborators fail to perform; (vi) our collaborators may terminate their collaborations with us, which could make it difficult for us to attract new collaborators or adversely affect the perception of us in the business or financial communities; (vii) our collaboration agreements are likely to be for fixed terms and subject to termination by our collaborators in the event of a material breach or lack of scientific progress by us; (viii) our collaborators may challenge our intellectual property rights or utilize our intellectual property rights in such a way as to invite litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential liability; (ix) our collaborators may not comply with all applicable regulatory requirements; (x) our collaborators may under fund or not commit sufficient resources to the testing or development of our product candidates; and (xi) our collaborators may develop alternative products either on their own or in collaboration with others, or encounter conflicts of interest or changes in business strategy or other business issues. Additionally, our collaborators will face the same development risks that we do and may not be successful in their efforts. Given these risks, it is possible that any collaborative alliance into which we enter may not be successful.

***If we are unable to establish additional collaborative alliances, our business may be materially harmed.***

Collaborators provide the necessary resources and product development experience to advance our compounds in their programs. We have entered into and expect to continue to seek to enter into collaborative alliances with pharmaceutical companies. Upfront payments and milestone payments received from collaborations help to provide us with the financial resources for our internal research and development programs. We believe additional resources will be required to advance compounds. If we do not reach agreements with additional collaborators in the future or if the terms of such a collaborative alliance on are not favorable to us, we may not be able to obtain the expertise and resources necessary to achieve our business objectives, our ability to advance our compounds will be jeopardized and we may fail to meet our business objectives. Moreover, collaborations are complex and time consuming to negotiate, document, and implement. We may not be successful in our efforts to establish and implement collaborations on a timely basis.

***We are subject to substantial customer concentration. If we fail to retain our revenues from the U.S. Government consistent with historical performance or acquire new customers cost-effectively, our business could be adversely affected.***

We are subject to substantial customer concentration risk. The U.S. Government accounted for all of the Company’s revenues for the year ended December 31, 2022. These revenues are from collaboration and license agreements and a contract with the U.S. Government. If these agreements are terminated or fees and funding are reduced under these agreements, our business could be adversely affected.

**Risks Relating to Intellectual Property**

***If we are unable to obtain and maintain patent protection for our discoveries, the value of our technology and any product candidates could be adversely affected.***

Our commercial success and ability to develop and commercialize products depends in significant part on our ability to: (i) obtain and maintain valid and enforceable patents and other intellectual property rights in the U.S. and other countries with respect to our technology and product candidates; (ii) obtain licenses to the proprietary rights of others on commercially reasonable terms; (iii) operate without infringing upon the proprietary rights of others; (iv) prevent others from infringing on our proprietary rights; and (v) protect our trade secrets. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. We seek to protect our proprietary position by filing and prosecuting patent applications in the U.S. and abroad related to our novel technologies and product candidates that are important to our business.

If the scope of the patent protection we or our licensors obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our patents have, or that any of our pending patent applications that

mature into issued patents will include, claims with a scope sufficient to protect our current and future product candidates or otherwise provide any competitive advantage. In addition, to the extent that we license intellectual property in the future, we cannot provide assurances that those licenses will remain in force. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

Our patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapy that provides benefits similar to one or more of our product candidates but that falls outside the scope of our patent protection. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business.

Patent positions of life sciences companies can be uncertain and involve complex factual and legal questions. No consistent policy governing the scope of claims allowable in the pharmaceutical and biotechnology fields have emerged in the United States. The scope of patent protection in jurisdictions outside of the United States is also uncertain. Changes in either the patent laws or their interpretation in any jurisdictions in which we seek patent protection may diminish our ability to protect our inventions and maintain and enforce our intellectual property rights; and, more generally, may affect the value of our intellectual property, including the narrowing of the scope of our patents and any that we may license.

The patent prosecution process is complex, expensive, time-consuming and inconsistent across jurisdictions. We may not be able to file, prosecute, maintain, enforce, or license all necessary or desirable patent rights at a commercially reasonable cost or in a timely manner or at all. In addition, we may not pursue or obtain patent protection in all relevant markets. It is possible that we will fail to identify important patentable aspects of our research and development efforts in time to obtain appropriate, or any, patent protection. While we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development efforts, including for example, our employees, external academic scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach the agreements and disclose our confidential or proprietary information before a patent application is filed, thereby endangering our ability to seek patent protection. In addition, publications of discoveries in the scientific and scholarly literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Consequently, we cannot be certain that we were the first to file for patent protection on the inventions claimed in our patents or pending patent applications.

The issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Pending patent applications cannot be enforced against third parties unless, and until, patents issue from such applications, and then only to the extent the issued claims cover the technology. There can be no assurance that our patent applications or any patent applications that we may license in the future will result in patents being issued. Further, the scope of the invention claimed in a patent application can be significantly reduced before the patent is issued, and this scope can be reinterpreted after issuance. Even if patent applications we currently own or that we may license in the future issue as patents, they may not issue in a form that will provide us with adequate protection to prevent competitors or other third parties from competing with us, or otherwise provide us with a competitive advantage. Any patents that eventually issue may be challenged, narrowed or invalidated by third parties. Consequently, we do not know whether our technology or any of our product candidates will be protectable or remain protected by valid and enforceable patent rights. Our competitors or other third parties may be able to evade our patent rights by developing new products that are similar to our product candidates, biosimilars of our product candidates, or alternative technologies or products in a non-infringing manner.

The issuance or grant of a patent is not irrefutable as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. We may in the future, become

subject to a third-party pre-issuance submission of prior art or opposition, derivation, revocation, re-examination, post-grant and inter partes review, or interference proceeding and other similar proceedings challenging our patent rights or the patent rights of others in the U.S. Patent and Trademark Office, or USPTO, or other foreign patent office. An unfavorable determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or limit or extinguish our ability to manufacture or commercialize products without infringing third-party patent rights.

In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. Moreover, some of our owned and in-licensed patents and patent applications may in the future be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we or our licensors may need the cooperation of any such co-owners of our owned and in-licensed patents in order to enforce such patents against third parties, and such cooperation may not be provided to us or our licensors. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

***We do not own or license patents or patent applications that claim the active pharmaceutical ingredient in our ACG-701 and ACG-801 product candidates.***

The active pharmaceutical ingredient in ACG-701, sodium fusidate, is an old compound that was published in the 1960s, has been approved for therapeutic use in jurisdictions outside the United States and is included in ex-US cystic fibrosis treatment guidelines. We do not own or license patents that claim sodium fusidate or its acid form, fusidic acid, as a composition of matter. We do own patents in the United States and in other jurisdictions that claim certain methods of treatment using ACG-701, and we seek additional patent claims on certain compositions. There can be no assurances that our granted patents will, or that any of our pending patent applications that grant as patents will, effectively prevent others from commercializing competitive technologies and products. There is a risk that others, including companies that make generic pharmaceuticals, may develop products that are the same as or similar to ACG-701 for the same or similar uses as us, and that our patents will not effectively prevent them from commercializing their products.

The active pharmaceutical ingredient in ACG-801 is a recombinant form of acid ceramidase. Human acid ceramidase is a naturally occurring protein and patentability of such proteins is limited under current patent law. We do not own or license patents that claim recombinant human acid ceramidase as a composition of matter. We own patents in the United States and in other jurisdictions that claim certain compositions of and methods of treatment using acid ceramidase, and we seek additional patent claims on certain compositions. There can be no assurances that our granted patents will, or that any of our pending patent applications that grant as patents will, effectively prevent others from commercializing competitive technologies and products. There is a risk that others, including companies that make biosimilar pharmaceuticals, may develop products that are similar to ACG-801 for the same or similar uses as us, and that our patents will not effectively prevent them from commercializing their products.

***Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Our rights to develop and commercialize ACG-801 are subject to the terms and conditions of our license agreement with Icahn School of Medicine at Mount Sinai, and our rights to develop and commercialize future product candidates may be subject to terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under our current or future intellectual property license agreements or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose intellectual property rights that are important to our business and lose the ability to continue to develop and commercialize the related product.***

We are and expect to continue to be reliant upon third-party licensors for certain patent and other intellectual property rights that are important or necessary to the development of our technology and product candidates. In particular, ACG-801 is dependent on our Amended and Restated License Agreement with Icahn School of Medicine at Mount Sinai ("Mt. Sinai License"). Pursuant to the Mt. Sinai License we are the exclusive licensee of patent

rights and know-how of Mt. Sinai. The Mt. Sinai License imposes, and we expect that any future license agreement will impose, specified diligence, milestone payment, royalty, commercialization, development and other obligations on us and require us to meet development timelines, or to exercise diligent or commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses.

Furthermore, Mt. Sinai has, and future licensors may have, the right to terminate the license if we materially breach the agreement and fail to cure such breach within a specified period or in the event we undergo certain bankruptcy events. In spite of our best efforts, our current or any future licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements. If the Mt. Sinai License is terminated, and we lose our intellectual property rights under the Mt. Sinai License, this may result in complete termination of our development and commercialization activities with respect to ACG-801. If the Mt. Sinai License is terminated, or if the underlying intellectual property fails to provide the intended exclusivity, our competitors or other third parties could have the freedom to seek regulatory approval of, and to market, products and technologies identical or competitive to ours and we may be required to cease our development and commercialization of certain of our product candidates and technology. In addition, we may seek to obtain additional licenses from Mr. Sinai or a future licensor and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with any product candidates we may develop and our technology. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- our or our licensors' ability to obtain, maintain and defend intellectual property and to enforce intellectual property rights against third parties;
- the extent to which our technology, product candidates and processes infringe, misappropriate or otherwise violate the intellectual property of the licensor that is not subject to the license agreement;
- the sublicensing of patent and other intellectual property rights under our license agreements;
- our diligence, development, regulatory, commercialization, financial or other obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, our license agreement with Mt. Sinai is, and future license agreements are likely to be, complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our diligence, development, regulatory, commercialization, financial or other obligations under the relevant agreement. In addition, if disputes over intellectual property that we have licensed or any other dispute related to our license agreements prevent or impair our ability to maintain our current license agreements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates and technology. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

License agreements we may enter into in the future may be non-exclusive. Accordingly, third parties may also obtain non-exclusive licenses from such licensors with respect to the intellectual property licensed to us under such license agreements. Accordingly, these license agreements may not provide us with exclusive rights to use such licensed patent and other intellectual property rights, or may not provide us with exclusive rights to use such patent and other intellectual property rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and any product candidates we may develop in the future.

Moreover, some of our in-licensed patent and other intellectual property rights may in the future be subject to third party interests such as co-ownership. If we are unable to obtain an exclusive license to such third-party co-owners' interest, in such patent and other intellectual property rights, such third-party co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. We or our licensors may need the cooperation of any such co-owners of our licensed patent and other intellectual property rights in order to enforce them against third parties, and such cooperation may not be provided to us or our licensors.

Additionally, under the terms of the Mt. Sinai License we do not control the preparation, filing, prosecution, maintenance, enforcement and defense of patents and patent applications that we exclusively license. Although we have a right to have our comments considered in connection with patent prosecution and maintenance, we cannot be certain that Mt. Sinai will prepare, file, prosecute and maintain the licensed patents and patent applications in a manner that is consistent with the best interests of with our business. If Mt. Sinai fails to prosecute or maintain or loses rights to any licensed patents or applications, the rights we have licensed may be reduced or eliminated, which could adversely affect our ability to develop and commercialize ACG-801. If we enter into additional licenses in the future with Mt. Sinai or other licensors we may not control the preparation, filing, prosecution, maintenance, enforcement and defense of patents and patent applications that we license from such licensors. It is possible that our licensors' filing, prosecution and maintenance of the licensed patents and patent applications, enforcement of patents against infringers or defense of such patents against challenges of validity or claims of enforceability may be less vigorous than if we had conducted them ourselves, and accordingly, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to file, prosecute, maintain, enforce and defend such patents and patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our technology and any product candidates we may develop that are the subject of such licensed rights could be adversely affected and we may not be able to prevent competitors or other third parties from making, using and selling competing products.

Furthermore, our owned and in-licensed patent rights may be subject to a reservation of rights by the licensor and or one or more third parties. For example, the research resulting in certain of our in-licensed patent rights and technology was funded in part by the U.S. government. Consequently, the U.S. government may have certain rights in to such patents. Certain patents that we exclusively license from Mt. Sinai are the results of research that was funded in part by the U.S. government and certain of our development activities for ACG-701 are funded in part by the U.S. government. When new technologies are developed with government funding, in order to secure ownership of patent rights related to the technologies, the recipient of such funding is required to comply with certain government regulations, including timely disclosing the inventions claimed in such patent rights to the U.S. government and timely electing title to such inventions. A failure to meet these obligations may lead to a loss of rights or the unenforceability of relevant patents or patent applications. In addition, the U.S. government may have certain rights in such patent rights, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology, which may include our confidential information, to third parties and to exercise march-in rights to use or allow third parties to use the technology that was developed using U.S. government funding. The U.S. government may exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such U.S. government-funded inventions may be subject to certain requirements to manufacture any product candidates we may develop embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations and prospects significantly.

***We may not be successful in obtaining all rights necessary to develop and commercialize our current or future product candidates***

We currently have exclusively licensed certain intellectual property from Mt. Sinai that is relevant for ACG-801. Our current and future product candidates may require the use of additional intellectual property rights held by third parties, and our ability to develop and commercialize such product candidates will depend, in part, on our ability to acquire, in-license or use any relevant intellectual property rights. We may be unable to secure such licenses or

otherwise acquire or in-license intellectual property rights from third parties that we identify as necessary for any product candidates we may develop and our technology on commercially reasonable terms, or at all. Even if we are able to in-license any such necessary intellectual property, it could be on non-exclusive terms, thereby giving our competitors and other third parties access to the same intellectual property licensed to us, and the applicable licensors could require us to make substantial licensing and royalty payments. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to third parties, potentially blocking our ability to pursue our research program and develop and commercialize our product candidates.

***Third parties may own or control patents or patent applications and require us to seek licenses, which could increase our development and commercialization costs, or prevent us from developing or marketing products.***

Although we have issued patents and pending patent applications in the U.S. and other countries, we may not have rights under certain third-party patents or patent applications related to our compounds under development and product candidates. Third parties may own or control these patents and patent applications in the U.S. and abroad. In addition, there may be other patents and patent applications related to our current or future product candidates of which we are not aware. Therefore, in some cases, in order to develop, manufacture, sell or import some of our product candidates, we or our collaborators may choose to seek, or be required to seek, licenses under third-party patents issued in the U.S. and abroad or under third-party patents that might issue from U.S. and foreign patent applications. In such an event, we would be required to pay license fees or royalties or both to the licensor. If licenses are not available to us on acceptable terms, we or our collaborators may not be able to develop, manufacture, sell, or import these products, or may be delayed in doing so. Either of these results could have a material adverse effect on our business.

***We may become involved in expensive patent litigation or other proceedings, which could result in our incurring substantial costs and expenses or substantial liability for damages, require us to stop our development and commercialization efforts or result in our patents being invalidated, interpreted narrowly or limited.***

Competitors may infringe our patents or the patents of our licensors. There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the biotechnology industry. We may become a party to various types of patent litigation or other proceedings regarding intellectual property rights from time to time even under circumstances where we are not practicing and do not intend to practice any of the intellectual property involved in the proceedings. In addition to litigation, we may become involved in patent office proceedings, including oppositions, reexaminations, supplemental examinations and *inter partes* reviews involving our patents or the patents of third parties. We may initiate such proceedings or have such proceedings brought against us. An adverse determination in any such proceeding, or in litigation, could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates. An adverse determination in a proceeding involving a patent in our portfolio could result in the loss of protection or a narrowing in the scope of protection provided by that patent.

Even if resolved in our favor, litigation or other proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our scientific, technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could

substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. The cost to us of any patent litigation or other proceeding, even if resolved in our favor, could be substantial. Some of our competitors may be able to sustain the cost of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. If any patent litigation or other proceeding is resolved against us, we or our collaborators may be enjoined from developing, manufacturing, selling, or importing our products without a license from the other party and we may be held liable for significant damages. We may not be able to obtain any required license on commercially acceptable terms or at all. In a patent office proceeding, such as an opposition, reexamination, or *inter partes* review, our patents may be narrowed or invalidated. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace. Patent litigation and other proceedings may also absorb significant management time.

***Our intellectual property may be infringed by a third party.***

Third parties may infringe one or more of our issued patents or trademarks. We cannot predict if, when or where a third party may infringe one or more of our issued patents or trademarks. To counter infringement, we may be required to file infringement claims, which can be expensive and time-consuming. Moreover, there is no assurance that we would be successful in proving that a third party is infringing one or more of our issued patents or trademarks. Any claims we assert against perceived infringers could also provoke these parties to assert counterclaims against us, alleging that we infringe their intellectual property. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly and/or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question, any of which may adversely affect our business. Even if we are successful in proving in a court of law that a third party is infringing one or more of our issued patents or trademarks there can be no assurance that we would be successful in halting their infringing activities.

***If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.***

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, contractors and other parties who have access to such technology and processes. However, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees and consultants who are parties to these agreements breach or violate the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets and third parties could use our trade secrets to compete with any product candidates we may develop and our technology. Additionally, we cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems; however, such systems and security measures may be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors or other third parties. Competitors or third parties could purchase any product candidates we may develop or our technology and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our intellectual property rights or develop their own competitive technologies that fall outside the scope of our intellectual property rights. If any of our trade

secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products, our business, financial condition, results of operations and prospects could be materially and adversely affected.

***Changes in patent laws could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.***

As is the case with other pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the pharmaceutical industry involve technological and legal complexity, and obtaining and enforcing pharmaceutical patents is costly, time-consuming, and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries could increase uncertainties and costs of prosecuting patent applications and the enforcement or defense of patents, and may diminish the value of our patents or narrow the scope of our patent protection. For example, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, or the America Invents Act, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third-party was the first to invent the claimed invention. The America Invents Act also includes a number of significant changes that affect the way patent applications are prosecuted and also may affect patent litigation. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO-administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. However, the America Invents Act and its implementation and any future changes to the patent law could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have an adverse effect on our business, financial condition, results of operations, and prospects. In addition, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce existing patents and patents we may obtain in the future. Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

***We may not be able to protect our intellectual property rights throughout the world.***

Filing, prosecuting and defending patents on our product candidates and any future product candidates throughout the world would be prohibitively expensive, and our or our licensors' intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. In addition, the laws and practices of some foreign countries, particularly those relating to pharmaceuticals, do not protect intellectual property rights to the same extent as federal and state laws in the U.S. For example, novel formulations and methods of medical treatment and manufacturing processes may not be patentable in certain jurisdictions, and the requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our patents, requiring us to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for, and launch generic versions of our products. Many countries, including EU countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under certain circumstances to grant licenses to third parties. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of our patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from our intellectual property.

We may not be able to prevent third parties from practicing our inventions in all countries outside the U.S., or from selling or importing products made using our inventions into or within the U.S. or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own

products, and may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as that in the U.S. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing with us in these jurisdictions.

Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful.

***Patent terms may not adequately protect our competitive position on our product candidates.***

Depending upon the timing, duration and specifics of development, testing and regulatory approval of our product candidates, such as ACG-701 and ACG-801, patents protecting such candidates might expire before or shortly after such candidate is commercialized. We plan to seek extension of patent terms in the United States and in other countries, if available, if we have patents that are eligible for term extension when a product candidate is approved. In the United States the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments, permit the term of a patent to be extended up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it, which is limited to the approved indication, or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review phase, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products and commercialize those products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

***Obtaining and maintaining our patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. We rely on our service providers or our licensors to pay these fees. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We rely on reputable law firms or our licensors for compliance with patent agency requirements. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

***We may be subject to claims by third parties asserting that we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.***

Some of our employees were previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Some of these employees, including each member of our senior management, executed proprietary rights, non-disclosure and non-competition agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in

defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

***We rely on government funding for certain aspects of our research and development activities and we may develop intellectual property through such activities and therefore may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for U.S. based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.***

In August 2021, we entered into a contract with Department of Defense's Defense Threat Reduction Agency (DTRA contract) for the completion of pre-clinical and clinical development activities for ACG-701 for the treatment of melioidosis. We may generate intellectual property rights through the use of this or other U.S. government funding and are therefore subject to certain federal regulations. Also, the research that lead to certain patents and technology that we exclusively license from Mt. Sinai used U.S. government funding. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980 (Bayh-Dole Act), and implementing regulations. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we to disclose the invention to the government and fail to file an application to register the intellectual property in the specified manner and within specified time limits. These time limits have recently been changed by regulation, and may change in the future. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the U.S. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the U.S. or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

***Numerous factors may limit any potential competitive advantage provided by our intellectual property rights.***

The degree of future protection afforded by our intellectual property rights, whether owned or in-licensed, is uncertain because intellectual property rights have limitations, and may not adequately protect our business, provide a barrier to entry against our competitors or potential competitors, or permit us to maintain our competitive advantage. Moreover, if a third party has intellectual property rights that cover the practice of our technology, we may not be able to fully exercise or extract value from our intellectual property rights. The factors that may limit any potential competitive advantage provided by our intellectual property rights include:

- others may be able to make compounds or formulations that are similar to our product candidates but that are not covered by the claims of the patents that we own or control;
- we may not be able to obtain and/or maintain necessary licenses on reasonable terms or at all;
- we or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or control;
- we might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;

- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or control may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the U.S. and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not be able to maintain the confidentiality of our trade secrets or other proprietary information;
- others may independently develop similar or alternative technologies without infringing our intellectual property rights;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

**Risks Relating to Product Manufacturing, Marketing and Sales, and Reliance on Third Parties**

***Even if the compounds we may develop are successful in clinical trials and receive regulatory approvals, we or our collaboration partners may not be able to successfully commercialize them.***

Even if the compounds were successful in clinical development and receive regulatory approvals, it may never reach or remain on the market, be successfully developed into commercial products or gain market acceptance among physicians, patients, healthcare payors or the medical community for a number of reasons including: (i) it may be found ineffective or cause harmful side effects; (ii) it may be difficult to manufacture on a scale necessary for commercialization; (iii) it may experience excessive product loss due to contamination, equipment failure, inadequate transportation or storage, improper installation or operation of equipment, vendor or operator error, natural disasters or other catastrophic events, inconsistency in yields or variability in product characteristics; (iv) it may be uneconomical to produce; (v) the timing of market introduction of the compounds we may develop and competitive products may be inopportune; (vi) political and legislative changes may make the commercialization of any product candidates we may develop in the future, more difficult; (vii) we may fail to obtain reimbursement approvals or pricing that is cost effective for patients as compared to other available forms of treatment or that covers the cost of production and other expenses; (viii) they may not compete effectively with existing or future alternatives; (ix) we may be unable to develop commercial operations and to sell marketing rights; (x) it may fail to achieve market acceptance; or (xi) we may be precluded from commercialization of a product due to proprietary rights of third parties.

***Because we have limited manufacturing experience, and no manufacturing facilities or infrastructure, we are dependent on third-party manufacturers to manufacture product candidates for us.***

We have limited manufacturing experience and no manufacturing facilities, infrastructure or clinical or commercial scale manufacturing capabilities. In order to continue to develop our product candidates, apply for regulatory approvals, and ultimately commercialize products, we need to develop, contract for or otherwise arrange for the necessary manufacturing capabilities. We currently rely upon third parties to produce material for nonclinical and clinical testing purposes and expect to continue to do so in the future. We also expect to rely upon third parties to produce materials that may be required for the commercial production of our product candidates, if approved. Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any product candidates on a timely and competitive basis. We currently do not have any long-term supply contracts.

There are a limited number of manufacturers who operate under the FDA's cGMP regulations capable of manufacturing our product candidates. As a result, we may have difficulty finding manufacturers for our product candidates suitable for our needs. If we are unable to arrange for third-party manufacturing of our product

candidates on a timely basis, or on acceptable terms, we may not be able to complete development of our product candidates or market them.

Any contract manufacturers with which we enter into manufacturing arrangements will be subject to extensive regulatory requirements and ongoing periodic, unannounced inspections or remote regulatory assessments by the FDA, or foreign equivalent, and corresponding state and foreign agencies or their designees to ensure compliance with cGMP requirements and other governmental regulations and corresponding foreign standards. Any failure by our third-party manufacturers to comply with such requirements, regulations or standards could lead to a delay in the conduct of our clinical trials, or a delay in, or failure to obtain, regulatory approval of any of our product candidates. Such failure could also result in sanctions being imposed, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, product seizures or recalls, imposition of operating restrictions, total or partial suspension of production or distribution, or criminal prosecution.

Additionally, contract manufacturers may not be able to manufacture our product candidates at a cost or in quantities necessary to make them commercially viable. Furthermore, changes in the manufacturing process or procedure, including a change in the location where the drug substance or product is manufactured or a change of a third-party manufacturer, may require prior FDA review and approval in accordance with the FDA's cGMP and marketing application regulations and may require that we conduct bridging studies to demonstrate that the product produced under prior processes and procedures or by prior manufacturers is comparable to future product. Contract manufacturers may also be subject to comparable foreign requirements. This review may be costly and time-consuming and could delay or prevent the launch of a product candidate. The FDA or similar foreign regulatory agencies at any time may also implement new standards or change their interpretation and enforcement of existing standards for manufacture, packaging or testing of products. If we or our contract manufacturers are unable to comply, we or they may be subject to regulatory action, civil actions or penalties.

***We have no experience selling, marketing or distributing potential products and no internal capability to do so.***

Advancing compounds through Phase 3 development and regulatory approval will require us to begin commercialization preparation activities and incur related expenses. These activities will include, among other things, the development of an in-house marketing organization and sales force, a market access and payor reimbursement strategy and a distribution function, which will require significant capital expenditures, management resources and time. If we are unable to adequately prepare the market for the potential future commercialization of compounds, we may not be able to generate product revenue once marketing authorization is obtained.

If we are unable or decide not to establish internal sales, marketing and distribution capabilities, we will pursue collaborative arrangements regarding the sales and marketing of our products; however, there can be no assurance that we will be able to establish or maintain such collaborative arrangements on commercially reasonable terms, or if we are able to do so, that they will have effective sales forces. Any revenue we receive will depend upon the efforts of such third parties, which may not be successful. We may have little or no control over the marketing and sales efforts of such third parties and our revenue from product sales may be lower than if we had commercialized our product candidates ourselves. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Finally, regardless of whether we contract out our sales and marketing functions, we will be responsible for the marketing and promotion of our products and may be held responsible should any products be improperly marketed or promoted.

***If third parties on whom we rely for clinical and preclinical trials do not perform as contractually required or as we expect, we may not be able to obtain regulatory approval for or commercialize our drug candidates and our business may suffer.***

We do not have the ability to independently conduct the clinical or preclinical trials required to obtain regulatory approval for our product candidates. We depend on independent investigators, contract research organizations ("CROs"), and other third-party service providers in the conduct of the trials of our product candidates and expect to continue to do so. We expect to contract with CROs, investigators, and other third parties for future clinical and preclinical trials but there is no guarantee that we will be able to at all or on favorable terms. We rely heavily on these parties for successful execution of our trials, but do not control many aspects of their activities. We are responsible for ensuring that each of our trials is conducted in accordance with the applicable regulations and protocols for the trial. Third parties may not complete activities on schedule, or at all, or may not conduct our trials in accordance with regulatory requirements or our protocols. If these third parties fail to carry out their obligations,

we may need to enter into new arrangements with alternative third parties. This could be difficult, costly or impossible. There can be no assurance that we will be able to sustain our relationships with these third-party entities, secure additional partnerships, or identify alternative sites or CROs in the event of any termination of our existing agreements or should we need to establish alternative arrangements. Moreover, if third parties fail to carry out their obligations and comply with the applicable regulatory requirements, our preclinical or clinical trials may need to be extended, delayed, terminated or repeated, and we may not be able to obtain regulatory approval in a timely fashion, or at all, for the applicable product candidate, or to commercialize such product candidate being tested in such trials. If we seek to conduct any of these activities ourselves in the future, we will need to recruit appropriately trained personnel and add to our research, clinical, quality and corporate infrastructure. Moreover, if we need to replace any third parties, we may not be able to do so in a timely fashion or on commercially reasonable terms.

***The commercial success of any product candidates that we may develop will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.***

Any products that we ultimately bring to the market, if they receive marketing approval, may not gain market acceptance by physicians, patients, third-party payors or others in the medical community. If our products do not achieve an adequate level of acceptance, we may not generate product revenue and we may not become profitable. The degree of market acceptance of our products, if approved for commercial sale, will depend on a number of factors, including: (i) the prevalence and severity of any side effects; (ii) the efficacy and potential advantages over alternative treatments; (iii) the ability to offer our product candidates for sale at competitive prices; (iv) relative convenience and ease of administration; (v) the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies; (vi) the strength of marketing and distribution support and the timing of market introduction of competitive products; and (vii) publicity concerning our products or competing products and treatments.

Even if a potential product displays a favorable efficacy and safety profile, market acceptance of the product will not be known until after it is launched. Our efforts to educate patients, the medical community, and third-party payors about our product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by conventional methods used by our competitors.

***If we are unable to obtain adequate reimbursement from third-party payors for any products that we may develop or acceptable prices for those products, our revenues and prospects for profitability will suffer.***

Most patients rely on Medicare, Medicaid, private health insurers, and other third-party payors to pay for their medical needs, including any drugs we may market. If third-party payors do not provide adequate coverage or reimbursement for any products that we may develop, our revenues and prospects for profitability will suffer.

Third-party payors are challenging the prices charged for medical products and services, and many third-party payors limit reimbursement for newly-approved products. These third-party payors may base their coverage and reimbursement on the coverage and reimbursement rate paid by carriers for Medicare beneficiaries. Furthermore, many such payors are investigating or implementing methods for reducing health care costs, such as the establishment of capitated or prospective payment systems. Cost containment pressures have led to an increased emphasis on the use of cost-effective products by health care providers, which could limit the price we might establish for products that we or our current or future collaborators may develop or sell, which would result in lower product revenues or royalties payable to us. In particular, third-party payors may limit the indications for which they will reimburse patients who use any products that we may develop or impose other patient access or utilization controls or limitations.

***We face a risk of product liability claims and may not be able to obtain insurance.***

Our business exposes us to the risk of product liability claims that is inherent in the manufacturing, testing, and marketing of prescription products. We face a risk of product liability exposure related to the testing of our product candidates in clinical trials and will face an even greater risk if we commercially sell any products. Regardless of merit or eventual outcome, liability claims and product recalls may result in: (i) decreased demand for our product candidates and products; (ii) damage to our reputation; (iii) regulatory investigations that could require costly recalls or product modifications; (iv) withdrawal of clinical trial participants; (v) costs to defend related ligation; (vi) substantial monetary awards to clinical trial participants or patients; (vii) loss of revenue; (viii) the diversion of

management's attention away from managing our business; and (ix) the inability to commercialize any products that we may develop.

Although we have product liability and clinical trial liability insurance that we believe is adequate, this insurance is subject to deductibles and coverage limitations. We may not be able to obtain or maintain adequate protection against potential liabilities. If we are unable to obtain insurance at acceptable cost or otherwise protect against potential product liability claims, we will be exposed to significant liabilities, which may materially and adversely affect our business and financial position. These liabilities could also prevent or interfere with our commercialization efforts.

**Risks Relating to Ownership of Our Preferred and Common Stock**

***Provisions in our restated certificate of incorporation ("Charter") and second amended and restated bylaws ("Bylaws") and Delaware law, may prevent a change in control that stockholders may consider desirable.***

Section 203 of the General Corporation Law of the State of Delaware (the "DGCL") and our Charter and Bylaws contain provisions that might enable our management to resist a takeover of our company or discourage a third party from attempting to take over our company. These provisions include: (i) a classified board of directors; (ii) limitations on the removal of directors; (iii) limitations on stockholder proposals at meetings of stockholders; (iv) the inability of stockholders to act by written consent or to call special meetings; and (v) the ability of our board of directors to designate the terms of and issue new series of preferred stock without stockholder approval. These provisions could: (i) have the effect of delay, defer, or prevent a change in control of us or a change in our management that stockholders may consider favorable or beneficial or (ii) discourage proxy contests and make it more difficult for stockholders to elect directors and take other corporate actions.

***The Company's Bylaws provide, to the fullest extent permitted by law, that the Court of Chancery of the State of Delaware will be the exclusive forum for certain legal actions between the Company and its stockholders, which could increase costs to bring a claim, discourage claims or limit the ability of the Company's stockholders to bring a claim in a judicial forum viewed by the stockholders as more favorable for disputes with the Company or the Company's directors, officers or other employees.***

Our Bylaws provide to the fullest extent permitted by law that unless the Company consents in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any (i) derivative action or proceeding brought on behalf of the Company, (ii) any action asserting a claim of breach of a fiduciary duty owed by any director, officer, other employee or stockholder of the Company to the Company or its stockholders, (iii) any action arising pursuant to any provision of the DGCL, the Company's Charter or the Bylaws, (iv) any action to interpret, apply, enforce or determine the validity of the Charter or the Bylaws, or (v) any action asserting a claim governed by the internal affairs doctrine. The choice of forum provision may increase costs to bring a claim, discourage claims or limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with the Company or its directors, officers or other employees, which may discourage such lawsuits against the Company or its directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in the Company's Bylaws to be inapplicable or unenforceable in an action, the Company may incur additional costs associated with resolving such action in other jurisdictions. The exclusive forum provision in our Bylaws would not apply to claims brought under the Exchange Act or the Securities Act, or any other claim for which the federal courts have exclusive jurisdiction. Additionally, such provision will not relieve us of our duty to comply with the federal securities laws and the rules and regulations thereunder, and stockholders will not be deemed to have waived our compliance with these laws, rules and regulations.

***Six stockholders beneficially own approximately 69% of our outstanding common stock in the aggregate. If these significant stockholders choose to act together, they could exert substantial influence over our business, and the interests of these stockholders may conflict with those of other stockholders.***

Following the Series Z Preferred Stock Conversion (as defined below) there is a concentration of ownership of our outstanding common stock because approximately 69% of our outstanding common stock is beneficially owned by six stockholders. As of March 1, 2023: (i) entities affiliated with Pillar Invest Corporation (the "Pillar Investment Entities") beneficially owned approximately 11.2% of our outstanding common stock; (ii) NovaQuest beneficially owned approximately 9.8% of our outstanding common stock; (iii) Dr. Atul Chopra, a founder and a member of Legacy Aceragen's board of directors, beneficially owned approximately 17.5% of our outstanding common stock; and (iv) Mr. John Taylor, Mr. Daniel Salain, and Mr. Andrew Jordan ("Executives") individually beneficially owned

17.5%, 17.5% and 5.4% of our common stock, respectively ("Pillar Investment Entities" and, together with NovaQuest, Dr. Chopra, and Executives, the "Significant Securityholders"). If any of our Significant Securityholders acted together, they could be able to exert substantial influence over our business. Additionally, the interests of the Significant Securityholders may be different from or conflict with the interests of our other stockholders. This concentration of voting power with the Significant Securityholders could delay, defer or prevent a change of control, entrench our management and the board of directors or delay or prevent a merger, consolidation, takeover or other business combination involving us on terms that other stockholders may desire. In addition, conflicts of interest could arise in the future between us, on the one hand, and either of our Significant Securityholders on the other hand, concerning potential competitive business activities, business opportunities, the issuance of additional securities and other matters.

***The issuance or sale of shares of our common stock could depress the trading price of our common stock.***

If (i) we issue additional shares of our common stock or rights to acquire shares of our common stock in other future transactions, (ii) any of our existing stockholders sells a substantial amount of our common stock, or (iii) the market perceives that such issuances or sales may occur, then the trading price of our common stock may significantly decrease. In addition, our issuance of additional shares of common stock will dilute the ownership interests of our existing common stockholders.

***Because we do not intend to pay dividends on our common stock, investor returns will be limited to any increase in the value of our stock.***

We have never declared or paid any cash dividends on our common stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business and do not anticipate declaring or paying any cash dividends on our common stock for the foreseeable future.

***Our Series X Preferred Stock have rights, preferences and privileges that are not held by, and are preferential to, the rights of our common stock, which could adversely affect our liquidity and financial condition, and may result in the interests of the holders of our Series X Preferred Stock differing from those of the holders of common stock.***

The Series X Preferred Stock ranks senior to our common stock with respect to dividend rights on the distribution of assets on any voluntary or involuntary liquidation, dissolution, or winding up of our affairs. The holders of our Series X Preferred Stock are entitled to receive distributions on shares of Series X Preferred Stock as set forth in (a) the Purchase Agreement, and (b) the PRV Agreement (any such distributions under the Purchase Agreement and the PRV Agreement, the "Preferred Distributions"), prior and in preference to any declaration or payment of any other distribution or dividend (other than dividends on shares of Common Stock payable in shares of Common Stock).

In addition, holders of Series X Preferred Stock are entitled to receive a distribution in the event either (i) Aceragen receives any proceeds from the sale of a PRV granted by the FDA in connection with regulatory approval of a ACG-801 (recombinant human acid ceramidase) or ACG-701 (sodium fusidate) product or (ii) Aceragen does not receive such a PRV or does not complete a PRV sale within a certain period after receipt. The holders of Series X Preferred Stock are also entitled to net sales distributions based upon future net sales of the ACG-801 and ACG-701.

The holders of our Series X Preferred Stock also have the right, subject to certain exceptions, to require us to repurchase all or any portion of the Series X Preferred Stock upon certain change of control events or Product Divestiture (as defined in the PRV Agreement) of a ACG-801 product without NovaQuests' consent with respect to certain change of control events or Product Divestiture (as defined in the PRV Agreement), and Aceragen may, and NovaQuest may require us to, redeem the Series X Preferred Stock at a price equal to the fair market value thereof or make certain distributions to the holders of Series X Preferred Stock.

These dividend, distribution, and share repurchase obligations could impact our liquidity and reduce the amount of cash flows available for general corporate purposes. Our obligations to the holders of the Series X Preferred Stock could also limit our ability to obtain additional financing or increase our borrowing costs, which could have an adverse effect on our financial condition. These preferential rights could also result in divergent interests between the holders of shares of Series X Preferred Stock and holders of our common stock.

## Item 1B. Unresolved Staff Comments.

None.

## Item 2. Properties.

We lease approximately 11,000 square feet of office space located in Exton, Pennsylvania. The lease expires on May 31, 2025. We may terminate the lease at any point as long as we remain a member of the landlord's group and require a space with more square footage. We have specified rights to sublease this facility.

In connection with the Aceragen Acquisition, we acquired an operating lease for an office in Basel, Switzerland which expired on March 31, 2023.

## Item 3. Legal Proceedings.

None.

## Item 4. Mine Safety Disclosures.

Not applicable.

# PART II.

## Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

**Market Information**

Our common stock is listed under the symbol "ACGN" on the Nasdaq.

**Holders of Record**

As of March 31, 2023, we had 27 common stockholders of record registered on our books, excluding shares held through banks and brokers.

**Dividends**

We have never declared or paid cash dividends on our common stock, and we do not expect to pay any cash dividends on our common stock in the foreseeable future. The declaration and payment of dividends in the future, of which there can be no assurance, will be determined by our board of directors in light of conditions then existing, including earnings, financial condition, capital requirements, and other factors.

**Recent Sales of Unregistered Securities**

Pursuant to the Merger Agreement (as defined below), on September 28, 2022, the Company issued 451,608 shares of Common Stock and 80,656 Series Z Preferred Stock to the common stockholders of Legacy Aceragen and 5 shares of Series X Preferred Stock to NovaQuest. Under the terms of the Merger Agreement, all options to purchase or acquire shares of Legacy Aceragen held by Continuing Employees (as defined in the Merger Agreement) and all issued and outstanding warrants to purchase shares of Legacy Aceragen's capital stock were assumed by the Company and converted into options or warrants to purchase shares of Common Stock and Series Z Preferred Stock on the same terms and conditions as applied to such options and warrants immediately prior to the Merger (but with such changes as the Company in good faith determined were necessary to reflect such assumption and conversion).

Such issuances were exempt from registration under the Securities Act, in reliance on Section 4(a)(2) thereof, and Regulation D promulgated thereunder. Each of the common stockholders of Legacy Aceragen and NovaQuest represented that it was an "accredited investor," as defined in Regulation D, and is acquiring the securities for investment only and not with a view towards, or for resale in connection with, the public sale or distribution thereof. The securities have not been registered under the Securities Act and such securities may not be offered or sold in the United States absent registration or an exemption from registration under the Securities Act and any applicable state securities laws.

As provided for the Series Z Certificate of Designation, following stockholder approval of the Conversion Proposal (as defined below), each share of Series Z Preferred Stock then outstanding automatically converted into a number of shares of Common Stock equal to the Conversion Ratio (as defined in the Series Z Certificate of Designation). Such conversion was subject to certain limitations, including that the Company was not permitted to effect any conversion of shares of Series Z Preferred Stock into shares of Common Stock if, as a result of such conversion, such holder, together with its affiliates, would beneficially own more 19.99% of the total number of shares of Common Stock issued and outstanding immediately after giving effect to such conversion.

**Issuer Purchases of Equity Securities**

We did not repurchase any of our equity securities during the year ended December 31, 2022.

## Item 6. [Reserved.]

## Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

*The following discussion and analysis of our financial condition and results of operations should be read together with our consolidated audited financial statements and accompanying notes appearing elsewhere in this Form 10-K. In addition to historical information, this discussion and analysis includes forward-looking statements that are subject to risks and uncertainties, including those discussed in the section titled "Risk Factors," set forth in Part I, Item 1A of this Form 10-K, that could cause actual results to differ materially from historical results or anticipated results.*

*Prior to January 17, 2023, we were known as Idera Pharmaceuticals, Inc. On September 28, 2022, we completed the Aceragen Acquisition, whereby we acquired all of the outstanding equity interests in Legacy Aceragen. In connection with the Aceragen Acquisition and related transactions, we changed our name to Aceragen, Inc. Unless the context indicates otherwise, references in this section to the "Company," "Aceragen," "Idera," "we," "us," "our" and similar terms refer to Aceragen, Inc. (f/k/a Idera Pharmaceuticals, Inc.) and our consolidated subsidiaries. References to "Legacy Aceragen" refer to Aceragen, Inc. prior to the consummation of the Aceragen Acquisition.*

### Overview

We are a clinical-stage biopharmaceutical company with a business strategy focused on the clinical development, and ultimately the commercialization, of product candidates for rare disease indications characterized by small, well-defined patient populations with serious unmet medical needs. Our current focus is to develop and optimize commercial value of ACG-701 (patented formulation of sodium fusidate) and ACG-801 (recombinant human acid ceramidase (rhAC)) for appropriate patients. We have in the past and may in the future explore collaborative alliances to support development and commercialization of any of our drug candidates. We may also seek to identify and potentially acquire rights to novel development and commercial-stage rare disease programs through new business development opportunities, including additional strategic alternatives.

Until December 2021, we were developing tilsotolimod, via intratumoral injection, for the treatment of solid tumors in combination with nivolumab, an anti-PD1 antibody marketed as Opdivo® by Bristol Myers Squibb Company ("BMS"), and/or ipilimumab, an anti-CTLA4 antibody marketed as Yervoy® by BMS. Due to Phase 3 results in anti-PD-1 refractory advanced melanoma (ILLUMINATE-301), reported in March 2021, which showed the study failed to meet its primary endpoint, as well as a decision in December 2021 to discontinue enrollment in ILLUMINATE-206, our Phase 2 study in solid tumors, Company-sponsored development of tilsotolimod in oncology was discontinued. Although clinical trials with tilsotolimod have not yet translated into a new treatment alternative for patients, we believe that data supporting tilsotolimod's mechanism of action and encouraging safety profile from across the array of pre-clinical and clinical work to date, together with its intellectual property protection, are noteworthy. As a result, in December 2021, we announced that we would consider an out-licensing arrangement so that tilsotolimod's full potential might continue to be explored on behalf of patients who did not respond to traditional immunotherapy, together with other alternatives.

In September 2022, we acquired Legacy Aceragen, a privately-held biotechnology company addressing rare, orphan pulmonary, and rheumatic diseases for which there are limited or no available treatments. Legacy Aceragen owned or controlled the intellectual property related to ACG-701 (patented formulation of sodium fusidate) and ACG-801 (recombinant human acid ceramidase (rhAC)). Following the Aceragen Acquisition, our business strategy is to develop and optimize commercial value of ACG-701 and ACG-801 for appropriate patients. Accordingly, we are developing ACG-701 to treat cystic fibrosis ("CF") pulmonary exacerbations ("PEx") and melioidosis, a severe, life-threatening infection, and ACG-801 to treat patients suffering from a genetic mutation in the ASAH 1 gene, also known as Farber disease.

### Recent Developments

***Business Acquisition***

On the Acquisition Date, in accordance with the Merger Agreement, we acquired 100% of the outstanding security interests of Legacy Aceragen in a "stock-for-stock" transaction, whereby all Legacy Aceragen outstanding equity interests were exchanged for a combination of shares of our common stock, shares of Series Z Preferred Stock,

and shares of the newly-designated Series X Preferred Stock. Under the terms of the Merger Agreement, Legacy Aceragen stockholders received (i) 451,608 shares of our common stock (inclusive of shares subject to repurchase), (ii) 80,656 shares of Series Z Preferred Stock (inclusive of shares subject to repurchase), and (iii) five shares of Series X Preferred Stock. In addition, all outstanding options and warrants to purchase Legacy Aceragen common stock were converted into stock options and warrants to purchase shares of our common stock and Series Z Preferred Stock on terms substantially identical to those in effect prior to the Aceragen Acquisition, except for adjustments to the underlying number of shares and the exercise price based on the Merger Agreement exchange ratio.

Pursuant to the Merger Agreement, we held the Special Meeting at which our stockholders approved, among other matters: the Conversion Proposal and (ii) the Reverse Stock Split Proposal. The transactions following the approval of the Merger Agreement Meeting Proposals are discussed in further detail below.

***Reverse Stock Split and Corporate Name Change***

Following approval of the Reverse Stock Proposal, our board of directors approved a one-for-seventeen (1:17) reverse split of our issued and outstanding shares of common stock (the "Reverse Stock Split"). On January 17, 2023, we filed with the Secretary of State of the State of Delaware a Certificate of Amendment to our Restated Certificate of Incorporation (the "Certificate of Amendment") to effect the Reverse Stock Split and the Company Name Change (as defined below). The Reverse Stock Split became effective as of 4:59 p.m. Eastern Time on January 17, 2023. As a result of the effectiveness of the Reverse Stock Split, every 17 shares of our issued and outstanding common stock were automatically combined, converted, and changed into one share of our common stock, without any change in the number of authorized shares or the par value per share. In addition, a proportionate adjustment was made to the per share exercise price and the number of shares issuable upon the exercise of all outstanding stock options, restricted stock units, and warrants to purchase shares of common stock and the number of shares reserved for issuance pursuant to our equity incentive compensation plans.

Also on January 17, 2023, and in connection with the previously-announced merger between Legacy Aceragen and us, our board of directors approved a change in name from "Idera Pharmaceuticals, Inc." to "Aceragen, Inc." (the "Company Name Change"), as reflected in the Certificate of Amendment. The Company Name Change became effective as of 4:59 p.m. Eastern Time on January 17, 2023.

***Conversion of Series Z Non-Voting Convertible Preferred Stock***

Following approval of the Conversion Proposal, effective January 17, 2023 at 5:00 p.m. Eastern Time, all 80,656 outstanding shares of our Series Z Preferred Stock were automatically converted into 4,744,467 shares of our common stock pursuant to the terms of the Series Z Preferred Stock (the "Series Z Preferred Stock Conversion").

***Nasdaq Compliance***

As previously disclosed by Current Reports on Form 8-K filed with the SEC on December 1, 2021, May 27, 2022, and November 23, 2022, the Company received deficiency letters from the Nasdaq Listing Qualifications Department (the "Staff") of The Nasdaq Stock Market, LLC ("Nasdaq"), notifying the Company that it was not in compliance with Nasdaq Listing Rule 5550(a)(2), which requires the Company to maintain a minimum closing bid price of $1.00 per share for continued listing on The Nasdaq Capital Market. In response to the Staff's letter, in November 2022, the Company timely requested a hearing before a Nasdaq Hearing Panel (the "Panel"), which was held on January 5, 2023. By decision dated January 10, 2023, the Panel granted the Company an extension until January 20, 2023, to complete the Special Meeting and attendant transactions, including the Reverse Stock Split, and thereby evidence compliance with all applicable criteria for continued listing on The Nasdaq Capital Market, including the initial listing $4.00 bid price requirement applicable due to the Series Z Preferred Stock Conversion, which constituted a "change of control" (as that term is defined by Nasdaq), requiring the Company to meet all criteria for initial listing on The Nasdaq Capital Market at that time. Following the Reverse Stock Split, which was effective upon the market open on Wednesday, January 18, 2023, the Company was in compliance with all applicable Nasdaq listing standards and Nasdaq issued an approval letter confirming Aceragen's listing.

***Cost-reduction Plan Implementation***

On April 13, 2023, the Board approved certain cost-cutting measures with a view to preserving capital to support our continuing operations. As part of this plan, we have commenced the furlough of 12 employees, representing approximately 46% of our workforce. Additionally, certain of our employees and executive officers will defer portions of their respective base salaries in amounts that exceed $200,000, with such deferrals having a retroactive effective date of April 5, 2023. We will continue to review operations for other opportunities to reduce costs and pursue financing opportunities. For more information, please see Item 9B of this Form 10-K.

## Results of Operations

*The following is a discussion of results of operations for fiscal 2022 compared to fiscal 2021. For a discussion of results of operations for fiscal 2021 compared to fiscal 2020, please refer to Item 7—Management's Discussion and Analysis of Financial Condition and Results of Operations in the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on March 31, 2022.*

### *Years ended December 31, 2022 and 2021*

***Overview***

During each of the years ended December 31, 2022 and 2021 our loss from operations totaled $27.8 million.

Prior to the Aceragen Acquisition, all our revenues had been from collaboration and license agreements, although we did not generate any such revenue in 2021 and have received no revenues from the sale of commercial products. Additionally, research and development expenses historically comprised the majority of our total operating expenses until we terminated our ILLUMINATE development program in December 2021, resulting in general and administrative expenses, acquisition-related costs and restructuring costs to comprise the majority of our total operating expenses for the year ended December 31, 2022. Following the Aceragen Acquisition, we expect to generate revenues from certain U.S. government contracts and, assuming adequate funding, for research and development expenses to comprise the majority of our total operating expenses, including in 2023 and beyond.

The following summarizes the components of loss from operations, as discussed further below:

| | Year Ended December 31, | | | |
|---|---|---|---|---|
| *($ in thousands)* | 2022 | 2021 | $ Change | % Change |
| Government contracts revenue | $ 4,862 | $ — | $ 4,862 | 100% |
| Operating expenses: | | | | |
| Research and development | 12,188 | 16,375 | (4,187) | (26)% |
| General and administrative | 12,213 | 9,976 | 2,237 | 22% |
| Acquisition-related costs | 4,566 | — | 4,566 | 100% |
| Restructuring and other costs | 3,713 | 1,322 | 2,391 | 181% |
| Total operating expenses | 32,680 | 27,673 | 5,007 | 18% |
| Loss from operations | $ (27,818) | $ (27,673) | $ (145) | 1% |

***Government Contracts Revenue***

In connection with the Aceragen Acquisition, we assumed certain U.S. government contracts. Revenues from reimbursable contracts are recognized as costs are incurred, generally based on allowable direct costs incurred during the period, plus allocable overheads together with any recognizable earned fee.

Government contracts revenue for the year ended December 31, 2022 totaled $4.9 million of which $4.6 million related to a contract assumed in the Aceragen Acquisition funded by the Department of Defense's DTRA to develop ACG-701 as a potential medical countermeasure against the pathogen that causes melioidosis, B. Pseudomallei. This contract is expected to further fund clinical and regulatory development of ACG-701 up to an additional $30.0 million. Our other government contracts are not currently significant to our operations and not expected to

be material in the future. We did not generate any government contracts revenue for the fiscal year ended December 31, 2021.

***Research and Development Expenses***

For each of our research and development programs, we incur both direct and indirect expenses. We track direct research and development expenses by program, which may include internal personnel costs and other third-party costs such as contract research, consulting and clinical trial and manufacturing costs. We do not allocate indirect research and development expenses, which may include regulatory, laboratory (equipment and supplies), personnel, facility, and other overhead costs (including depreciation and amortization), to specific programs.

During the fiscal year ended December 31, 2022, our overall research and development expenses declined by 26%, as compared to 2021, primarily due to decreases in external development costs associated with tilsotolimod (IMO-2125) and other drug development expenses. These decreases are primarily attributed to (i) lower costs incurred with contract research organizations supporting our ILLUMINATE development program as a result of our decision to discontinue development of tilsotolimod, (ii) lower costs incurred with drug manufacturing activities, and (iii) lower expenses incurred in connection with the Scriptr Agreement.

We also expect that our research and development costs will increase in future periods as we proceed with the development of ACG-701 and ACG-801, however, such development will depend on our ability to raise capital, as discussed further below under the caption “Liquidity and Capital Resources.”

In the table below, research and development expenses are set forth in the following categories: (i) ACG-701 (REPRIEVE Study (CF PEx)) external development expense, (ii) ACG-701 (TERRA Study (Melioidosis)) external development expense, (iii) ACG-801 (Farber disease) external development expense, (iv) Tilsotolimod (IMO-2125), and (v) other drug development expenses.

| | Year Ended December 31, | | | |
|---|---|---|---|---|
| ***($ in thousands)*** | **2022** | **2021** | **$ Change** | **% Change** |
| ACG-701 development expense | | | | |
| REPRIEVE Study (CF PEx) | $ 673 | $ — | $ 673 | 100% |
| TERRA Study (Melioidosis) | 3,128 | — | 3,128 | 100% |
| Subtotal | 3,801 | — | 3,801 | 100% |
| ACG-801 development expense (Farber disease) | 1,684 | — | 1,684 | 100% |
| Tilsotolimod (IMO-2125) development expense | 3,234 | 9,247 | (6,013) | (65)% |
| Other drug development expense | 3,469 | 7,128 | (3,659) | (51)% |
| Total research and development expenses | $ 12,188 | $ 16,375 | (4,187) | (26)% |

*ACG-701 Development Expenses*

These expenses are comprised of expenses we incurred in connection with the development of ACG-701 for CF PEx and Melioidosis, including our ongoing REPRIEVE and TERRA Studies, and include external development expenses incurred with contract research organizations, contract development and manufacturing organizations, subcontractors, and other third-party vendors. In addition, these expenses include salary costs, but exclude other internal personnel-related costs, such as stock-based compensation and other benefits, and overhead expenses.

We acquired the ACG-701 development program in connection with the Aceragen Acquisition and began to incur program-related expenses following the Acquisition Date. We expect to continue to incur significant expenses related to the development of ACG-701 in 2023 and beyond, subject to adequate financing.

*ACG-801 Development Expenses*

These expenses are comprised of expenses we incurred in connection with the development of ACG-801 for Farber disease, including our anticipated ADVANCE Study, which we expect to initiate clinical activities for in the first half of 2024, subject to funding. Such expenses include external development expenses incurred with contract research organizations, contract development and manufacturing organizations, subcontractors, and other third-

party vendors. In addition, these expenses include salary costs, but exclude other internal personnel-related costs, such as stock-based compensation and other benefits, and overhead expenses.

We acquired the ACG-801 development program in connection with the Aceragen Acquisition and began to incur program-related expenses following the Acquisition Date. We expect to continue to incur significant expenses related to the development of ACG-801 in 2023 and beyond, subject to adequate financing.

*Tilsotolimod (IMO-2125) Development Expenses*

These expenses include external expenses we have incurred in connection with the development of tilsotolimod, as part of our ILLUMINATE development program, which we commenced clinical development of in July 2015 but was discontinued in December 2021. These external expenses include payments to independent contractors and vendors for drug development activities conducted after the initiation of tilsotolimod clinical development in immuno-oncology but exclude internal costs such as salaries and other personnel-related costs and overhead expenses.

Following the announcement that all Company-sponsored development of tilsotolimod was discontinued in December 2021, all significant study-related activities concluded. As such, we do not anticipate incurring significant additional expenses related to tilsotolimod in 2023 and beyond.

*Other Drug Development Expenses*

These expenses include internal costs, such as salary and other personnel-related costs and overhead expenses not allocated to a specific development program. In addition, these expenses include costs incurred related to our research collaboration with Scriptr and other external expenses, such as payments to contract vendors, associated with compounds that were previously being developed but are not currently being developed, other than tilsotolimod. For the years ended December 31, 2022 and 2021, we incurred $3.5 million and 7.1 million, respectively, of other drug development expenses. The decrease in other drug development expenses during 2022, as compared to 2021, was primarily due to (i) lower expenses incurred pursuant to the Scriptr Agreement of $1.6 million, and (ii) lower personnel-related costs resulting from the April 2021 reduction-in-force (discussed below), which primarily impacted research and development personnel as we were focused in 2022 on strategic alternatives, which resulted in the Aceragen Acquisition.

***General and Administrative Expenses***

General and administrative expenses consist primarily of payroll, stock-based compensation expense, consulting fees, and professional legal fees associated with our patent applications and maintenance, our corporate regulatory filing requirements, our corporate legal matters, and our business development initiatives.

For the year ended December 31, 2022, general and administrative expenses totaled $12.2 million, a 22% increase, as compared to $10.0 million for the year ended December 31, 2021. The increase in general and administrative expenses during 2022, as compared to 2021, was primarily due to increases in: (i) personnel costs related to the acquisition of Legacy Aceragen employees (including salaries, stock-based compensation and bonuses), (ii) professional and consulting fees (including accounting and legal costs), and (iii) other overhead costs.

***Acquisition-related Costs***

Acquisition-related costs consist of charges for transaction, integration-related professional fees, retention bonuses and other incremental costs directly related to these activities.

Acquisition-related costs for the year ended December 31, 2022 was $4.6 million. All acquisition-related costs related to the Aceragen Acquisition and primarily consisted of legal and transaction related fees, and retention bonuses to certain employees. No such costs were incurred during 2021.

***Restructuring and Other Costs***

In April 2021, following the announcement that the ILLUMINATE-301 trial did not meet its primary endpoint of ORR, we implemented a reduction-in-force, which affected approximately 50% of our workforce through December 31,

2021, primarily in the area of research and development. The decision was made in order to align our workforce with its needs in light of the outcome of ILLUMINATE-301's ORR endpoint, its ongoing ILLUMINATE development program, and other business development activities focused on identifying new portfolio opportunities. In September 2022, in connection with the Aceragen Acquisition, we restructured our operations and implemented a reduction-in-force which affected approximately 54% of our pre-Aceragen Acquisition workforce.

For the year ended December 31, 2022, restructuring and other costs totaled $3.7 million, a 185% increase, compared to $1.3 million for the year ended December 31, 2021. The increase in restructuring costs during 2022, as compared to 2021, was primarily due to increases in severance and related benefits resulting from the composition of the workforce effected from the reduction-in-force implemented in September 2022, which included several executives, in connection with the Aceragen Acquisition.

***Interest Income (Expense), net***

Interest income, net of interest expense (which included non-cash charges for accretion of discounts on the Acquisition Obligation of approximately $0.1 million), for the year ended December 31, 2022 totaled $0.2 million. Interest income, net of interest expense, for the year ended December 31, 2021 was not material. The increase in 2022, as compared to 2021, was primarily due to higher interest rates.

Amounts may fluctuate from period to period due to changes in average investment balances, money market funds classified as cash equivalents, and composition of investments.

***Warrant Revaluation Gain***

During the years ended December 31, 2022 and 2021, we recorded a non-cash warrant revaluation gain of approximately $0.4 million and $7.0 million, respectively.

The non-cash gain for the fiscal year ended December 31, 2022 related to the change in fair value of our liability classified warrants assumed in connection with the Aceragen Acquisition in September 2022. Due to the nature of and inputs in the model used to assess the fair value of our outstanding warrants, it is not abnormal to experience significant fluctuations during each remeasurement period. These fluctuations may be due to a variety of factors, including changes in our stock price and changes in estimated stock price volatility over the remaining life of the warrants. Warrant revaluation loss for 2022 was driven primarily by a decrease in our stock price during the period.

The non-cash gain for the fiscal year ended December 31, 2021 related to the derecognition of the warrant liability in the first quarter of 2021 due to the termination of such liability-classified warrants that were issued pursuant to the December 2019 Securities Purchase Agreement as more fully described in Note 9 of the notes to condensed consolidated financial statements appearing elsewhere in this Form 10-K .

***Series X Preferred Stock Liability Loss***

During the year ended December 31, 2022, we recorded a non-cash Series X Preferred Stock liability loss of approximately $2.4 million. No such gain or loss was recorded during the year ended December 31, 2021.

The non-cash loss for the year ended December 31, 2022 related to the change in fair value of our liability-classified Series X Preferred Stock, which was issued in connection with the Aceragen Acquisition in September 2022.

***Future Tranche Right Revaluation Gain or Loss***

During the year ended December 31, 2021, we recorded a non-cash future tranche right revaluation gain of approximately $118.8 million. No such gain or loss was recorded during the year ended December 31, 2022.

The non-cash gain for the year ended December 31, 2021 related to the derecognition of the future tranche right liability during the first quarter of 2021 associated with the future tranche rights issued pursuant to the December 2019 Securities Purchase Agreement, as more fully described in Note 9 of the notes to condensed consolidated financial statements appearing elsewhere in this Form 10-K, due to the termination of the future tranche rights.

***Foreign Currency Exchange and Other Gain (Loss), net***

During each of the years ended December 31, 2022 and 2021, we recorded a net foreign currency exchange and other loss of less than $0.1 million. Such gains and losses, net, are not material to our business and not expected to be material in the foreseeable future.

***Income Tax Benefit***

During the year ended December 31, 2022, we recorded $6.3 million in non-cash income tax benefit related to our evaluation of the realizability of our deferred tax assets that we determined that the valuation allowance should be decreased in consideration of positive and negative evidence bearing upon our ability to realize certain of our deferred tax assets.

There was no income tax benefit or expense recorded during the year ended December 31, 2021.

***Net Income or Loss to Common Stockholders***

As a result of the factors discussed above, we incurred a net loss of $23.4 million for the year ended December 31, 2022, compared to net income of $98.1 million for the year ended December 31, 2021.

Basic net loss applicable to common stockholders for the year ended December 31, 2022 was $23.4 million, as compared to basic net income applicable to common stockholders for the year ended December 31, 2021 of $96.9 million. Excluding the non-cash warrant revaluation gain of $7.0 million and future tranche right revaluation gain of $118.8 million for the year ended December 31, 2021, basic net loss applicable to common stockholders was $28.8 million.

Diluted net loss applicable to common stockholders for the year ended December 31, 2022 was $23.4 million, as compared to diluted net loss applicable to common stockholders for the year ended December 31, 2021 of $28.8 million.

***Net Operating Loss Carryforwards***

As of December 31, 2022, the Company had cumulative federal, various state, and Switzerland net operating loss carryforwards (“NOLs”) of approximately $355.8 million, $362.7 million, and $0.9 million, respectively, available to reduce federal and state taxable income, respectively. As a result of the Tax Cuts and Jobs Act of 2017, federal net operating losses incurred for taxable years beginning after January 1, 2018 have an unlimited carryforward period, but can only be utilized to offset 80% of taxable income in future taxable periods. Of the $355.8 million of federal NOLs, $158.4 million have an unlimited carryforward and the remaining NOLs are subject to expiration through 2037. In addition, at December 31, 2022, the Company had cumulative federal and state tax credit carryforwards of $28.3 million and $1.9 million, respectively. The federal credits expire through 2042 and the state credits expire through 2033.

Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, prescribe limitations on the amount of NOLs and tax credit carryforwards that may be utilized in any one year. Under Internal Revenue Code Section 382, if a corporation undergoes an “ownership change,” the corporation’s ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income may be limited. In December 2017, the Company completed a study which determined that ownership changes had occurred. The ownership changes have and will continue to subject the Company’s pre-ownership change NOL carryforwards to an annual limitation, which will significantly restrict the Company’s ability to use them to offset taxable income in periods following the ownership change. The federal and state net operating loss and tax credit carryforwards and related deferred tax assets discussed above and included in Note 16 to the consolidated financial statements appearing elsewhere in this Form 10-K have been adjusted to reflect the limitations that resulted from this study. As no study has been completed subsequent to 2017, additional ownership change limitations may result from ownership changes that have occurred, or may occur in the future. In conjunction with the Aceragen Acquisition, the Company acquired Legacy Aceragen’s federal, various state, and Switzerland NOL’s of $8.1 million, $19.1 million, and $0.8 million, respectively.

## Financial Condition, Liquidity and Capital Resources

***Financial Condition***

As of December 31, 2022, we had an accumulated deficit of $758.8 million. As of the date of this Form 10-K, substantially all our revenues have been from collaboration and license agreements and a contract with the U.S. government that we assumed in the Aceragen Acquisition, and we have received no revenues from the sale of commercial products.

We have devoted substantially all our efforts to research and development, including clinical trials, and we have not completed development of any commercial products. Our research and development activities, together with our general and administrative expenses, are expected to continue to result in substantial operating losses for the foreseeable future. These losses, among other things, have had and will continue to have an adverse effect on our stockholders' equity, total assets, and working capital. Due to the numerous risks and uncertainties associated with developing drug candidates, and if approved, commercial products, we are unable to predict the extent of any future losses, whether or when any of our drug candidates will become commercially available or when we will become profitable, if at all.

***Liquidity and Capital Resources***

*Overview*

We require cash to fund our operating expenses and to make capital expenditures. Historically, we have funded our cash requirements primarily through the following:

(i) sale of common stock, preferred stock and future tranche rights, and warrants (including pre-funded warrants);

(ii) exercise of warrants;

(iii) debt financing, including capital leases;

(iv) license fees, research funding and milestone payments under collaborative and license agreements, and clinical funding arrangements, including reimbursements under U.S. Government funded programs; and

(v) interest income.

*SVB*

On March 10, 2023, Silicon Valley Bank ("SVB"), at which we maintain cash and cash equivalents in multiple accounts, was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation (FDIC) as receiver. The failure of SVB exposed us to liquidity and credit risk prior to the completion of the FDIC resolution of SVB in a manner that fully protects all depositors. As a result, we do not anticipate any losses with respect to our funds that had been deposited with SVB.

*ATM Agreement*

In November 2018, we entered into an Equity Distribution Agreement (the "ATM Agreement") with JMP Securities LLC ("JMP") pursuant to which we may issue and sell shares of our common stock having an aggregate offering price of up to $50.0 million through JMP as our agent.

During the year ended December 31, 2021, we sold 301,021 shares of common stock pursuant to the ATM Agreement resulting in net proceeds, after deduction of commissions and other offering expenses, of $15.3 million. No shares were sold during the year ended December 31, 2022. As of March 31, 2023, we may sell up to an additional $19.5 million of shares under the ATM Agreement

*Funding Requirements*

We had cash and cash equivalents of approximately $12.0 million at December 31, 2022. We believe based on our current operating plan, our existing cash and cash equivalents on hand as of December 31, 2022 will enable us to fund our operations into May 2023. Accordingly, we need to raise additional capital to continue to fund our operations and service our obligations in the future. If we are unable to raise additional capital when needed, we will not be able to continue as a going concern. We do not currently have any products approved for sale and do not generate any revenue from product sales. Accordingly, we expect to rely primarily on equity and/or debt financings and our clinical funding arrangements (e.g., contract with Department of Defense's DTRA) to fund our continued operations. We are seeking and expect to continue to seek additional funding through financings of equity and/or debt securities, strategic research and development collaborations, clinical funding arrangements, or the sale or license of technology assets. We may also engage in strategic alternative conversations of mergers, acquisition, or sale of the company or assets.

Financing may not be available to us when we need it, or on favorable or acceptable terms, or at all. We could be required to seek funds through collaborative alliances or through other means that may require us to relinquish rights to some of our technologies, drug candidates or drugs that we would otherwise pursue on our own. In addition, if we raise additional funds by issuing equity securities, our then existing stockholders may experience dilution. The terms of any financing may adversely affect the holdings or the rights of existing stockholders. An equity financing that involves existing stockholders may cause a concentration of ownership. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, and are likely to include rights that are senior to the holders of our common stock. Any additional debt or equity financing may contain terms which are not favorable to us or to our stockholders, such as liquidation and other preferences, or liens or other restrictions on our assets. As discussed in Note 16 to the consolidated financial statements included elsewhere in this Form 10-K, additional equity financings may also result in cumulative changes in ownership over a three-year period in excess of 50% which would limit the amount of net operating loss and tax credit carryforwards that we may utilize in any one year.

If we are unable to raise additional capital when required or on acceptable terms, we may be required to:

- significantly delay, scale back, or discontinue the development or commercialization of our product candidates;
- seek strategic alliances, or amend existing alliances, for research and development programs at an earlier stage than otherwise would be desirable or that we otherwise would have sought to develop independently, or on terms that are less favorable than might otherwise be available in the future;
- dispose of technology assets, including current product candidates, or relinquish or license on unfavorable terms, our rights to technologies or any of our product candidates that we otherwise would seek to develop or commercialize ourselves;
- pursue the sale of our company to a third party at a price that may result in a loss on investment for our stockholders; or
- file for bankruptcy or cease operations altogether.

Any of these events could have a material adverse effect on our business, operating results, and prospects.

We believe the key factors which will affect our ability to obtain funding are:

- the receptivity of the capital markets to financings by biotechnology companies generally and companies with drug candidates and technologies similar to ours specifically;
- the receptivity of the capital markets to any in-licensing, product acquisition or other transaction we may enter into or attempt to enter into;
- the results of our clinical development activities in our drug candidates we develop on the timelines anticipated;

- competitive and potentially competitive products and technologies and investors' receptivity to our drug candidates we develop and the technology underlying them in light of competitive products and technologies;
- the cost, timing, and outcome of regulatory reviews; and
- our ability to enter into additional collaborations with biotechnology and pharmaceutical companies and the success of such collaborations.

In addition, increases in expenses or delays in clinical development may adversely impact our cash position and require additional funds or cost reductions.

***Cash Flows***

The following table presents a summary of the primary sources and uses of cash for the years ended December 31, 2022 and 2021:

| | Year Ended December 31, | |
|---|---|---|
| ***(in thousands)*** | **2022** | **2021** |
| Net cash provided by (used in): | | |
| Operating activities | $ (24,495) | $ (24,597) |
| Investing activities | 5,482 | 4,500 |
| Financing activities | (1,488) | 19,413 |
| **Decrease in cash and cash equivalents** | **$ (20,501)** | **$ (684)** |

*Operating Activities*. The net cash used in operating activities for the periods presented consisted primarily of our net (loss) income adjusted for non-cash charges/gains and changes in components of working capital. The change in our operating cash outflows for the year ended December 31, 2022, as compared to 2021, was nominal.

*Investing Activities*. Net cash provided by investing activities for the year ended December 31, 2022 consisted of $5.5 million in cash acquired in connection with the Aceragen Acquisition. Net cash provided by investing activities for the year ended December 31, 2021 consisted of $4.5 million in proceeds from the maturity of available-for-sale securities.

*Financing Activities*. Net cash used in financing activities for the year ended December 31, 2022 consisted of $1.5 million in repayments of outstanding debt assumed in connection with the Aceragen Acquisition, offset in part by proceeds from employee stock purchases and exercise of common stock warrants. Net cash provided by financing activities for the year ended December 31, 2021 included aggregate net proceeds of $19.5 million from financing arrangements consisting of $4.2 million received pursuant to the LPC Purchase Agreement (as defined below) and $15.3 million received under the ATM Agreement, plus $0.3 million received from the exercise of stock options and warrants, partially offset by $0.4 million in payments related to our short-term insurance premium financing arrangement.

***Material Cash Requirements***

*Aceragen Acquisition Obligation*

As of December 31, 2022, we had a material debt obligation in the amount of $6.1 million related to a $7.5 million debt obligation we assumed in connection with the Aceragen Acquisition, of which approximately $1.5 million was paid in October 2022. On January 31, 2023, we issued 12% convertible unsecured promissory notes (the "Convertible Notes") to certain of the debtholders in an aggregate amount of approximately $5.9 million. The Convertible Notes bear annual interest at 12%, beginning on April 1, 2023, and may be converted into shares of Company's common stock. The terms of the Convertible Notes also provide the noteholders with customary registration rights covering the common stock issued following any conversion of the Convertible Notes.

See Note 7 of the notes to our consolidated financial statements appearing elsewhere in this Form 10-K for additional information.

*Lease Obligations*

As of December 31, 2022, we had a material lease commitment in an aggregate amount of $0.6 million relating to our facility in Exton, Pennsylvania. This lease expires on May 31, 2025. See Note 15 of the notes to our consolidated financial statements appearing elsewhere in this Form 10-K for additional information.

## Critical Accounting Estimates

This management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these financial statements requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting periods. On an ongoing basis, management evaluates its estimates and judgments, including those related to (i) indefinite-lived intangible assets, (ii) warrants and Series X Preferred Stock liabilities and related revaluation gains (losses), (iii) research and development prepayments, accruals and related expenses, and (iv) stock-based compensation. Management bases its estimates and judgments on historical experience and on various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We regard an accounting estimate or assumption underlying our financial statements as a "critical accounting estimate" if:

- the nature of the estimate or assumption is material due to the level of subjectivity and judgment necessary to account for highly uncertain matters or the susceptibility of such matters to change; and
- the impact of the estimates and assumptions on financial condition or operating performance is material.

While our significant accounting policies are described in more detail in Note 2 to our financial statements appearing elsewhere in this Form 10-K, we believe the following accounting policies to be the most critical to the judgments and estimates used in the preparation of our financial statements.

***Indefinite-lived Intangible Assets***

Indefinite-lived intangible assets consist of In-Process Research & Development ("IPR&D"). The fair values of IPR&D project assets acquired in business combinations are capitalized. We generally utilize the Multi-Period Excess Earning Method to determine the estimated fair value of the IPR&D assets acquired in a business combination. The projections used in this valuation approach are based on many factors, such as relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population and estimated payments (e.g., royalty). The estimated future net cash flows are then discounted to the present value using an appropriate discount rate. These assets are treated as indefinite-lived intangible assets until completion or abandonment of the projects, at which time the assets are amortized over the remaining useful life or written off, as appropriate.

Intangible assets with indefinite lives, including IPR&D, are tested for impairment if impairment indicators arise and, at a minimum, annually. However, an entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that an indefinite-lived intangible asset's fair value is less than its carrying amount. Otherwise, no further impairment testing is required. The indefinite-lived intangible asset impairment test consists of a one-step analysis that compares the fair value of the intangible asset with its carrying amount. If the carrying amount of an intangible asset exceeds its fair value, an impairment loss is recognized in an amount equal to that excess. We consider many factors in evaluating whether the value of our intangible assets with indefinite lives may not be recoverable, including, but not limited to, expected growth rates, the cost of equity and debt capital, general economic conditions, our outlook and market performance of our industry and recent and forecasted financial performance.

***Warrant and Series X Preferred Stock Liabilities and Related Revaluation Gain (Loss)***

*Warrant Liability*. In connection with the Aceragen Acquisition, a portion of the consideration paid to Legacy Aceragen warrant holders was in the form of warrants to purchase shares of Series Z Preferred Stock (the "Series Z Warrants"). The Series Z Warrants are classified as liabilities because the underlying Series Z Preferred Stock is contingently redeemable. We use a Black-Scholes option pricing model to value our liability-classified warrants, which incorporates assumptions and estimates, including (i) the remaining contractual term of the warrants, (ii) risk-free interest rate, (iii) expected dividend yield, and (iv) expected volatility of the price of the underlying shares of Series Z Preferred Stock. The estimated the expected stock volatility is based on the historical volatility of our common stock for a term equal to the remaining contractual term of the warrants. The risk-free interest rate was determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the remaining contractual term of the warrants. Expected dividend yield was determined based on the fact that we had never paid cash dividends and did not expect to pay any cash dividends in the foreseeable future. Due to the nature of and inputs in the model used to assess the fair value of the warrants, it is not abnormal to experience significant fluctuations during each remeasurement period.

*Series X Preferred Stock Liability*. In conjunction with the Aceragen Acquisition, we determined that the newly issued Series X Preferred Stock represents a sale of future revenues and is classified as a liability under Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") 470, *Debt*, and have elected to account for the Series X Preferred Stock liability under the fair value option. The fair value of the Series X Preferred Stock liability represents the present value of estimated future payments, including royalty payments, as well as estimated payments that are contingent upon the achievement of specified milestones. The fair value of the Series X Preferred Stock liability is based on the cumulative probability of the various estimated payments. The fair value measurement is based on significant Level 3 unobservable inputs such as estimated sales proceeds related to the PRV, the relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population, sales by region, estimated royalty payments and discount rate. Any changes in the fair value of the liability in the reporting periods are recognized in the consolidated statement of operations until it is settled.

***Research and Development Prepayments, Accruals and Related Expenses***

As part of the process of preparing our financial statements, we are required to estimate our accrued and prepaid expenses for research and development activities performed by third parties, including CROs clinical investigators and our research collaboration partners. These estimates are made as of the reporting date of the work completed over the life of the individual study in accordance with agreements established with CROs and clinical trial sites. Some CROs invoice us on a monthly basis, while others invoice upon achievement of milestones and the expense is recorded as services are rendered. We determine the estimates of research and development activities incurred at the end of each reporting period through discussion with internal personnel and outside service providers and research collaboration partners as to the progress or stage of completion of trials or services, as of the end of each reporting period, pursuant to contracts with clinical trial centers and CROs and the agreed upon fee to be paid for such services. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Clinical trial site costs related to patient enrollments are recorded as patients are entered into the trial.

***Stock-Based Compensation***

We recognize all share-based payments to employees and directors as expense in our statements of operations based on their fair values. We record compensation expense over an award's requisite service period, or vesting period, based on the award's fair value at the date of grant. Our policy is to charge the fair value of stock options as an expense, adjusted for forfeitures, on a straight-line basis over the vesting period, which is generally four years for employees and one year for directors.

We use the Black-Scholes option pricing model to estimate the fair value of stock option grants. The Black-Scholes option pricing model relies on a number of key assumptions to calculate estimated fair values, including assumptions as to average risk-free interest rate, expected dividend yield, expected life, and expected volatility. For the assumed risk-free interest rate, we use the U.S. Treasury security rate with a term equal to the expected life of the option. Our assumed dividend yield of zero is based on the fact that we have never paid cash dividends to

common stockholders and have no present intention to pay cash dividends. We use an expected option life based on actual experience. Our assumption for expected volatility is based on the actual stock-price volatility over a period equal to the expected life of the option.

If factors change and we employ different assumptions for estimating stock-based compensation expense in future periods, or if we decide to use a different valuation model, the stock-based compensation expense we recognize in future periods may differ significantly from what we have recorded in the current period and could materially affect our loss from operations, net income (loss) and earnings (loss) per share. It may also result in a lack of comparability with other companies that use different models, methods, and assumptions. The Black-Scholes option pricing model was developed for use in estimating the fair value of traded options that have no vesting restrictions and are fully transferable. These characteristics are not present in our option grants. Although the Black-Scholes option pricing model is widely used, existing valuation models, including the Black-Scholes option pricing model, may not provide reliable measures of the fair values of our stock-based compensation.

***New Accounting Pronouncements***

New accounting pronouncements are discussed in Note 2 of the notes to our consolidated financial statements appearing elsewhere in this Form 10-K.

## Item 7A. Quantitative and Qualitative Disclosures about Market Risk.

As of December 31, 2022, all material assets and liabilities are in U.S. dollars, which is our functional currency. Although our foreign subsidiary is included in our consolidated financial statements, operations at this entity are not material and there are currently no significant financial assets or liabilities denominated in a foreign currency in which exchange rate fluctuations would result in a material adverse impact to our results of operations, financial position, or cash flows.

We maintain investments in accordance with our investment policy. The primary objectives of our investment activities are to preserve principal, maintain proper liquidity to meet operating needs, and maximize yields. Although our investments are subject to credit risk, our investment policy specifies credit quality standards for our investments and limits the amount of credit exposure from any single issue, issuer, or type of investment. We regularly review our investment holdings in light of the then current economic environment. At December 31, 2022, all our invested funds were invested in money market funds classified in cash and cash equivalents on the accompanying balance sheet.

Based on a hypothetical ten percent adverse movement in interest rates, the potential losses in future earnings, fair value of risk sensitive financial instruments, and cash flows are immaterial to our earnings, although the actual effects may differ materially from the hypothetical analysis.

## Item 8. Financial Statements and Supplementary Data.

All financial statements required to be filed hereunder are filed as listed under Item 15(a) of this Form 10-K and are incorporated herein by reference.

We are a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act, and therefore we are permitted to provide scaled Item 8 disclosure.

There have been no retrospective changes to our consolidated statements of operations for any of the quarters within the two years ended December 31, 2022, except for the retroactive effect of the 1-for-17 reverse stock split as described in Note 19 to the consolidated financial statements appearing elsewhere in this Form 10-K.

## Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

None.

## Item 9A. Controls and Procedures.

**Disclosure Controls and Procedures**

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of December 31, 2022. In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applied its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on this evaluation, our principal executive officer and principal financial officer concluded that, as of December 31, 2022, our disclosure controls and procedures were (1) designed to ensure that material information relating to us is made known to our principal executive officer and principal financial officer by others, particularly during the period in which this report was prepared, and (2) effective, in that they provide reasonable assurance that information required to be disclosed by us in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the applicable SEC rules and forms.

**Internal Control over Financial Reporting**

***a) Management's Annual Report on Internal Control over Financial Reporting***

Our management, with the participation of our principal executive officer and principal financial officer, is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rule 13a-15(f) and 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the Company's principal executive and principal financial officers and effected by the Company's Board of Directors, management, and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of the Company;
- Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the Company are being made only in accordance with authorizations of management and directors of the Company; and
- Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the Company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2022. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in *Internal Control — Integrated Framework* (2013).

We acquired Legacy Aceragen in September 2022. Due to the timing of the Aceragen Acquisition and as allowed under SEC guidance, management's assessment of and conclusion regarding the design and effectiveness of internal control over financial reporting excluded the internal control over financial reporting of Legacy Aceragen, which is relevant to our 2022 consolidated financial statements as of and for the year ended December 31, 2022.

Based on its assessment, management believes that, as of December 31, 2022, the Company's internal control over financial reporting was effective based on those criteria.

**b) Attestation Report of the Registered Public Accounting Firm**

Not Applicable.

**c) Changes in Internal Control over Financial Reporting.**

No change in our internal control over financial reporting occurred during the fourth quarter of the fiscal year ended December 31, 2022 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. However, we are in the process of integrating Legacy Aceragen into our system of internal control over financial reporting which may result in future changes to our internal control environment.

## Item 9B. Other Information.

The information included in this Item 9B is provided, at our option, in lieu of filing such information under Item 5.02 and Item 8.01 of Current Report on Form 8-K.

On April 13, 2023, the Board approved certain cost-cutting measures with a view to preserving capital to support our continuing operations. As part of this plan, we have commenced the furlough of 12 employees (including an executive officer), representing approximately 46% of our workforce. Additionally, certain of our employees and executive officers, including our principal executive officer, John Taylor, and our principal financial officer, John Kirby, will have a portion of their salaries deferred in amounts that exceed $200,000, with such deferrals having a retroactive effective date of April 5, 2023. Such deferrals are expected to continue until such time as the Company is able to secure sufficient additional financing, as mutually agreed upon. If such financing is obtained, the impacted employees and executive officers, including Mr. Taylor and Mr. Kirby, will receive lump-sum payments equal to their respective aggregate deferred base salary amounts.

## Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

# PART III.

## Item 10. Directors, Executive Officers, and Corporate Governance.

**Information about our Directors**

Set forth below is information about each member of our board of directors, including (a) the year in which each director first became a director, (b) their age as of March 30, 2023, (c) their positions and offices with our Company, (d) their principal occupations and business experience during at least the past five years and (e) the names of other public companies for which they currently serve, or have served within the past five years, as a director. We have also included information about each director's specific experience, qualifications, attributes, or skills that led our board of directors to conclude that such individual should serve as one of our directors. We also believe that all of our directors have a reputation for integrity, honesty and adherence to high ethical standards. They each have demonstrated business acumen and an ability to exercise sound judgment, as well as a commitment of service to our Company and our board of directors.

John Taylor, our Chief Executive Officer and a member of our board, is the son-in-law of Andy Jordan, our Chief Strategy Officer. With the exception of the foregoing, there are no family relationships among our directors and executive officers.

The following table provides information about those persons who currently serve as directors of the Company.

| | | | Committee Memberships (1) | | | |
|---|---|---|---|---|---|---|
| **Name** | **Age** | **Relationship** | **Audit** | **Comp** | **N&CG** | **Class – Election Year** |
| Vincent J. Milano | 59 | Chair of the Board | | | | Class I – 2023 |
| Dr. Cristina Csimma | 64 | Director | | X | C | Class I – 2023 |
| Michael Dougherty | 65 | Director | C | | X | Class I – 2023 |
| Dr. Maxine Gowen | 65 | Director | X | C | | Class II – 2024 |
| Ronald Wooten | 63 | Director | X | | | Class III – 2025 |
| John C. Taylor | 53 | Director | | | | Class III – 2025 |

- "C" indicates Chair of applicable committee.

***Vincent J. Milano*** was appointed Chair of our board of directors in 2022, having previously served as our President and Chief Executive Officer and as a member of our board of directors since 2014. Prior to joining us, Mr. Milano served as Chairman, President, and Chief Executive Officer of ViroPharma Incorporated ("ViroPharma"), a pharmaceutical company that was acquired by Shire plc in 2014, from 2008 to 2014, as its Vice President, Chief Financial Officer, and Chief Operating Officer from 2006 to 2008, and as its Vice President, Chief Financial Officer, and Treasurer from 1996 to 2005. Mr. Milano also served on the board of directors of ViroPharma from 2008 to 2014. Prior to joining ViroPharma, Mr. Milano served in increasingly senior roles, most recently senior manager, at KPMG LLP, an independent registered public accounting firm, from 1985 to 1996.

Mr. Milano currently serves on the board of directors of Aclaris Therapeutics, Inc. (Nasdaq: ACRS) and Biocryst Pharmaceuticals, Inc. (Nasdaq: BCRX), since 2020 and 2021, respectively. Mr. Milano previously served as a director of Spark Therapeutics, Inc., Vanda Pharmaceuticals Inc., and privately held VenatoRx Pharmaceuticals, Inc. from 2014 to 2019, from 2010 to 2019, and from 2013 to 2022, respectively. Mr. Milano holds a Bachelor of Science degree in Accounting from Rider College.

We believe that Mr. Milano's qualifications to sit on our board of directors include his significant public company management and board experience and knowledge of our industry.

***Dr. Cristina Csimma,*** *PharmD, MHP* is a biopharmaceutical leader and strategic advisor with decades of experience in biotechnology, large pharma, and venture capital. Dr. Csimma currently serves on the board of directors of Syncona Partners, LLP (LON: SYNC), having been elected to its board of directors in February 2022. She also serves as a board director and a member of the compensation committee of Palisade Bio, Inc. (Nasdaq: PALI), having been elected to its board of directors in 2017. She also serves as the chair of the board of directors of Caraway Therapeutics, Inc. since 2019 (executive chair in 2019). Dr. Csimma also serves on advisory boards,

including: the Muscular Dystrophy Association Venture Philanthropy Scientific Advisory Committee since 2006; the Harvard and Brigham and Women's Hospital MRCT Center External Advisory Board since 2015; the TREAT-NMD Advisory Committee for Therapeutics (TACT) since 2009; and the Executive Oversight Board to the National Institutes of Health (NIH) NeuroNext Network since 2013.

Dr. Csimma previously served as chair of the board of directors of Forendo Pharma between 2020 and 2021 (executive chair in 2021) when it was acquired by Organon & Co. Dr. Csimma also previously served as a director on the boards of Seneca Biopharma, Inc. (formerly Neuralstem Inc., from 2017 until 2021 when it merged with Leading BioSciences Inc. to form Palisade Bio), Juniper Pharmaceuticals, Inc. (from 2010 until its acquisition by Catalent, Inc. in 2018), and Vtesse Inc. (from 2014 until its acquisition by Sucampo Pharmaceuticals, Inc. in 2017). Dr. Csimma was the executive chair and a senior advisor of Exonics Therapeutics, Inc. (from 2016 to 2017), and was President, founding Chief Executive Officer, and board director of Cydan Inc. from 2012 to 2014. She also served on the board of directors of T1D Exchange (non-profit Type 1 Diabetes) from 2018 to 2020 and the NIH Blueprint Neurotherapeutics Network External Oversight Committee from 2014 to 2018, was Vice President of Drug Development at Virdante Pharmaceuticals Inc. from 2009 to 2011, Principal at Clarus Ventures LLC (now Blackstone Life Science), and held roles of increasing responsibility in Clinical Development and Translational Research at Wyeth (now Pfizer Inc.), Genetics Institute, and Dana Farber Cancer Institute. Dr. Csimma holds both a Doctor of Pharmacy and a Bachelor of Science in Pharmacy from the Massachusetts College of Pharmacy and Allied Health Sciences, as well as a Master of Health Professions from Northeastern University.

We believe that Dr. Csimma's qualifications to sit on our board of directors include her significant public company management and board experience and knowledge of our industry.

***Michael Dougherty*** was appointed as a member of our board of directors in 2019 and served as the board's chair from 2021 to 2022. Mr. Dougherty currently serves on the board of directors of Trevena, Inc. (Nasdaq: TRVN). Mr. Dougherty was executive chairman of Celator Pharmaceuticals, Inc., or Celator, from 2015 until its acquisition by Jazz Pharmaceuticals plc in 2016; he also served as a director of Celator from 2013 to 2016. Mr. Dougherty previously served in a variety of senior positions in the biopharmaceutical industry, including as Chief Executive Officer at Kalidex Phamaceuticals, Inc.; President and Chief Executive Officer at Adolor Corporation; President and Chief Operating Officer at Genomics Collaborative, Inc.; President and Chief Executive Officer at Genaera Corporation; and Chief Financial Officer at Centocor, Inc. He also previously served as a member of the board of directors of and Marinus Pharmaceuticals, Inc. (Nasdaq: MRNS), Foundation Medicine, Inc., Adolor Corporation, Genaera Corporation, Aviragen Therapeutics, Inc., Cempra, Inc., and ViroPharma Incorporated. Mr. Dougherty received a Bachelor of Science in Accounting from Villanova University.

We believe that Mr. Dougherty's qualifications to sit on our board of directors include his significant public company management and board experience and knowledge of our industry.

***Dr. Maxine Gowen*** served as the Chief Executive Officer and a board director of TamuroBio Inc., a privately held drug development company, from 2019 to 2021, and she remains on the board of directors. She was the founding President and Chief Executive Officer of Trevena, Inc. ("Trevena") (Nasdaq: TRVN), a publicly traded biopharmaceutical company, from 2007 until her retirement in 2018; she remained a member of its board of directors until 2021. Prior to joining Trevena, Dr. Gowen was Senior Vice President for the Center of Excellence for External Drug Discovery at GlaxoSmithKline plc ("GSK"), where she held a variety of leadership positions in research and development ("R&D"), venture capital, and business development during her 15-year tenure. Before GSK, Dr. Gowen was Senior Lecturer and Head, Bone Cell Biology Group, Department of Bone and Joint Medicine, of the University of Bath, U.K. Dr. Gowen has served as a director of Aclaris Therapeutics, Inc. (Nasdaq: ACRS) since 2019, Passage Bio, Inc. (Nasdaq: PASG), and as its Chair since 2021, and Merus NV (Nasdaq: MRUS) since 2021. She previously held board seats in the state biotechnology industry association, Life Sciences of Pennsylvania (from 2015 to 2021) and in the national biotechnology industry association ("BIO") (from 2008 to 2018). Dr. Gowen previously served as a director of Human Genome Sciences, Inc. (from 2008 to 2012) and Akebia Therapeutics, Inc. (from 2014 to 2021), both publicly traded companies, as well as Panorama Medicine, a privately held biotechnology company (from 2020 to 2021). She received her Ph.D. from the University of Sheffield, U.K., an M.B.A. with academic honors from The Wharton School of the University of Pennsylvania, and a B.Sc. with Honors in Biochemistry from the University of Bristol, U.K.

We believe that Dr. Gowen's qualifications to sit on our board of directors include her significant public company management and board experience and knowledge of our industry.

***Ronald Wooten*** was appointed to our Board of Directors in connection with the closing of the Aceragen Acquisition. Prior to the closing of the Aceragen Acquisition, Mr. Wooten served as a member of the board of directors of Legacy Aceragen since May 2021. Mr. Wooten has been a partner of NovaQuest Capital Management, L.L.C., an investment firm that focuses on the biopharmaceutical sector, since its inception in 2010. Since 2010, Mr. Wooten has been a member of the investment committee of NovaQuest Pharma Opportunities Fund III, NovaQuest Pharma Opportunities Fund IV, NovaQuest Pharma Opportunities Fund V, NovaQuest Private Equity Fund I, and NovaQuest Animal Health Fund I. From 2000 to 2010, he was President of the NovaQuest business unit of Quintiles Inc., a contract research company ("Quintiles"). Mr. Wooten was previously Executive Vice President at Quintiles and served on its board of directors from 2008 to 2010. Mr. Wooten's previous experience includes nine years with First Union Securities, where he served as a Managing Director of Investment Banking. Mr. Wooten holds a B.A. degree in Chemistry from the University of North Carolina at Chapel Hill and an M.B.A. from Boston University.

We believe that Mr. Wooten's qualifications to sit on our board of directors include his significant public company management and board experience and knowledge of our industry.

***John C. Taylor*** was appointed as our Chief Executive Officer and to our board of directors in connection with the closing of the Aceragen Acquisition. Mr. Taylor was a co-founder and previously served as the Chief Executive Officer of Legacy Aceragen since its founding in 2021. Since 2018, Mr. Taylor has served as the Entrepreneur In Residence for the North Carolina Biotechnology Center. From 2013 to 2018, Mr. Taylor served as the Chief Executive Officer for Spyryx Biosciences, Inc., a company focused on developing therapies targeting novel regulation of ENaC for lung complications associated with cystic fibrosis. Prior to this, Mr. Taylor served as the Vice President, Corporate Development for Synageva BioPharma Corp. from 2009 to 2013 and Vice President, Business Development for Javelin Pharmaceuticals, Inc. from 2008 to 2009. Mr. Taylor holds a Bachelor of Science in Biological Sciences from Clemson University and a Master of Science in Technology Management from the University of Pennsylvania.

We believe that Mr. Taylor's qualifications to sit on our board of directors include his significant public company management and board experience and knowledge of our industry.

**Information about our Executive Officers**

Our currently-serving executive officers and their respective ages and positions are described below. Our executive officers serve until they resign, or the board terminates their position.

| Name | Age | Position |
|---|---|---|
| John Taylor* | 53 | President and Chief Executive Officer |
| John J. Kirby | 51 | Chief Financial Officer |
| Andy Jordan | 75 | Chief Strategy Officer |
| Dr. Carl Kraus | 54 | Chief Medical Officer |
| Daniel Salain | 55 | Chief Operating Officer |

* Mr. Taylor is a member of our board of directors. See "Information about our Directors" above for more information about Mr. Taylor.

***John J. Kirby*** has served as the Chief Financial Officer of Idera since 2019. Mr. Kirby joined the Company in 2015 as our Vice President of Corporate Accounting. He served as Vice President of Finance from 2018 to 2019 and has served as Senior Vice President and Chief Financial Officer since 2019 (and as principal financial officer and principal accounting officer since 2018). Prior to joining us, Mr. Kirby served as Assistant Controller at Endo Pharmaceuticals, Inc. from 2014 to 2015. From 2012 to 2014, Mr. Kirby served as Vice President, Chief Accounting Officer and Corporate Controller at ViroPharma Incorporated, which was acquired by Shire Plc in 2014. Mr. Kirby began his career at KPMG, LLP in its Healthcare and Life Science Practice and served as a Regional Audit Director at AstraZeneca Pharmaceuticals L.P. prior to joining ViroPharma Incorporated. Mr. Kirby received his B.S. in Accountancy from Villanova University and is a licensed certified public accountant in the Commonwealth of Pennsylvania. Mr. Kirby has also served on the board of trustees of the Delaware Museum of Nature & Science (formerly the Delaware Museum of Natural History) since 2018.

***Andy Jordan*** was appointed as the Chief Strategy Officer of the Company in connection with the closing of the Aceragen Acquisition. Previously, Mr. Jordan served as the Chief Financial Officer of Legacy Aceragen from 2021 to September 2022. Prior to joining Legacy Aceragen, Mr. Jordan founded AR Jordan Consulting, working primarily with biotechnology companies, focusing on bringing to market drugs to treat orphan/rare diseases, serving as its principal from 2008 to 2021. Prior to this, Mr. Jordan held executive-level positions with Guilford Pharmaceuticals, Inc., Odyssey Pharmaceuticals, Inc., and InfraReDx, Inc. Mr. Jordan served as a member of the board of directors for Gemin X BioPharmaceuticals, Inc. from 2003 to 2011 and Spyryx Biosciences, Inc. from 2013 to 2018. Mr. Jordan holds a Bachelor of Arts in Liberal Arts from Rutgers University and an M.B.A. in Professional Accounting from Rutgers University — Newark.

***Dr. Carl Kraus*** was appointed as the Chief Medical Officer of the Company in connection with the closing of the Aceragen Acquisition. Prior to this appointment, Dr. Kraus served as the Chief Medical Officer of Legacy Aceragen from 2021 to September 2022. Prior to this, in 2017, Dr. Kraus founded Arrevus, Inc. ("Arrevus"), a clinical-stage biotechnology company developing novel therapies for orphan diseases, and served as its Chief Executive Officer from 2017 to 2020. Prior to founding Arrevus, Dr. Kraus served as the Chief Medical Officer of Nanotherapeutics, Inc. from 2013 to 2017, the Vice President, Medical Affairs, Risk Management/REMS of Medscape, LLC from 2010 to 2013, and the Vice President, Infectious Diseases, Scientific Affairs of PRA International from 2008 to 2010. Prior to joining industry, Dr. Kraus was a medical officer in the Center for Drug Evaluation and Research at the Food & Drug Administration from 2002 to 2005. Dr. Kraus holds a Bachelor of Arts in Biology from Washington University in St. Louis and received his M.D. in Medicine from Washington University School of Medicine in St. Louis.

***Daniel Salain*** was appointed as the Chief Operating Officer of the Company in connection with the closing of the Aceragen Acquisition. Prior to this appointment, Mr. Salain served as Chief Operating Officer of Legacy Aceragen from 2020 to September 2022. Previously, Mr. Salain was the Chief Operating Officer of Graybug Vision, Inc., a clinical-stage biopharmaceutical company focused on developing medicines for ocular diseases, from 2017 to 2020, and was the Senior Vice President, Global Head of Manufacturing & Supply Chain at Ophthotech, a clinical-stage biotechnology company, from 2015 to 2017. Prior to working at Ophthotech, Mr. Salain was the Vice President of Global Operations, Manufacturing & Supply Chain at Aptalis Pharmaceuticals Inc., a company that focused on developing products to treat gastrointestinal diseases and disorders, from 1999 to 2014. Mr. Salain has a Bachelor of Science degree in Chemistry and Marketing from the University of Indianapolis.

**Code of Business Conduct and Ethics**

We have adopted a written code of business conduct and ethics that applies to our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. We have posted a current copy of the Code of Business Conduct and Ethics in the "Investors — Corporate Governance" section of our website, which is located at www.aceragen.com. We intend to satisfy the disclosure requirements under Item 5.05 of Form 8-K regarding an amendment to, or waiver from, a provision of our code of business conduct and ethics by posting such information on our website at www.aceragen.com

**Audit Committee**

Our board has established a formal standing audit committee. The current members of our audit committee are Mr. Dougherty (Chair), Dr. Gowen, and Mr. Wooten. Our board has determined that Mr. Dougherty is an "audit committee financial expert" within the meaning of SEC rules and regulations. Each member of the audit committee is independent as defined under applicable rules of the Nasdaq, including the independence requirements contemplated by Rule 10A-3 under the Exchange Act.

## Item 11. Executive Compensation.

In accordance with Item 402(l) of Regulation S-K, the Company has elected to avail itself of the scaled disclosure requirements available to smaller reporting companies.

This section discusses the material components of the Company's executive compensation program for our named executive officers ("NEOs") for the fiscal year ended December 31, 2022:

- John C. Taylor, President and Chief Executive Officer[1]
- John J. Kirby, Senior Vice President and Chief Financial Officer
- Bryant D. Lim, former Chief Business Officer, General Counsel and Secretary[2]
- Vincent J. Milano, former President and Chief Executive Officer[3]
- Daniel B. Soland, former Senior Vice President and Chief Operating Officer[4]

(1) Mr. Taylor was appointed as the Company's President and Chief Executive Officer on September 28, 2022 in connection with the Aceragen Acquisition.

(2) Mr. Lim was an executive officer for the entire fiscal year ended December 31, 2022. On February 17, 2023, Mr. Lim resigned as the Company's Senior Vice President, Chief Business Officer and General Counsel.

(3) Mr. Milano served as the Company's President and Chief Executive Officer until September 28, 2022.

(4) Mr. Soland served as the Company's Senior Vice President and Chief Operating Officer until September 28, 2022.

**Summary Compensation Table**

The following table sets forth information concerning the compensation of our named executive officers during the fiscal years ended December 31, 2021 and 2022:

| NAME AND PRINCIPAL POSITION | YEAR | SALARY ($) | STOCK AWARDS ($)(1) | OPTION AWARDS ($)(2) | NON-EQUITY INCENTIVE PLAN COMPENSATION ($) | ALL OTHER COMPENSATION ($)(3) | TOTAL ($) |
|---|---|---|---|---|---|---|---|
| **John C. Taylor** | 2022 | 77,629 | — | — | 134,541 | 9,019 | 221,189 |
| Chief Executive Officer | | | | | | | |
| **John J. Kirby** | 2022 | 374,015 | — | 45,670 | 143,423 | 295,633 | 858,741 |
| Chief Financial Officer | 2021 | 365,000 | — | 182,575 | 73,000 | 39,408 | 659,983 |
| **Bryant D. Lim** | 2022 | 374,015 | — | 45,670 | 143,423 | 304,028 | 867,136 |
| Former Chief Business Officer, | 2021 | 365,000 | — | 182,575 | 73,000 | 42,965 | 663,540 |
| General Counsel, and Secretary | | | | | | | |
| **Vincent J. Milano** | 2022 | 460,953 | — | 84,444 | — | 883,348 | 1,428,745 |
| Former Chief Executive Officer | 2021 | 600,000 | — | 340,189 | 150,000 [4] | 44,244 | 1,134,433 |
| **Daniel B. Soland** | 2022 | 318,750 | — | 45,670 | — | 630,329 | 994,749 |
| Former Chief Commercial Officer | 2021 | 423,390 | — | 534,621 | 85,000 [4] | 44,437 | 1,087,448 |

(1) Represents the aggregate grant date fair value of RSUs as computed in accordance with ASC 718. See Note 14 to the consolidated financial statements included in this Form 10-K regarding assumptions we made in determining these values. The grant date fair value of RSUs is determined using the fair value of

our common stock on the date of grant. The equity incentive awards included in this column were all awarded under the Company's 2013 Stock Incentive Plan, as amended and restated.

(2) Represents the aggregate grant date fair value of options granted to each of the NEOs as computed in accordance with ASC 718. These amounts do not represent the actual amounts paid to or realized by the NEOs. See Note 14 to the consolidated financial statements included in this Form 10-K regarding assumptions we made in determining the fair value of option awards. The equity incentive awards included in this column were all awarded under the Company's 2013 Stock Incentive Plan, as amended and restated.

(3) "All Other Compensation" for 2022 for each of the NEOs includes the following:

| | Premiums Paid by us for all Insurance Plans ($) | Company Match on 401(K) ($) | Severance ($) | Retention ($) | Total ($) |
|---|---|---|---|---|---|
| Mr. Taylor | 5,259 | 3,760 | — | — | 9,019 |
| Mr. Kirby | 24,883 | 15,250 | — | 255,500 | 295,633 |
| Mr. Lim | 33,278 | 15,250 | — | 255,500 | 304,028 |
| Mr. Milano | 36,741 | 15,250 | 831,357 | — | 883,348 |
| Mr. Soland | 27,825 | 15,250 | 587,254 | — | 630,329 |

Amounts included in the table above under the caption "Severance" represent payments made to Messrs. Milano and Soland pursuant to the Milano Separation Agreement (as defined below) and Soland Separation Agreement (as defined below), respectively, which consist of a 2022 prorated bonus ($225,000 for Mr. Milano and $127,500 for Mr. Soland) and cash severance ($606,357 for Mr. Milano and $459,754 for Mr. Soland) payable in equal installments over a twelve-month period commencing in October 2022. Amounts above exclude the Milano Contingent Severance (as defined below) and the Soland Contingent Severance (as defined below).

Amounts included in the table above under the caption "Retention" represent the Kirby Stock Retention Bonus (as defined below) and Lim Stock Retention Bonus (as defined below). Amounts above exclude the Kirby Cash Retention Bonus (as defined below) and Lim Cash Retention Bonus (as defined below) as such payments were contingent on termination of employment prior to the Six-Month Anniversary (as defined below), which had not occurred as of December 31, 2022.

(4) Messrs. Milano and Soland voluntarily declined to accept their respective earned 2021 bonuses. Accordingly, neither Mr. Milano nor Mr. Soland received an annual cash incentive award for 2021.

**Narrative Disclosure to Summary Compensation Table**

***Employment, Retention, and Separation Agreements with Our NEOs***

We have entered into employment agreements with each of our NEOs. All the NEOs are at-will employees.

John Taylor Employment Agreement

In connection with the execution of the Merger Agreement, Mr. Taylor's "at will" employment agreement with Legacy Aceragen, dated February 25, 2021 (the "Taylor Employment Agreement"), was assumed by the Company on the same terms as entered into by Legacy Aceragen, except as otherwise described herein. Pursuant to certain approvals by Legacy Aceragen prior to the Aceragen Acquisition, Mr. Taylor's annual base salary was increased to $450,000, effective as of the Effective Date. In addition, Mr. Taylor is eligible for a discretionary annual incentive bonus, which will be determined by the Board, and the target bonus will be 50% of Mr. Taylor's annual base salary. All other terms of the Taylor Employment Agreement remain the same.

Pursuant to the Taylor Employment Agreement, if the Company terminates Mr. Taylor's employment for any reason other than Cause or Permanent Disability (each as defined by the Taylor Employment Agreement) (such termination, a "Taylor Separation"), provided that Mr. Taylor returns all Company property in his possession and executes a general release of claims in favor of the Company, Mr. Taylor will be entitled to severance benefits in

the form of: (i) continued payment of his base salary for a period of up to 12 months from the date of Taylor Separation; (ii) if Mr. Taylor elects to continue his health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA"), payment of the Company portion of the monthly COBRA premiums for the Company's medical benefit plan for 12 months; and (iii) 12 additional months of service-based vesting (in addition to vesting determined by the actual period of service that has been completed with the Company or with Legacy Aceragen, as applicable) on any equity positions in the Company Mr. Taylor owns or controls. Such vested portion of the equity positions will be exercisable by Mr. Taylor for 12 months following the Taylor Separation. In the event of a change of control, any unvested portions of equity position owned or controlled by Mr. Taylor shall, as of the closing of such transaction, accelerate and become fully vested.

Mr. Taylor is also entitled to participate in the Company-sponsored employee benefit plans, including its medical, dental, vision, and 401(k) plans or similar arrangements. Additionally, the Company has agreed to provide an allowance, not to exceed $2,500 per month, for the cost of the health, dental, and vision plans. Mr. Taylor is entitled to use paid time off, in accordance with the Company policies. Mr. Taylor is also entitled to receive equity-based awards under the Company's equity incentive plans.

The foregoing description of the Taylor Employment Agreement does not purport to be complete and is qualified in its entirety by reference to the Taylor Employment Agreement, which was filed as Exhibit 10.2 to the Company's Current Report on Form 8-K filed with the SEC on September 30, 2022.

John J. Kirby Employment Agreement and Retention Agreement

We are a party to an Employment Offer Letter, dated October 15, 2015, with Mr. Kirby, our Senior Vice President and Chief Financial Officer, and in connection with the execution of the Merger Agreement, the Company and Mr. Kirby entered into an employment continuation and retention bonus letter agreement (the "Kirby Employee Retention Agreement"). Pursuant to the Kirby Employee Retention Agreement, Mr. Kirby's annual base salary increased from $365,000 to $400,000, less applicable taxes and withholdings, and Mr. Kirby's current target bonus (i.e., 40% of his base salary) was prorated to reflect the increase in his annual base salary. Mr. Kirby had previously entered into an individual severance agreement (the "Kirby Severance Agreement") with the Company based on the Company's form Severance and Change of Control Agreement (the "Severance Agreement Form"), pursuant to which he is eligible to receive certain severance payments and benefits upon certain terminations of employment with the Company, including for Good Reason (as defined in the Kirby Severance Agreement). Pursuant to the Kirby Employee Retention Agreement, Mr. Kirby agreed to waive his right to resign for Good Reason solely in connection with the closing of the Aceragen Acquisition. The remaining terms of the Kirby Severance Agreement remain in full force and effect. See "Severance and Change in Control Benefits and Agreements with NEOs" for a description of the material terms of the Severance Agreement Form.

Pursuant to the Kirby Employee Retention Agreement, Mr. Kirby is eligible to receive an amount in stock and/or cash with an aggregate value equal to $766,500 (the "Kirby Retention Bonus"), which will be paid in two installments. Mr. Kirby will receive fully vested shares of Common Stock in a number of shares calculated by dividing (a) one-third of the Kirby Retention Bonus by (b) the volume-weighted average price of the Common Stock based on the 20 trading days prior to the first business day that is within the next available trading window following the Effective Date under the Company's applicable trading policies (the "Kirby Stock Retention Bonus"). If Mr. Kirby's employment with the Company terminates for any reason (other than by the Company for Cause (as defined in the Kirby Severance Agreement)) prior to the six-month anniversary of the approval of the Reverse Stock Split Proposal (the "Six-Month Anniversary"), Mr. Kirby will receive a lump sum amount in cash equal to two-thirds of the Kirby Retention Bonus, less applicable taxes and withholdings (the "Kirby Cash Retention Bonus"). If Mr. Kirby's employment with the Company continues following such Six-Month Anniversary, or if the Company terminates Mr. Kirby's employment for Cause (as defined in the Kirby Severance Agreement) prior to such date, Mr. Kirby's right to receive the Kirby Cash Retention Bonus will terminate.

If Mr. Kirby's employment with the Company continues past the Six-Month Anniversary, in lieu of the Kirby Cash Retention Bonus, Mr. Kirby will receive: (a) a number of restricted shares of Common Stock calculated by dividing (1) two-thirds of the Kirby Retention Bonus by (2) the volume-weighted average price per share of Common Stock based on the 20 trading days prior to the date of grant, rounded down to the nearest full share (the "Restricted Stock") or (b) a restricted cash award in an amount equal to two-thirds of the Retention Bonus, less applicable taxes and withholding ("Restricted Cash") within 30 days of the Six Month Anniversary. The Restricted Stock or Restricted Cash will vest over two years, with 50% vesting upon the first anniversary and the remainder vesting in

equal quarterly installments thereafter (each, a "Kirby Vesting Date"). Upon termination of Mr. Kirby's employment or service with the Company for any reason prior to the final Kirby Vesting Date, Mr. Kirby will forfeit the unvested portion of the Restricted Stock or Restricted Cash, as applicable.

The foregoing description of the Kirby Employee Retention Agreement and Kirby Severance Agreement does not purport to be complete and is qualified in its entirety by reference to the Kirby Employee Retention Agreement and the Severance Agreement Form, which were filed as Exhibit 10.4 to the Company's Current Report on Form 8-K filed with the SEC on September 30, 2022 and as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed with the SEC on May 4, 2017, respectively.

Bryant D. Lim Employment Agreement and Retention Agreement

Prior to Mr. Lim's resignation in February 2023, we were party to an Employment Offer Letter, dated as of August 20, 2018, with Mr. Lim, our Senior Vice President, General Counsel, and Secretary, and in connection with the execution of the Merger Agreement, the Company and Mr. Lim entered into an employment continuation and retention bonus letter agreement (the "Lim Employee Retention Agreement"). Pursuant to the Lim Employee Retention Agreement, Mr. Lim's annual base salary increased from $365,000 to $400,000, less applicable taxes and withholdings, and Mr. Lim's current target bonus (i.e., 40% of his base salary) was prorated to reflect the increase in his annual base salary. Mr. Lim and the Company previously entered into a severance agreement (the "Lim Severance Agreement") based on the Severance Agreement Form, pursuant to which he is eligible to receive certain severance payments and benefits upon certain terminations of employment with the Company, including for Good Reason (as defined in the Lim Severance Agreement). Pursuant to the Lim Employee Retention Agreement, Mr. Lim agreed to waive his right to resign for Good Reason, as defined in the Lim Severance Agreement, solely in connection with the closing of the Aceragen Acquisition. The remaining terms of the Lim Severance Agreement remain in full force and effect. See "Severance and Change in Control Benefits and Agreements with NEOs" for a description of the material terms of the Severance Agreement Form.

Pursuant to the Lim Employee Retention Agreement, Mr. Lim will be eligible to receive an amount in stock and/or cash with an aggregate value equal to $766,500 (the "Lim Retention Bonus"), which will be paid in two installments. Mr. Lim will receive fully vested shares of Common Stock in a number of shares calculated by dividing (a) one-third of the Lim Retention Bonus by (b) the volume-weighted average price of the Common Stock based on the 20 trading days prior to the first business day that is within the next available trading window following the Effective Date under the Company's applicable trading policies (the "Lim Stock Retention Bonus"). If Mr. Lim's employment with the Company terminates for any reason (other than by the Company for Cause (as defined in the Lim Severance Agreement)) prior to the Six-Month Anniversary, Mr. Lim will receive a lump sum amount in cash equal to two-thirds of the Lim Retention Bonus, less applicable taxes and withholdings (the "Lim Cash Retention Bonus"). If Mr. Lim's employment with the Company continues following the Six-Month Anniversary, or if the Company terminates Mr. Lim's employment for Cause (as defined in the Lim Severance Agreement) prior to such date, Mr. Lim's right to receive the Lim Cash Retention Bonus will terminate.

If Mr. Lim's employment with the Company continues past the Six-Month Anniversary, in lieu of the Lim Cash Retention Bonus, Mr. Lim will receive (a) Restricted Stock or (b) Restricted Cash within 30 days of the Six-Month Anniversary. The Restricted Stock or Restricted Cash will vest over two years, with 50% vesting upon the first anniversary and the remainder vesting in equal quarterly installments thereafter (each, a "Lim Vesting Date"). Upon termination of Mr. Lim's employment or service with the Company for any reason prior to the final Lim Vesting Date, Mr. Lim will forfeit the unvested portion of the Restricted Stock or Restricted Cash, as applicable.

As Mr. Lim resigned from the Company in February 2023, he is entitled to receive the Lim Cash Retention Bonus.
The foregoing description of the Lim Employee Retention Agreement and the Lim Severance Agreement does not purport to be complete and is qualified in its entirety by reference to the Lim Employee Retention Agreement and the Severance Agreement Form, which were filed as Exhibit 10.5 to the Company's Current Report on Form 8-K filed with the SEC on September 30, 2022 and as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed with the SEC on May 4, 2017, respectively.

Vincent J. Milano Employment Agreement and Separation Agreement

Prior to Mr. Milano's transition to the role of non-employee Chair of the Board on the Effective Date (described below), we were party to an Employment Agreement, dated as of December 1, 2014, with Mr. Milano (the "Milano

Employment Agreement"). Under the Milano Employment Agreement, Mr. Milano was entitled to receive an annual base salary of $600,000 or such higher amount as our compensation committee or our board may determine. In addition, pursuant to the Milano Employment Agreement, Mr. Milano was eligible to receive an annual bonus of 50% of his base salary, subject to adjustment, based on the achievement of both individual and Company performance objectives as developed and determined by our board. Mr. Milano's severance and change in control benefits were governed by the Severance Agreement Form, however, such benefits were waived in lieu of the benefits provided for under the Milano Separation Agreement (defined below).

In connection with the execution of the Merger Agreement, the Company entered into an employee separation agreement with Mr. Milano (the "Original Milano Separation Agreement"), as amended pursuant to that certain amendment to employee separation agreement, dated as of February 10, 2023 (the "Milano Amendment" and the Original Milano Separation Agreement as amended by the Milano Amendment, the "Milano Separation Agreement"). Pursuant to the Milano Separation Agreement, Mr. Milano transitioned from Chief Executive Officer to the role of non-employee Chair of the Board and was entitled to receive: (i) any earned and unpaid base salary through the date of the Aceragen Acquisition; (ii) any earned and unpaid annual incentive cash bonus payable with respect to any fiscal year that ended prior to the date of the Aceragen Acquisition; (iii) any accrued but unused personal time off days; (iv) reimbursement for any outstanding expenses for which Mr. Milano has not been reimbursed and which are authorized; and (v) any vested benefits under the Company's employee benefit plans in accordance with the terms of such plans, as accrued through the date of the Aceragen Acquisition (collectively, the "Accrued Obligations"). The Accrued Obligations were paid following the closing of the Aceragen Acquisition at such times and in accordance with such plans and policies as would normally apply to such amounts or benefits.

In addition, provided that Mr. Milano does not revoke the Milano Separation Agreement (including the general release of claims in favor of the Company as set forth therein) and that Mr. Milano continues to comply with the restrictive covenants incorporated into the Milano Separation Agreement, Mr. Milano is entitled to receive: (i) a cash payment of $225,000, representing a prorated portion of the 2022 calendar year annual cash incentive award, at target, based on the period that Mr. Milano was employed through the date of the Aceragen Acquisition (paid in a lump sum within 30 days following the Effective Date); (ii) $606,357, payable in substantially equal installments in accordance with the Company's payroll practices, over the 12 months following the Effective Date and starting with the first payroll date following such date; and (iii) following Approval (as defined in the Milano Separation Agreement) fully vested shares of common stock with a value of $800,000 (the "Milano Contingent Severance"), based on the volume-weighted average price of common stock on the 20 days prior to the grant date, rounded down to the nearest full share (to be granted as soon as practicable following the date the Company consummates an equity financing pursuant to which it sells shares of common stock in exchange for the payment of the purchase price of such stock in cash which results in net proceeds of at least five million dollars ($5,000,000) (the "Equity Financing Date"), but in no event later than the earlier of (i) thirty (30) days following the Equity Financing Date and (ii) June 30, 2023).

The foregoing description of the Milano Separation Agreement does not purport to be complete and is qualified in its entirety by reference to the Original Milano Separation Agreement, which was filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed with the SEC on September 30, 2022, and the Milano Amendment, which is filed as Exhibit 10.30 hereto.

Daniel B. Soland Employment Agreement and Separation Agreement

Prior to Mr. Soland's termination of employment in connection with the Aceragen Acquisition, we were party to an Employment Offer Letter, dated as of November 16, 2020, with Mr. Soland (the "Soland Employment Agreement"). Under the terms of the Soland Employment Agreement, Mr. Soland was entitled to receive an annual base salary of $425,000 or such higher amount as our compensation committee or our board may determine. In addition, under the Soland Employment Agreement, Mr. Soland was eligible to receive an annual bonus of 40% of his base salary, subject to adjustment, based on the achievement of both individual and Company performance objectives. Mr. Soland's severance and change in control benefits were governed by the Severance Agreement Form, however, such benefits were waived in lieu of the benefits provided for under the Soland Separation Agreement (defined below).

In connection with the execution of the Merger Agreement, the Company entered into an executive transition and separation agreement (the "Original Soland Separation Agreement"), as amended pursuant to that certain amendment to employee separation agreement, dated as of February 10, 2023 (the "Soland Amendment" and the

Original Soland Separation Agreement as amended by the Soland Amendment, "Soland Separation Agreement") with Mr. Soland. Under the Soland Separation Agreement, Mr. Soland was entitled to receive: (i) any earned and unpaid base salary through the Effective Date; (ii) any earned and unpaid annual incentive bonus payable with respect to any fiscal year that ended prior to the Effective Date; (iii) any accrued but unused personal time-off days; (iv) reimbursement for any outstanding expenses for which Mr. Soland has not been reimbursed and which are authorized; and (v) any vested benefits under the Company's employee benefit plans in accordance with the terms of such plans, as accrued through the Effective Date.

Under the terms of the Soland Separation Agreement, Mr. Soland agreed to provide certain advisory and transition services to the Company through June 30, 2023. In consideration for his services during such period, Mr. Soland was entitled to $500 per hour performed for services requested by the Company in an independent contractor capacity.

In addition, provided that Mr. Soland does not revoke the Soland Separation Agreement (including the general release of claims in favor of the Company as set forth therein) and Mr. Soland continues to comply with the restrictive covenants incorporated into the Soland Separation Agreement, Mr. Soland is entitled to receive (i) a cash payment of $127,500, representing the prorated portion of the 2022 calendar year annual cash incentive award, measured at target performance, based on the period Mr. Soland was employed through the Effective Date (paid in a lump sum within 30 days following the Effective Date); (ii) $459,754, payable in substantially equal installments over the 12-month period starting on the first payroll date following the Effective Date; and (iii) following Approval (as defined in the Soldan Separation Agreement) fully vested shares of Common Stock equal to a number of shares, calculated by dividing $500,000 (the "Soland Contingent Severance")based on the volume-weighted average price of Common Stock on the 20 days prior to the grant date, rounded down to the nearest full share (to be granted as soon as practicable following the Equity Financing Date, but in no event later than the earlier of (i) thirty (30) days following the Equity Financing Date and (ii) June 30, 2023).

The foregoing description of the Soland Separation Agreement does not purport to be complete and is qualified in its entirety by reference to the Original Soland Separation Agreement, which was filed as Exhibit 10.6 to the Company's Current Report on Form 8-K filed with the SEC on September 30, 2022, and the Soland Amendment, which is filed as Exhibit 10.39 hereto.

***Annual Cash Incentives***

The annual cash incentive award provides an opportunity for additional compensation to NEOs if pre-established annual performance goals are attained. The compensation committee generally links cash awards to the achievement of the annual corporate goals; however, the compensation committee may take into consideration unexpected corporate performance outside of the corporate goals and individual performance. The amount of the bonus paid, if any, may vary among the NEOs depending on individual performance, individual contribution to the achievement of our annual corporate goals, and corporate performance generally. The compensation committee may exercise discretion in its determinations. The annual cash incentive award targets are based on a target percentage of each NEO's salary, as established in his or her employment agreement.

For 2022, the annual cash incentive award for Messrs. Taylor, Kirby, Lim, Milano, and Soland were targeted at 50% (prorated for 35% target applicable prior to Aceragen Acquisition), 40%, 40%, 50% and 40%, respectively, of their respective base salaries.

For 2022, all NEOs were eligible to earn their annual cash bonuses pursuant to the achievement of certain corporate performance goals, as applicable to each NEO, and determined by the compensation committee. Mr. Taylor was eligible to earn his 2022 annual cash bonus pursuant to the achievement of corporate performance goals, as approved by the compensation committee, which considered the achievement of 2022 corporate goals applicable to Legacy Aceragen (prior to the Aceragen Acquisition) and us (subsequent to the Aceragen Acquisition). These goals primarily included achievement of certain clinical-related milestones and completion of a public listing (e.g., IPO, reverse merger or acquisition) and financing transaction. Mr. Kirby and Mr. Lim were eligible to earn their 2022 annual cash bonuses pursuant to the achievement of corporate goals established by the compensation committee, which considered the achievement of 2022 goals applicable to us both prior to and subsequent to the Aceragen Acquisition. These goals primarily included (i) completion of a business development transaction (e.g., merger or acquisition) (prior to the Aceragen Acquisition) and (ii) achievement of certain clinical-related milestones and the completion of a financing transaction (subsequent to the Aceragen Acquisition). Following a review of 2022

performance, our compensation committee approved, and, in the case of Mr. Taylor, our compensation committee recommended and our board or directors approved, 2022 annual cash bonuses of $134,541 (pro-rated), $143,423, and $143,423 to Mr. Taylor, Mr. Kirby, and Mr. Lim, respectively.

In accordance with the Milano Separation Agreement and the Soland Separation Agreement (each as described above), Mr. Milano and Mr. Soland received 2022 annual cash bonuses of $225,000 and $127,500, respectively.

***Long-Term Equity Compensation***

The compensation committee approves all equity awards to our NEOs. Our equity awards have typically been in the form of stock options. However, under the terms of our 2022 Stock Incentive Plan (the "2022 Plan") and its predecessor plan, the 2013 Stock Incentive Plan, as amended (the "2013 Plan"), we may grant equity restricted stock awards, stock appreciation rights, and Restricted Stock Units ("RSUs").

The compensation committee typically makes initial stock option awards to our NEOs upon commencement of their employment. Thereafter, the compensation committee grants annual stock option awards, and as it deems appropriate, may occasionally grant retention or recognition awards, usually in the form of RSUs. Determination of the total annual award amount typically occurs at the regularly scheduled meeting of the compensation committee held in the first quarter of each fiscal year. The 2022 annual stock option awards for all employees, including our NEOs, were split into two tranches for fiscal year 2022, consistent with historical practice. Both tranches of the annual award were approved by the compensation committee before being granted.

In general, 25% of the stock option grant vests on the first anniversary of the date of grant with the balance of the shares subject to the option vesting in 12-equal quarterly installments over the three-year period thereafter. The exercise price of stock options equals the fair market value of our common stock on the date of grant, which is typically equal to the closing price of our common stock on Nasdaq on the date of compensation committee approval, except in the case of new hire grants, which are approved in advance by the compensation committee and granted on the first day of employment.

Consistent with historic practice, in January 2022, the compensation committee approved the first tranche of option grants to our then-serving NEOs (i.e., Mr. Kirby, Mr. Lim, Mr. Milano, and Mr. Soland) (the "January 2022 Option Awards"). In light of insufficient shares available for issuance under the 2013 Plan, only a portion of the January 2022 Option Awards were granted on January 24, 2022. The remaining portion of the January 2022 Option Awards was subsequently granted on April 1, 2022, following additional shares becoming available for issuance. The second tranche of option awards was approved by the compensation committee and granted to the then-serving NEOs (i.e., Mr. Kirby, Mr. Lim, Mr. Milano, and Mr. Soland) on July 6, 2022. The following table sets forth the aggregate number of options granted to our NEOs in during 2022, as retroactively adjusted for the Reverse Stock Split:

| Name | Aggregate Number of Options Granted in 2022 (#) |
|---|---|
| Mr. Taylor | — |
| Mr. Kirby | 7,822 |
| Mr. Lim[(1)] | 7,822 |
| Mr. Milano[(2)] | 14,585 |
| Mr. Soland[(3)] | 7,822 |

(1) Mr. Lim's unvested options were forfeited following his resignation in February 2023.

(2) Mr. Milano's unvested options continue to vest following termination of employment as a result of Mr. Milano continuing to provide service to us as Chair of our board of directors.

(3) Mr. Soland's unvested options continue to vest following termination of employment as a result of Mr. Milano continuing to provide service to us pursuant to the Soland Separation Agreement.

***Other Compensation***

We maintain broad-based benefits, including health and dental insurance, life and disability insurance, and a 401(k) plan, that are provided to all employees. During 2022, we matched 100% of the employee contributions to our 401(k) plan up to a maximum of 5% of the participating employee's annual salary, subject to annual IRS limitations. Our NEOs are eligible to participate in all our employee benefit plans, in each case, on the same basis as other employees and subject to any limitations in such plans. In 2022, each of our NEOs contributed to our 401(k) plan and their contributions were matched by us.

Apart from the discussed benefits, we do not provide our NEOs with perquisites.

**Outstanding Equity Awards at Fiscal End**

The following table sets forth information regarding the outstanding equity held by our NEOs as of December 31, 2022.

| | Option Awards | | | | Stock Awards | | | |
|---|---|---|---|---|---|---|---|---|
| **Name** | **Number of Securities Underlying Unexercised Options Exercisable (#)(1)** | **Number of Securities Underlying Unexercised Options Unexercisable (#)(1)** | **Option Exercise Price ($)** | **Option Expiration Date** | **Number of Shares or Units of Stock That Have Not Vested (#)** | **Market Value of Shares or Units of Stock That Have Not Vested ($)(2)** | **Equity Incentive Plan Awards: Number of Unearned Shares, Units or Other Rights that have Not Vested (#)(3)** | **Equity Incentive Plan Awards: Market or Payout Value of Unearned Shares, Units or Other Rights Not Vested ($)(2)** |
| John C. Taylor [4] | | | | | | | | |
| John J. Kirby | 1,102 | — | 422.96 | 11/2/2025 | | | | |
| | 661 | — | 391.68 | 1/6/2026 | | | | |
| | 734 | — | 216.24 | 1/4/2027 | | | | |
| | 991 | — | 304.64 | 1/3/2028 | | | | |
| | 587 | — | 125.63 | 8/13/2028 | | | | |
| | 1,505 [5][6] | — | 53.38 | 1/3/2029 | | | | |
| | 2,676 [5][7] | — | 42.84 | 7/9/2029 | | | | |
| | 2,941 [5][8] | — | 30.43 | 1/10/2030 | | | | |
| | 2,941 [5][9] | — | 42.16 | 7/21/2030 | | | | |
| | 3,000 [5][10] | — | 72.76 | 1/8/2031 | | | | |
| | 1,222 | 2,689 [11] | 18.87 | 7/8/2031 | | | | |
| | — | 1,470 [12] | 9.77 | 1/24/2032 | | | | |
| | — | 2,441 [13] | 9.10 | 4/1/2032 | | | | |
| | — | 3,911 [14] | 7.36 | 7/6/2032 | | | | |
| | | | | | | | 3,364 | 20,017 |
| Bryant D. Lim | 7,646 | — | 157.93 | 9/10/2028 | | | | |
| | 2,676 [5][15] | — | 53.38 | 1/3/2029 | | | | |
| | 2,676 [5][7] | — | 42.84 | 7/9/2029 | | | | |
| | 2,941 [5][8] | — | 30.43 | 1/10/2030 | | | | |
| | 2,941 [5][9] | — | 42.16 | 7/21/2030 | | | | |
| | 3,000 [5][10] | — | 72.76 | 1/8/2031 | | | | |
| | 1,222 | 2,689 [11] | 18.87 | 7/8/2031 | | | | |
| | — | 1,470 [12] | 9.77 | 1/24/2032 | | | | |

| | Option Awards | | | | Stock Awards | | | |
|---|---|---|---|---|---|---|---|---|
| Name | Number of Securities Underlying Unexercised Options Exercisable (#)(1) | Number of Securities Underlying Unexercised Options Unexercisable (#)(1) | Option Exercise Price ($) | Option Expiration Date | Number of Shares or Units of Stock That Have Not Vested (#) | Market Value of Shares or Units of Stock That Have Not Vested ($)(2) | Equity Incentive Plan Awards: Number of Unearned Shares, Units or Other Rights that have Not Vested (#)(3) | Equity Incentive Plan Awards: Market or Payout Value of Unearned Shares, Units or Other Rights Not Vested ($)(2) |
| | — | 2,441 (13) | 9.1 | 4/1/2032 | | | | |
| | — | 3,911 (14) | 7.36 | 7/6/2032 | | | | |
| | | | | | | | 148 | 881 |
| Vincent J. Milano | 14,705 | — | 424.32 | 12/1/2024 | | | | |
| | 2,205 | — | 391.68 | 1/6/2026 | | | | |
| | 2,205 | — | 216.24 | 1/4/2027 | | | | |
| | 4,411 | — | 304.64 | 1/3/2028 | | | | |
| | 3,823 | — | 125.63 | 8/13/2028 | | | | |
| | 4,659 | 310 (16) | 53.38 | 1/3/2029 | | | | |
| | 4,038 | 932 (17) | 42.84 | 7/9/2029 | | | | |
| | 3,720 | 1,691 (18) | 30.43 | 1/10/2030 | | | | |
| | 3,044 | 2,367 (19) | 42.16 | 7/21/2030 | | | | |
| | 2,444 | 3,144 (20) | 72.76 | 1/8/2031 | | | | |
| | 2,279 | 5,014 (11) | 18.87 | 7/8/2031 | | | | |
| | — | 1,470 (12) | 9.77 | 1/24/2032 | | | | |
| | — | 5,822 (13) | 9.10 | 4/1/2032 | | | | |
| | — | 7,293 (14) | 7.36 | 7/6/2032 | | | | |
| | | | | | 1,585 (21) | 9,431 | | |
| | | | | | | | 5,882 | 35,000 |
| Daniel B. Soland | 11,764 | — | 68.34 | 1/4/2031 | | | | |
| | 1,222 | 2,689 (11) | 18.87 | 7/8/2031 | | | | |
| | — | 1,470 (12) | 9.77 | 1/24/2032 | | | | |
| | — | 2,441 (13) | 9.10 | 4/1/2032 | | | | |
| | — | 3,911 (14) | 7.36 | 7/6/2032 | | | | |

(1) Reflects effect of retroactive application of Reverse Stock Split.

(2) Market Value is calculated based on a price per share of $5.95, which was the closing price of our common stock on December 30, 2022 (last closing price of our common stock as of December 31, 2022).

(3) Represents unvested, performance-based RSUs granted to Messrs. Kirby, Lim, and Milano on July 21, 2020, which vest subject to and on the date the market capitalization of the Company equals or exceeds $500,000,000.

(4) Mr. Taylor did not have any outstanding equity awards as of December 31, 2022.

(5) In March 2021, the compensation committee approved the acceleration of the vesting terms of all outstanding, unvested options that had a value equal to less than $5.00 and RSUs (the “2021 Acceleration”). The 2021 Acceleration applied to all employees, including the NEOs, except Mr. Milano; however, in January 2022, for the NEOs, the compensation committee implemented a holding period (the ‘‘Holding Period’’) prohibiting the sale of shares associated with the 2021

Acceleration according to a schedule not more favorable than the original vesting schedule (i.e., 6.25% of the total option grant every quarter and 25% of the total RSU grant every year). No NEOs exercised any options that became exercisable as a result of the 2021 Acceleration or sold any shares that related to the RSUs that vested in connection with the 2021 Acceleration during the intervening period of March 2021 to January 2022.

(6) 94 options are subject to the Holding Period.

(7) 502 options are subject to the Holding Period.

(8) 919 options are subject to the Holding Period.

(9) 1,287 options are subject to the Holding Period.

(10) 1,688 options are subject to the Holding Period.

(11) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the July 8, 2021 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until July 8, 2025), provided the NEO is still employed with us on each vesting date.

(12) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the January 24, 2022 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until January 24, 2026), provided the NEO is still employed with us on each vesting date.

(13) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the April 1, 2022 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until April 1, 2026), provided the NEO is still employed with us on each vesting date.

(14) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the July 6, 2022 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until July 6, 2026), provided the NEO is still employed with us on each vesting date.

(15) 167 options are subject to the Holding Period.

(16) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the January 3, 2019 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until January 3, 2023), provided the NEO is still employed with us on each vesting date.

(17) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the July 9, 2019 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until July 9, 2023), provided the NEO is still employed with us on each vesting date.

(18) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the January 10, 2020 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until January 10, 2024), provided the NEO is still employed with us on each vesting date.

(19) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the July 21, 2020 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until July 21, 2024), provided the NEO is still employed with us on each vesting date.

(20) Represents unvested portion of stock option award that will vest 25% on the first anniversary date following the January 8, 2021 grant date, with the remainder vesting in 12 equal quarterly installments thereafter (until January 8, 2025), provided the NEO is still employed with us on each vesting date.

(21) Includes the unvested portion of RSUs granted to Mr. Milano on January 3, 2019 and January 10, 2020, which vest in four equal installments over the four-year period following the grant date.

***Severance and Change in Control Agreements***

In 2017, the board approved the Severance Agreement Form, which the Company subsequently entered into with Messrs. Kirby, Lim, Milano, and Soland. The Severance Agreement Form provides that if we consummate a change of control (as defined therein), we will employ the NEO for a period of 24 months from the date of the consummation of the change of control. Pursuant to the Severance Agreement Form, during such period:

- the NEO's position and duties for the Company will be commensurate with the most significant of the duties and positions held by the NEO during the 90-day period preceding the date of the change of control;
- the NEO's annual base salary will equal at least 12 times the highest monthly base salary paid to the NEO during the 12 months prior to the date of the change of control;
- the NEO will be entitled to an annual bonus equal to at least the greatest of (a) the average bonus paid to the NEO in respect of the three years immediately preceding the year in which the change of control occurs, (b) the annual bonus paid for the year immediately preceding the year in which the change of control occurs, and (c) 100% of the target bonus for (1) the year immediately preceding the year in which the change of control occurs, (2) the year in which the change of control occurs, or (3) any year following the year in which the change of control occurs and prior to the then-current year, whichever is highest; and
- the NEO will be entitled to certain other benefits as are consistent with the benefits paid to the NEO during the year prior to the change of control.

The Severance Agreement Form also provides that if a NEO is terminated without "cause" or resigns for "good reason" (as such terms are defined therein) in either case, within 24 months following a change of control, subject to the NEO's timely execution and non-revocation of a general release of claims in a form provided by us and the NEO's continued compliance with the invention, non-disclosure, and non-competition agreement previously entered into in connection with the commencement of NEO's employment, the NEO would receive a lump sum cash payment payable within 30 days after the date of termination equal to:

- the NEO's target bonus for the year of termination prorated for the portion of the year worked;
- 150% of the sum of (a) such NEO's annual base salary for the year immediately preceding the year of termination and (b) the greatest of (1) the average bonus paid or earned and accrued, but unpaid to the NEO in respect of the three years immediately preceding the year of termination, (2) the annual bonus paid for the year immediately preceding the year of termination, and (3) the target bonus for the year of termination; and
- 150% of the Company's share of the annual premium for group medical and/or dental insurance coverage that was in place for the NEO immediately prior to the date of termination.

In addition, all unvested options, restricted stock, RSUs, or stock appreciation rights held by the NEO as of the date of termination will be immediately and automatically vested and/or exercisable in full as of the date of termination, and the NEO will have the right to exercise any such options or stock appreciation rights for the longer of (A) the period of time provided for in the applicable equity award agreement or plan, or (B) the shorter of one year after the date of termination or the remaining term of the applicable equity award.

If the NEO is terminated without "cause" or resigns for "good reason," without regard to whether a change of control has occurred, such NEO will be entitled to the following under the Severance Agreement Form (collectively, the "Without Cause/For Good Reason Benefits"), subject to the NEO's timely execution and non-revocation of a general release of claims in a form provided by us and the NEO's continued compliance with the invention, non-disclosure, and non-competition agreement previously entered into in connection with the commencement of NEO's employment:

- a lump sum cash payment payable within 30 days after the date of termination in an amount equal to the greater of (x) the average bonus paid or earned and accrued, but unpaid to the NEO in respect of the three years immediately preceding the year of termination, and (y) the annual bonus paid for the year immediately preceding the year of termination prorated for the portion of the year worked;

- continued payment of the NEO's base salary payable in accordance with our standard payroll practices over the one-year period following termination; and

- if the NEO elects to continue receiving group medical and/or dental insurance under COBRA (to the extent the NEO previously participated in such group insurance plans immediately prior to the date of termination), payment by us of our share of the premium for such coverage that we pay for active and similarly-situated employees who receive the same type of coverage for the one-year period following termination.

As discussed above, pursuant to the Kirby Employee Retention Agreement and the Lim Employee Retention Agreement, Mr. Kirby and Mr. Lim each agreed to waive their rights to resign for Good Reason, solely in connection with the closing of the Aceragen Acquisition. The remaining terms of the Kirby Severance Agreement and Lim Severance Agreement remain in full force and effect.

As discussed above, Mr. Milano's and Mr.'s Soland's severance and change in control benefits were previously governed by the Severance Agreement Form, however, such benefits were waived in lieu of the benefits provided for under the Milano Separation Agreement and the Soland Separation Agreement. See the subsections entitled "Vincent J. Milano Employment Agreement and Separation Agreement" and "Daniel B. Soland Employment Agreement and Separation Agreement" for a description of the respective severance benefits that Mr. Milano and Mr. Soland were entitled to receive in connection with their employment separations in 2022.

**Retirement Policy Regarding the Treatment of Equity Awards**

Our board has adopted a retirement policy to address the treatment of options and RSUs in the event of an employee's retirement that applies to all employees, including all officers and NEOs. For purposes of this policy, an employee will be deemed to have retired if (i) the employee terminates his or her employment with us, (ii) has been an employee of ours for more than 10 years, and (iii) is older than 65 upon termination of employment. Under the policy, if an employee retires, then:

- all outstanding, unvested equity awards held by the employee will automatically vest in full; and
- the period during which the employee may exercise the options will be extended to the expiration of the term of the option under the applicable option agreement.

Our board adopted this policy for our employees in recognition of the importance of equity awards to the compensation of employees and in order to provide each of our employees with the opportunity to get the full benefit of the options and RSUs held by the employee in the event of his or her retirement after making 10 years of contributions to our Company.

**Director Compensation Table**

The following table sets forth a summary of the compensation we paid to our non-employee directors who served on our board in 2022. Mr. Milano served as our Chief Executive Officer and as a member of our board until September 28, 2022, when Mr. Taylor was appointed Chief Executive Officer and to our board of directors. During his tenure as Chief Executive Officer, Mr. Milano did not receive any compensation for his board service. On September 28, 2022, he was appointed as chair of our board of directors and, following his resignation as Chief Executive Officer, he became eligible to participate in our non-employee director compensation program. However, Mr. Milano declined to accept any board fees in 2022. Information regarding Mr. Milano's and Mr. Taylor's 2022 compensation is set forth in the Summary Compensation Table above.

| | Fees Earned or Paid in Cash ($) | Option Awards ($)(4) | Total ($) |
|---|---|---|---|
| Cristina Csimma | 51,250 | 8,869 | 60,119 |
| Michael Dougherty | 80,563 (5) | 8,869 | 89,432 |
| James A. Geraghty | 46,000 | 8,869 | 54,869 |
| Mark Goldberg(1) | 41,625 | 8,869 | 50,494 |
| Maxine Gowen | 57,375 | 8,869 | 66,244 |
| Carol A. Schafer(2) | 44,250 | 8,869 | 53,119 |
| Ronald Wooten(3) | 11,875 | — | 11,875 |

(1) Dr. Goldberg served as a director until September 28, 2022.

(2) Ms. Schafer served as a director until September 28, 2022.

(3) Mr. Wooten was appointed to the board on September 28, 2022.

(4) These amounts represent the aggregate grant date fair value of option awards made to each listed director in 2022 as computed in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718, "Stock Compensation," ("ASC 718"). These amounts do not represent the actual amounts paid to or realized by the directors during 2022. See Note 14 to the consolidated financial statements included in this Form 10-K regarding assumptions we made in determining the fair value of option awards. As of December 31, 2022, our non-employee directors held options to purchase shares of our common stock as follows: Dr. Csimma: 5,762; Mr. Dougherty: 5,762; Mr. Geraghty: 10,448; Dr. Goldberg: 0; Dr. Gowen: 5,915; Mr. Milano: 75,576; Ms. Schafer: 0; and Mr. Wooten: 0.

(5) Mr. Dougherty elected to receive shares of our common stock in lieu of $80,563 of cash compensation, and accordingly, received 10,781 shares.

**Narrative to Director Compensation Table**

Under our director compensation program, we pay our non-employee directors retainers in cash. Each director receives a cash retainer for service on the board and for service on each committee on which the director is a member. The chairperson of each committee receives higher retainers for such service. These fees are paid quarterly in arrears. The fees paid to non-employee directors for service on the board and for service on each committee of the board on which the director was a member during 2022 were as follows:

| | Member Annual Fee | Chairperson Annual Fee |
|---|---|---|
| Board of Directors | $40,000 | $70,000 |
| Audit Committee | $7,500 | $15,000 |
| Compensation Committee | $6,250 | $12,500 |
| Nominating and Corporate Governance Committee | $4,000 | $8,000 |
| Scientific Advisory Committee | $4,000 | $8,000 |

Our director compensation program includes a stock-for-fees policy, under which directors have the right to elect to receive common stock in lieu of cash fees. During 2022, these shares of common stock are issued under our

2013 Stock Incentive Plan, and going forward, they will be issued under our 2022 Stock Incentive Plan. The number of shares issued to participating directors is determined on a quarterly basis by dividing the cash fees to be paid through the issuance of common stock by the fair market value of our common stock, which is the closing price of our common stock, on the first business day of the quarter following the quarter in which the fees are earned. In 2022, several of our directors elected to receive shares of our common stock in lieu of cash fees as set forth in the footnotes to the Director Compensation table below.

Under our director compensation program, we also reimburse our directors for reasonable travel and other related expenses for attendance at meetings. Additionally, upon their initial election to the board, new non-employee directors receive an initial option grant to purchase 1,353 shares of our common stock (adjusted to reflect the effect of the Reverse Stock Split), and all non-employee directors receive an annual option grant to purchase 1,529 shares of our common stock (adjusted to reflect the effect of the Reverse Stock Split). The annual grants are made on the date of our annual meeting of stockholders and fully vest one year from that date of grant. The initial options granted to our non-employee directors vest with respect to one-third of the underlying shares on the first anniversary of the date of grant and the balance of the underlying shares vest in eight equal quarterly installments following the first anniversary of the date of grant, subject to continued service as a director. During 2022, these grants were made under our 2013 Stock Incentive Plan, and going forward, they will be made under our 2022 Stock Incentive Plan. These options are granted with exercise prices equal to the fair market value of our common stock, which is the closing price of our common stock, on the date of grant and will become immediately exercisable in full if there is a change in control of our Company.

## Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

**Securities Authorized for Issuance Under Equity Compensation Plans**

The following table provides information as of December 31, 2022 regarding total shares subject to outstanding stock options, warrants, and rights and total additional shares available for issuance under our existing equity incentive and employee stock purchase plans. In addition, from time to time, we may grant "inducement grants" pursuant to Nasdaq Listing Rule 5635(c)(4).

| Plan Category | Number of Securities to be Issued Upon Exercise of Outstanding Options, Warrants and Rights (a) | Weighted-Average Exercise Price of Outstanding Options, Warrants and Rights (b) | Remaining Available for Future Issuance Under Equity Compensation Plans (Excluding Securities Reflected in Column (a)) (c) |
|---|---|---|---|
| Equity compensation plans approved by stockholders (1) | 1,466,661 | $ 22.31 | 291,575 |
| Equity compensation plans not approved by stockholders (2) | 19,116 | $ 472.96 | — |
| Total | 1,485,777 | $ 28.30 | 291,575 |

(1) Consists of: Idera Pharmaceuticals, Inc. 2008 Stock Incentive Plan, Idera Pharmaceuticals, Inc. 2013 Stock Incentive Plan, Aceragen, Inc. 2021 Stock Incentive Plan, Idera Pharmaceuticals, Inc. 2022 Stock Incentive Plan, and Idera Pharmaceuticals, Inc. 2017 Employee Stock Purchase Plan. Amounts in column (a) include stock options and unvested restricted stock units outstanding.

Amounts in column (b) include shares available for issuance under Idera Pharmaceuticals, Inc. 2013 Stock Incentive Plan and Idera Pharmaceuticals, Inc. 2017 Employee Stock Purchase Plan. Following shareholder approval of Idera Pharmaceuticals, Inc. 2022 Stock Incentive Plan in January 2023, shares are available for future issuance only under Idera Pharmaceuticals, Inc. 2022 Stock Incentive Plan and Idera Pharmaceuticals, Inc. 2017 Employee Stock Purchase Plan.

The amount reported for number of shares issuable upon exercise and the weighted-average exercise price reflects, as applicable, the common stock equivalent exercise price for options exercisable for Series Z Preferred Stock as of December 31, 2022, that were automatically converted into options exercisable for common stock in January 2023.

(2) Consists of stock options issued as inducement grants (issued prior to 2017) as of December 31, 2022. These stock options are generally subject to the same terms and conditions as those awarded pursuant to the plans approved by our stockholders.

**Security Ownership of Certain Beneficial Owners and Management**

The following table sets forth, as of March 1, 2023 (except as otherwise indicated below), information we know about the beneficial ownership of our common stock by:

- each person or entity, including any "group" as that term is used in Section 13(d)(3) of the Exchange Act, who is known by us to own beneficially more than 5% of the issued and outstanding shares of our common stock;
- each of our current directors and director nominees;
- each of our named executive officers, as set forth in the Summary Compensation Table set forth in this proxy; and
- all of our current directors and executive officers as a group.

We have determined beneficial ownership in accordance with the rules of the SEC, and the information in the table below is not necessarily indicative of beneficial ownership for any other purpose. The SEC has defined "beneficial" ownership of a security to mean the possession, directly or indirectly, of voting power and/or investment power. In computing the percentage ownership of each person, shares of common stock subject to options, warrants, or

rights held by that person that are currently exercisable, or exercisable within 60 days of March 1, 2023, are deemed to be outstanding and beneficially owned by that person. These shares, however, are not deemed outstanding for the purpose of computing the percentage ownership of any other person.

To our knowledge and except as indicated in the notes to this table and pursuant to applicable community property laws, each stockholder named in the table has sole voting and investment power with respect to the shares set forth opposite such stockholder's name. The percentage of ownership is based on 8,408,966 shares of our common stock issued and outstanding on March 1, 2023. All fractional common share amounts have been rounded to the nearest whole number. To our knowledge, except as noted below, no person or entity is the beneficial owner of more than 5% of the voting power of the Company's stock.

| | Shares beneficially owned | |
|---|---|---|
| **Name and Address of Beneficial Owner[1]** | **Number** | **Percentage** |
| *5% Stockholders:* | | |
| NovaQuest Co-Investment Fund XV, L.P.<br>4208 Six Forks Road, Suite 920<br>Raleigh, North Carolina 27609 | 909,326 [2] | 9.8% |
| Pillar Investment Entities<br>c/o Stuarts Corporate Services Ltd.<br>Kensington House, 69 Dr. Roy's Drive<br>Georgetown, Grand Cayman KY1-1104<br>Cayman Islands | 985,204 [3] | 11.2% |
| Atul Chopra<br>4002 White Oak Trail<br>Beachwood, Ohio 44122 | 1,469,482 [4] | 17.5% |
| Daniel Salain | 1,469,482 | 17.5% |
| Andrew Jordan | 451,889 | 5.4% |
| *Company Officers and Directors:* | | |
| John Taylor | 1,469,482 | 17.5% |
| John J. Kirby | 39,420 [5] | * |
| Bryant D. Lim | 43,028 [6] | * |
| Vincent J. Milano | 65,431 [7] | * |
| Daniel Soland | 19,333 [8] | * |
| Cristina Csimma | 4,233 [9] | * |
| Michael R. Dougherty | 26,300 [10] | * |
| Maxine Gowen | 4,437 [11] | * |
| Ronald Wooten | 909,326 [12] | 9.8% |
| All current directors and executive officers as a group (10 individuals) | 4,605,316 [13] | 48.1% |

* Denotes less than 1% beneficial owner.

(1) Except as otherwise noted, the address for each person listed above is c/o Aceragen, Inc., 505 Eagleview Boulevard, Suite 212, Exton, PA 19341.

(2) Based on the Schedule 13D filed with the SEC on January 18, 2023 by NovaQuest Co-Investment Fund XV, L.P. ("NovaQuest"), which reported sole voting power with respect to zero shares, shared voting power with respect to 909,326 shares, sole dispositive power with respect to zero shares, and shared dispositive power with respect to 909,326 shares as of January 17, 2023.

(3) Based on Amendment No. 14 to the Schedule 13D filed with the SEC on January 30, 2023 by Pillar Pharmaceuticals 6, L.P. ("Pillar 6"), together with Pillar Invest Corporation ("Pillar GP"), Pillar Partners Foundation, L.P. ("Pillar Foundation," and, together with Pillar 6 and Pillar GP, the "Pillar Entities"), Abude Umari and Youssef El Zein (together with the Pillar Entities and Mr. Umari, the "Reporting Persons"), which reported sole voting power with respect to zero shares, shared voting power with respect to 985,204

shares, sole dispositive power with respect to zero shares, and shared dispositive power with respect 985,204 shares.

The Reporting Persons expressly disclaim status as a “group” for purposes of Amendment No. 14 to the Schedule 13D. The Pillar Entities exercise no voting or dispositive power over and expressly disclaim beneficial ownership of any shares held directly by Messrs. Umari and El Zein, and Messrs. Umari and El Zein expressly disclaim beneficial ownership of any shares of common stock held directly by Pillar 6, Pillar Foundation and indirectly by Pillar GP.

(4) Based on the Schedule 13D filed with the SEC on March 28, 2023 by Atul Chopra, MD, PhD, as trustee of the Chopra Revocable Trust, which reported sole voting power with respect to 1,469,482 shares, shared voting power with respect to 0 shares, sole dispositive power with respect to 1,469,482 shares, and shared dispositive power with respect to 0 shares as of January 17, 2023.

(5) Includes 20,070 shares of common stock subject to outstanding stock options that are exercisable within 60 days after March 1, 2023.

(6) Includes 24,811 shares of common stock subject to outstanding stock options that are exercisable within 60 days after March 1, 2023.

(7) Includes 53,704 shares of common stock subject to outstanding stock options that are exercisable within 60 days after March 1, 2023.

(8) Includes of 14,696 shares of common stock subject to outstanding stock options that are exercisable within 60 days after March 1, 2023.

(9) Consists of 4,233 shares of common stock subject to outstanding stock options that are exercisable within 60 days after March 1, 2023.

(10) Includes of 4,233 shares of common stock subject to outstanding stock options that are exercisable within 60 days after March 1, 2023.

(11) Includes 4,386 shares of common stock subject to outstanding stock options that are exercisable within 60 days after March 1, 2023.

(12) Consists of 909,326 shares beneficially owned by NovaQuest. Mr. Wooten is a member of the investment committee of NQ POF V GP, Ltd. ("NovaQuest GP"), which is the general partner of NovaQuest. NovaQuest GP has the power to vote and dispose of any securities directly owned by NovaQuest. NovaQuest GP's investment committee makes voting and investment decisions regarding securities held by NovaQuest. Mr. Wooten disclaims beneficial ownership of any securities held by NovaQuest except to the extent of its pecuniary interest therein.

(13) Includes 251,942 shares of common stock subject to outstanding stock options held by our current directors and executive officers as a group that are exercisable within 60 days after March 1, 2023.

## Item 13. Certain Relationships and Related Transactions, and Director Independence.

**Transactions with Related Persons**

Since January 1, 2021, we have not entered into or engaged in any related party transactions, as defined by the SEC, with our directors, officers, and stockholders who beneficially owned more than 5% of our outstanding common stock (‘‘5% holders’’), as well as affiliates or immediate family members of those directors, officers, and 5% holders, except with respect to the transactions described below.

*NovaQuest*

Pursuant to the Purchase Agreement, NovaQuest, a 5% holder and affiliate of our director, Ron Wooten, provided Legacy Aceragen $20.0 million in capital contributions for development funding of ACG-801 for the treatment of Farber disease which was fully paid by NovaQuest to Legacy Aceragen prior to the Aceragen Acquisition. We are not eligible for future capital contributions pursuant to the Purchase Agreement. The NovaQuest transaction includes tiered royalty payments on net sales based on a mid-double-digit percentage which drops to mid-single digits after reaching a predetermined milestone cap, and a required 35% share of the proceeds from the possible sale of a PRV, which may be awarded by the FDA upon regulatory approval in the U.S. for ACG-801.

In connection with the Aceragen Acquisition, NovaQuest was issued five shares of newly issued Series X Preferred Stock on the Acquisition Date. Additionally, warrants to purchase Legacy Aceragen common stock held by NovaQuest prior to the Aceragen Acquisition were assumed by us and converted into warrants to purchase 79,032 shares of the Company’s common stock and warrants to purchase 14,115 shares of Series Z Preferred Stock on terms substantially identical to those in effect prior to the Acquisition Date, except for adjustments to the underlying number of shares and the exercise price based on the Merger Agreement exchange ratio.

*Agreement with Dr. Atul Chopra*

In March 2021, Legacy Aceragen entered into a consulting agreement with Dr. Atul Chopra, a founder and a member of Legacy Aceragen’s board of directors, pursuant to which Dr. Chopra provides consulting and advisory services in exchange for (i) $16,667 per month and (ii) a right to purchase 1,000,000 fully vested shares of Legacy Aceragen’s common stock at a price equivalent to par value $0.001 per share. Subsequent to the executed consulting agreement, Dr. Chopra purchased all 1,000,000 shares, which were converted into shares of the Company’s common stock and Series Z Preferred Stock in connection with the Aceragen Acquisition based on the Merger Agreement exchange ratio. The term of the consulting agreement was to remain in effect for a period of one year automatically renew for successive one-year terms until terminated. At the effective time of the Aceragen Acquisition, the consulting agreement was terminated. Since March 2021 (inception of consulting agreement) through the termination of the agreement, Dr. Chopra received $316,667 in consulting fees pursuant to the agreement, of which $150,000 was received in 2022.

As of December 31, 2022, Dr. Chopra owned 127,718 shares of the Company’s common stock and 22,810 shares of Series Z Preferred Stock, which in January automatically converted into 1,341,764 shares of the Company’s common stock. Following the conversion of the Series Z Preferred Stock, Dr. Chopra owned 1,469,482 shares of the Company’s common stock.

*Warrant Exercises and Preferred Stock Conversions*

During March 2021, affiliates of Baker Brothers Advisors, LLC (collectively, ‘‘Baker Brothers’’) exercised warrants to purchase 159,342 shares of the Company’s common stock at an exercise price of $1.36 per share for a total exercise price of approximately $0.2 million. Additionally, Baker Brothers converted 14,150 shares Series B1 Preferred Stock into 83,235 shares of the Company’s common stock. In April 2021, Baker Brothers converted 9,534 shares Series B1 Preferred Stock into 56,082 shares of the Company’s common stock. At the time of such transaction, Baker Brothers was a 5% holder.

As discussed in further detail in Note 17 of the Notes to Financial Statements included in this Form 10-K, during the years ended December 31, 2022 and 2021, certain of the Pillar Entities exercised warrants to purchase 90,186 and 185,787 shares of the Company’s common stock, respectively, at an exercise price of $0.17 per share for a total exercise price of less than $0.1 million in the aggregate.

**Policies and Procedures for Related Person Transactions**

Our board is committed to upholding the highest legal and ethical conduct in fulfilling its responsibilities and recognizes that related party transactions can present a heightened risk of potential or actual conflicts of interest. Accordingly, as a general matter, it is our preference to avoid related party transactions.

In accordance with our audit committee charter, members of the audit committee, all of whom are independent directors, review and approve all related party transactions for which approval is required under applicable laws or regulations, including SEC and the Nasdaq Listing Rules. Current SEC rules define a related party transaction for smaller reporting companies to include any transaction, arrangement, or relationship in which we are a participant and the amount involved is the lesser of $120,000 or 1% of total assets, and in which any of the following persons has or will have a direct or indirect interest:

- our executive officers, directors, or director nominees;
- any person who is known to be the beneficial owner of more than 5% of our common stock
- any person who is an immediate family member, as defined under Item 404 of Regulation S-K, of any of our executive officers, directors, or director nominees or beneficial owners of more than 5% of our common stock; or
- any firm, corporation, or other entity in which any of the foregoing persons is employed or is a partner or principal or in a similar position or in which such person, together with any other of the foregoing persons, has a 5% or greater beneficial ownership interest.

Under our code of business conduct and ethics, our directors, officers, and employees are expected to avoid any relationship, influence or activity that would cause or even appear to cause a conflict of interest. Under our code of business conduct and ethics, a director is required to promptly disclose to our board any potential or actual conflict of interest involving him or her. In accordance with our code of business conduct and ethics, the board will determine an appropriate resolution on a case-by-case basis. All directors must recuse themselves from any discussion or decision affecting their personal, business, or professional interests. In addition, the audit committee is responsible for reviewing with our primary counsel the results of their review of the monitoring of compliance with our code of business conduct and ethics.

**Director Independence**

Our securities are listed on the Nasdaq Capital Market, and we use the standards of “independence” prescribed by rules set forth by Nasdaq. Under Nasdaq rules, a majority of a listed company’s board of directors must be comprised of independent directors. In addition, Nasdaq rules require that, subject to specified exceptions, each member of a listed company’s audit committee and compensation committee be independent and satisfy additional independence criteria set forth in Rules 10A-3 and 10C-1, respectively, under the Exchange Act. Under the applicable Nasdaq rules, a director will only qualify as an “independent director” if, in the opinion of our board, that person does not have a relationship which would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. Our board determined that each of Dr. Csimma, Mr. Dougherty, Dr. Gowen, and Mr. Wooten are independent as defined under applicable rules of the Nasdaq, and, in the case of all members of the audit and compensation committees, the independence requirements contemplated by Rule 10A-3 and Rule 10C-1 under the Exchange Act. Additionally, the board determined that Dr. Goldberg and Ms. Schafer, each of whom served on the board until September 28, 2022, and Mr. Geraghty, who served on the board until March 27, 2023, were independent. As Mr. Taylor is our President and Chief Executive Officer, he is not independent, and as Mr. Milano served as our President and Chief Executive Officer until September 28, 2022, he is not independent.

## Item 14. Principal Accountant Fees and Services.

**Independent Registered Public Accounting Firm Fees**

The following table sets forth all fees paid or accrued by us for professional services rendered by Ernst & Young LLP during the years ended December 31, 2022 and 2021:

| Fee Category | 2022 | 2021 |
|---|---|---|
| Audit Fees | $ 1,351,000 | $ 490,000 |
| Total Fees | $ 1,351,000 | $ 490,000 |

*Audit Fees*

Audit fees represent the aggregate fees for professional services rendered by our independent registered public accounting firm for the audit of our annual financial statements and review of our quarterly financial statements on Form 10-Q that are customary under the standards of the Public Company Accounting Oversight Board (United States). Also included are the fees for audit services related to (i) the Aceragen Acquisition and related filings through the closing date of the Aceragen Acquisition, (ii) the issuance of consents in connection with our registration statements on Form S-8 filed prior to and subsequent to the Aceragen Acquisition, (iii) comfort letters issued in connection with securities offerings, and (iv) technical accounting matters pertaining to the Aceragen Acquisition, which are non-recurring in nature.

**Audit Committee Pre-Approval Policies and Procedures**

Our audit committee has adopted policies and procedures relating to the approval of all audit and non-audit services that are to be performed by our independent registered public accounting firm. This policy generally provides that we will not engage our independent registered public accounting firm to render audit or non-audit services unless the service is specifically approved in advance by the audit committee, or the engagement is entered into pursuant to the pre-approval procedures described below.

From time to time, the audit committee may pre-approve specified types of services that are expected to be provided to us by our independent registered public accounting firm during the next 12 months. Any such pre-approval is detailed as to the particular service or type of services to be provided and is also generally subject to a maximum dollar amount. All of the services described above under the headings “Audit Fees” and “Audit-Related Fees” were pre-approved by our audit committee.

## PART IV.

## Item 15. Exhibits and Financial Statement Schedules.

(a) (1) *Financial Statements.*



(2) We are not filing any financial statement schedules as part of this Form 10-K because they are not applicable or the required information is included in the financial statements or notes thereto.

(3) The list of Exhibits filed as a part of this Form 10-K is set forth on the Exhibit Index below.

(b) The list of Exhibits filed as a part of this Form 10-K is set forth on the Exhibit Index below.

(c) None.

## Exhibit Index

| Exhibit Number | Description | Incorporated by Reference to | | | |
|---|---|---|---|---|---|
| | | Form | SEC File No. | Exhibit(s) | Filing Date |
| 1.1 | Equity Distribution Agreement, dated November 26, 2018, by and between Idera Pharmaceuticals, Inc. and JMP Securities LLC | 8-K | 001-31918 | 1.1 | November 26, 2018 |
| 2.1†† | Agreement and Plan of Merger, by and among Idera Pharmaceuticals, Inc., Bell Merger Sub I, Inc., Bell Merger Sub II, LLC, and Aceragen, Inc., dated September 28, 2022 | 8-K | 001-31918 | 2.1 | September 30, 2022 |
| 2.2†† | Agreement and Plan of Merger, by and among Aceragen, Inc., Aceragen Merger Sub, Inc., Arrevus, Inc., and Carl Kraus, dated October 18, 2021 | 10-Q | 001-31918 | 2.2 | November 14, 2022 |
| 3.1 | Restated Certificate of Incorporation of Idera Pharmaceuticals, Inc., as amended. | 10-Q | 001-31918 | 3.1 | August 2, 2018 |
| 3.2 | Certificate of Amendment to the Restated Certificate of Incorporation of Idera Pharmaceuticals, Inc. | 8-K | 001-31918 | 3.1 | May 18, 2020 |
| 3.3 | Certificate of Amendment to the Restated Certificate of Incorporation of Idera Pharmaceuticals, Inc. | 8-K | 001-31918 | 3.1 | January 17, 2023 |
| 3.4 | Second Amended and Restated Bylaws of Idera Pharmaceuticals, Inc. | 8-K | 001-31918 | 3.1 | November 17, 2022 |
| 3.5 | Certificate of Designations, Preferences and Rights of Series B1 Convertible Preferred Stock, Series B2 Convertible Preferred Stock, Series B3 Convertible Preferred Stock and Series B4 Convertible Preferred Stock of the Company | 8-K | 001-31918 | 3.1 | December 23, 2019 |
| 3.6 | Certificate of Designations of Series Z Non-Voting Convertible Preferred Stock | 8-K | 001-31918 | 3.1 | September 30, 2022 |
| 3.7 | Certificate of Designations of Series X Preferred Stock | 8-K | 001-31918 | 3.2 | September 30, 2022 |
| 3.8 | Certificate of Designation of Series B Preferred Stock of Idera Pharmaceuticals, Inc. | 8-K | 001-31918 | 3.2 | November 17, 2022 |
| 4.1 | Specimen Certificate for shares of Common Stock, $0.001 par value, of Idera Pharmaceuticals, Inc. | S-1 | 33-99024 | 4.1 | December 8, 1995 |
| 4.2 | Unit Purchase Agreement by and among Idera Pharmaceuticals, Inc. and certain persons and entities listed therein, dated April 1, 1998 | 10-K | 000-27352 | 10.39 | April 1, 2002 |
| 4.3 | Form of Warrant issued in May 2013 to purchasers in Idera Pharmaceuticals, Inc.'s registered public offering on Idera Pharmaceuticals, Inc.'s registration statement on Form S-1 (File No. 333-187155) | 10-Q | 001-31918 | 10.5 | May 15, 2013 |
| 4.4 | Form of Warrant issued in September 2013 to purchasers in Idera Pharmaceuticals, Inc.'s | 8-K | 001-31918 | 4.1 | September 26, 2013 |

| | | | | | |
|---|---|---|---|---|---|
| | registered public offering on Idera Pharmaceuticals, Inc.'s registration statement on Form S-3 (File No. 333-191073) | | | | |
| 4.5 | Form of Warrant issued in February 2014 to purchasers in Idera Pharmaceuticals, Inc.'s registered public offering on Idera Pharmaceuticals, Inc.'s registration statement on Form S-3 (File No. 333-191073) | 8-K | 001-31918 | 4.1 | February 5, 2014 |
| 4.6 | Form of Warrant issued in December 2019 to purchasers in Idera Pharmaceuticals, Inc. private placement transaction | 8-K | 001-31918 | 4.1 | December 23, 2019 |
| 4.7 | Warrant Amendment Agreement, dated as of December 23, 2019, by and among Idera Pharmaceuticals, Inc. and certain holders of warrants named therein | 10-K | 001-31918 | 4.13 | March 12, 2020 |
| 4.8 | Form of Pre-Funded Warrant issuable pursuant to the April 2020 Securities Purchase Agreement | 8-K | 001-31918 | 4.1 | April 7, 2020 |
| 4.9 | Form of Warrant issuable pursuant to the April 2020 Securities Purchase Agreement | 8-K | 001-31918 | 4.2 | April 7, 2020 |
| 4.10 | Form of Pre-Funded Warrant issuable pursuant to the July 2020 Securities Purchase Agreement | 8-K | 001-31918 | 4.1 | July 15, 2020 |
| 4.11 | Form of Warrant issuable pursuant to the July 2020 Securities Purchase Agreement | 8-K | 001-31918 | 4.2 | July 15, 2020 |
| 4.12 | Registration Rights Agreement, dated March 24, 2006, by and among Idera Pharmaceuticals, Inc. and the Investors named therein | 8-K | 001-31918 | 10.2 | March 29, 2006 |
| 4.13 | Registration Rights Agreement, dated February 9, 2015, among Idera Pharmaceuticals, Inc. and the Selling Stockholders named therein | 8-K | 001-31918 | 4.1 | February 9, 2015 |
| 4.14 | Amendment to the Registration Rights Agreement, dated January 21, 2018, by and among Idera Pharmaceuticals, Inc., 667, L.P., Baker Brothers Life Sciences, L.P. and 14159, L.P. | 8-K | 001-31918 | 10.1 | January 22, 2018 |
| 4.15 | Registration Rights Agreement, dated as of March 4, 2019, by and between Idera Pharmaceuticals, Inc. and Lincoln Park Capital Fund, LLC | 10-K | 001-31918 | 4.5 | March 6, 2019 |
| 4.16 | Registration Rights Agreement, dated December 23, 2019, by and among Idera Pharmaceuticals, Inc. and certain investors named therein | 10-K | 001-31918 | 4.11 | March 12, 2020 |
| 4.17 | Voting Agreement, dated as of December 23, 2019, by and among Idera Pharmaceuticals, Inc. and certain investors named therein | 10-K | 001-31918 | 4.12 | March 12, 2020 |

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|---|---|---|---|---|---|
| 4.18 | Registration Rights Agreement, dated April 7, 2020, by and among Idera Pharmaceuticals, Inc. and Pillar Partners Foundation, L.P. | 8-K | 001-31918 | 4.4 | April 7, 2020 |
| 4.19 | Voting Agreement, dated April 7, 2020, by and among Idera Pharmaceuticals, Inc. and Pillar Partners Foundation, L.P. | 8-K | 001-31918 | 4.3 | April 7, 2020 |
| 4.20 | Registration Rights Agreement, dated July 13, 2020, by and among Idera Pharmaceuticals, Inc. and Pillar Partners Foundation, L.P. | 8-K | 001-31918 | 4.3 | July 15, 2020 |
| 4.21 | Form of Idera Pharmaceuticals, Inc. Convertible Unsecured Promissory Notes | 8-K | 001-31918 | 4.1 | February 3, 2023 |
| 4.22* | Amended and Restated Warrant to Purchase Common Stock, by and between Aceragen, Inc. and NovaQuest Co-Investment Fund XV, L.P., dated March 30, 2023 | | | | |
| 4.23* | Description of the Aceragen, Inc. Securities Registered Under Section 12 of the Securities Exchange Act of 1934 | | | | |
| 10.1† | 2008 Stock Incentive Plan, as amended | 8-K | 001-31918 | 99.2 | June 17, 2011 |
| 10.2† | 2013 Stock Incentive Plan, as amended | 8-K | 001-31918 | 10.1 | June 13, 2014 |
| 10.3† | Amendment to 2013 Stock Incentive Plan, as amended | 8-K | 001-31918 | 10.1 | June 11, 2015 |
| 10.4† | Amendment to 2013 Stock Incentive Plan, as amended | 8-K | 001-31918 | 10.1 | June 9, 2017 |
| 10.5† | Amendment to 2013 Stock Incentive Plan, as amended | DEF14A | 001-31918 | Appendix A | April 25, 2019 |
| 10.6† | Amendment to 2013 Stock Incentive Plan, as amended | 8-K | 001-31918 | 10.1 | June 24, 2022 |
| 10.7† | 2017 Employee Stock Purchase Plan | 8-K | 001-31918 | 10.2 | June 9, 2017 |
| 10.8† | Amendment to 2017 Employee Stock Purchase Plan | DEF14A | 001-31918 | Appendix C | April 25, 2019 |
| 10.9† | Amendment to 2017 Employee Stock Purchase Plan, as amended | 8-K | 001-31918 | 10.2 | June 24, 2022 |
| 10.10† | Aceragen, Inc. 2021 Stock Incentive Plan and Forms of Award Agreements | 10-Q | 001-31918 | 10.14 | November 14, 2022 |
| 10.11† | First Amendment to Aceragen, Inc. 2021 Stock Incentive Plan Form of Stock Option Agreement | 10-Q | 001-31918 | 10.15 | November 14, 2022 |
| 10.12† | Idera Pharmaceuticals, Inc. 2022 Stock Incentive Plan | 8-K | 001-31918 | 10.1 | January 13, 2023 |
| 10.13 | Policy on Treatment of Stock Options in the Event of Retirement, approved April 28, 2014 | 10-Q | 001-31918 | 10.1 | August 12, 2014 |
| 10.14† | Form of Incentive Stock Option Agreement Granted Under the 2008 Stock Incentive Plan | 8-K | 001-31918 | 10.2 | June 10, 2008 |

| | | | | | |
|---|---|---|---|---|---|
| 10.15† | Form of Nonstatutory Stock Option Agreement Granted Under the 2008 Stock Incentive Plan | 8-K | 001-31918 | 10.3 | June 10, 2008 |
| 10.16† | Form of Nonstatutory Stock Option Agreement (Non-Employee Directors) Granted Under the 2008 Stock Incentive Plan | 8-K | 001-31918 | 10.4 | June 10, 2008 |
| 10.17† | Form of Restricted Stock Agreement Under the 2008 Stock Incentive Plan | 8-K | 001-31918 | 10.5 | June 10, 2008 |
| 10.18† | Form of Incentive Stock Option Agreement granted under the 2013 Stock Incentive Plan | 8-K | 001-31918 | 10.2 | July 29, 2013 |
| 10.19† | Form of Nonstatutory Stock Option Agreement granted under the 2013 Stock Incentive Plan | 8-K | 001-31918 | 10.3 | July 29, 2013 |
| 10.20† | Form of Nonstatutory Stock Option Agreement (Non-Employee Directors) granted under the 2013 Stock Incentive Plan | 8-K | 001-31918 | 10.4 | July 29, 2013 |
| 10.21† | Form of Inducement Stock Option Award – Nonstatutory Stock Option Agreement | 10-Q | 001-31918 | 10.1 | November 6, 2015 |
| 10.22† | Form of Restricted Stock Agreement under the 2013 Stock Incentive Plan | 10-Q | 001-31918 | 10.3 | August 8, 2019 |
| 10.23† | Form of Performance-Based Restricted Stock Agreement under the 2013 Stock Incentive Plan | 10-Q | 001-31918 | 10.3 | October 29, 2020 |
| 10.24†* | Form of Incentive Stock Option Agreement granted under the 2022 Stock Incentive Plan | | | | |
| 10.25†* | Form of Nonstatutory Stock Option Agreement granted under the 2022 Stock Incentive Plan | | | | |
| 10.26†* | Form of Nonstatutory Stock Option Agreement (Non-Employee Directors) granted under the 2022 Stock Incentive Plan | | | | |
| 10.27† | Employment Letter Agreement, dated December 1, 2014, by and between Idera Pharmaceuticals, Inc. and Vincent Milano | 10-K | 001-31918 | 10.24 | March 12, 2015 |
| 10.28† | Amendment to Employment Agreement, dated January 10, 2020, by and between the Company and Vincent J. Milano | 8-K | 001-31918 | 10.1 | January 15, 2020 |
| 10.29† | Executive Transition and Separation Agreement, by and among Vincent Milano and Idera Pharmaceuticals, Inc., dated September 28, 2022 | 8-K | 001-31918 | 10.1 | September 30, 2022 |
| 10.30†* | Amendment No. 1 to Executive Transition and Separation Agreement, by and among Vincent Milano and Aceragen, Inc., dated February 10, 2023 | | | | |
| 10.31† | Form of Vincent J. Milano Restricted Stock Unit Agreement | 8-K | 001-31918 | 10.2 | January 15, 2020 |
| 10.32† | Employment Letter, dated January 26, 2015, by and between Idera Pharmaceuticals, Inc. and Clayton Fletcher | 10-Q | 001-31918 | 10.1 | May 11, 2015 |

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|---|---|---|---|---|---|
| 10.33† | Consulting Agreement, dated December 29, 2020, between the Company and R. Clayton Fletcher | 8-K | 001-31918 | 10.1 | January 5, 2021 |
| 10.34† | Employment Offer Letter, dated October 15, 2015, by and between Idera Pharmaceuticals, Inc. and John J. Kirby | 10-K | 001-31918 | 10.26 | March 6, 2019 |
| 10.35† | Employment Continuation and Retention Bonus Letter Agreement, by and among John Kirby and Idera Pharmaceuticals, Inc., dated September 28, 2022 | 8-K | 001-31918 | 10.4 | September 30, 2022 |
| 10.36† | Employment Offer Letter, dated November 16, 2020, by and between Idera Pharmaceuticals, Inc. and Daniel Soland | 10-K | 001-31918 | 10.25 | March 1, 2021 |
| 10.37† | Severance and Change of Control Agreement, dated February 19, 2021, by and between the Company and Daniel Soland | 10-K | 001-31918 | 10.26 | March 1, 2021 |
| 10.38† | Executive Transition and Separation Agreement, by and among Daniel Soland and Idera Pharmaceuticals, Inc., dated September 28, 2022 | 8-K | 001-31918 | 10.6 | September 30, 2022 |
| 10.39†* | Amendment No. 1 to Executive Transition and Separation Agreement, by and among Daniel Soland and Aceragen, Inc., dated February 10, 2023 | | | | |
| 10.40† | Employment Offer Letter, dated August 20, 2018, by and between Idera Pharmaceuticals, Inc. and Bryant D. Lim | 10-Q | 001-31918 | 10.1 | November 6, 2018 |
| 10.41† | Employment Continuation and Retention Bonus Letter Agreement, by and among Bryant Lim and Idera Pharmaceuticals, Inc., dated September 28, 2022 | 8-K | 001-31918 | 10.5 | September 30, 2022 |
| 10.42† | Letter Agreement, by and among John Taylor and Aceragen, Inc., dated February 25, 2021 | 8-K | 001-31918 | 10.2 | September 30, 2022 |
| 10.43† | Letter Agreement, by and among Dan Salain and Aceragen, Inc., dated February 25, 2021 | 8-K | 001-31918 | 10.3 | September 30, 2022 |
| 10.44† | Form of Director and Officer Indemnification Agreement | 10-Q | 001-31918 | 10.1 | May 4, 2017 |
| 10.45† | Form of Executive Severance and Change of Control Agreement | 10-Q | 001-31918 | 10.2 | May 4, 2017 |
| 10.46†† | Development and Commercialization Agreement, dated May 1, 2014, by and between Abbott Molecular Inc. and Idera Pharmaceuticals, Inc. | 10-Q | 001-31918 | 10.3 | August 12, 2014 |
| 10.47†† | Clinical Trial Collaboration and Supply Agreement, by and between Idera Pharmaceuticals, Inc. and Bristol-Myers Squibb Company, dated May 18, 2018 | 10-Q | 001-31918 | 10.1 | August 2, 2018 |
| 10.48†† | Clinical Trial Collaboration and Supply Agreement, by and between Idera Pharmaceuticals, Inc. and Bristol-Myers Squibb Company, dated March 11, 2019 | 10-Q | 001-31918 | 10.1 | May 2, 2019 |

| | | | | | |
|---|---|---|---|---|---|
| 10.49†† | Clinical Trial Collaboration and Supply Agreement, effective August 27, 2019, by and between AbbVie Inc. and Idera Pharmaceuticals, Inc. | 10-Q | 001-31918 | 10.1 | November 6, 2019 |
| 10.50 | Lease Agreement dated March 31, 2015, between Idera Pharmaceuticals, Inc. and 505 Eagleview Boulevard Associates, L.P. | 10-K | 001-31918 | 10.45 | March 7, 2018 |
| 10.51 | First Amendment dated September 23, 2015 to Lease Agreement dated March 31, 2015 between Idera Pharmaceuticals, Inc. and 505 Eagleview Boulevard Associates, L.P. | 10-K | 001-31918 | 10.46 | March 7, 2018 |
| 10.52 | Second Amendment dated January 13, 2020 to Lease Agreement dated March 31, 2015 between Idera Pharmaceuticals, Inc. and 505 Eagleview Boulevard Associates, L.P. | 10-K | 001-31918 | 10.42 | March 12, 2020 |
| 10.53 | Purchase Agreement, dated as of March 4, 2019, by and between Idera Pharmaceuticals, Inc. and Lincoln Park Capital Fund, LLC | 10-K | 001-31918 | 10.37 | March 6, 2019 |
| 10.54 | First Amendment to Purchase Agreement, dated as of September 2, 2020, by and between Idera Pharmaceuticals, Inc. and Lincoln Park Capital Fund, LLC | 8-K | 001-31918 | 10.1 | September 3, 2020 |
| 10.55 | Securities Purchase Agreement, dated December 23, 2019, by and among the institutional investors named therein | 8-K | 001-31918 | 10.1 | December 23, 2019 |
| 10.56 | Securities Purchase Agreement, dated April 7, 2020, by and among Idera Pharmaceuticals, Inc. and Pillar Partners Foundation, L.P. | 8-K | 001-31918 | 10.1 | April 7, 2020 |
| 10.57 | Securities Purchase Agreement, dated July 13, 2020, by and among Idera Pharmaceuticals, Inc. and Pillar Partners Foundation, L.P. | 8-K | 001-31918 | 10.1 | July 15, 2020 |
| 10.58 | Amendment to the Securities Purchase Agreement and Registration Rights Agreement, dated December 11, 2020, by and among Idera Pharmaceuticals, Inc., Pillar Partners Foundation, L.P. and Pillar Pharmaceuticals 6, L.P. | 8-K | 001-31918 | 10.2 | December 15, 2020 |
| 10.59 | Side Letter Agreement, by and among Idera Pharmaceuticals, Inc., Bell Merger Sub II, LLC and NovaQuest Co-Investment Fund XV, L.P., dated September 28, 2022 | 10-Q | 001-31918 | 10.7 | November 14, 2022 |
| 10.60 | Stock and Warrant Purchase Agreement, by and between Aceragen, Inc. and NovaQuest Co-Investment Fund XV, L.P., dated March 24, 2021 | 10-Q | 001-31918 | 10.8 | November 14, 2022 |
| 10.61 | Amendment to Stock and Warrant Purchase Agreement, by and between Aceragen, Inc. and NovaQuest Co-Investment Fund XV, L.P., dated October 25, 2021 | 10-Q | 001-31918 | 10.9 | November 14, 2022 |
| 10.62 | Sales Distribution and PRV Agreement, by and between Aceragen, Inc. and NovaQuest | 10-Q | 001-31918 | 10.10 | November 14, 2022 |

| | | | | | |
|---|---|---|---|---|---|
| | Co-Investment Fund XV, L.P., dated October 25, 2021 | | | | |
| 10.63 | Therapeutic Development Award Agreement, by and between Arrevus, Inc. and Cystic Fibrosis Foundation, dated December 13, 2021 | 10-Q | 001-31918 | 10.11 | November 14, 2022 |
| 10.64 | Base Agreement, by and between Advanced Technology International and Arrevus, Inc., dated May 28, 2021 | 10-Q | 001-31918 | 10.12 | November 14, 2022 |
| 10.65 | Project Agreement No. 01, by and between Advanced Technology International and Arrevus, Inc., dated August 24, 2021 | 10-Q | 001-31918 | 10.13 | November 14, 2022 |
| 23.1* | Consent of Independent Registered Public Accounting Firm | | | | |
| 31.1* | Certification of Chief Executive Officer pursuant to Exchange Act Rules 13a-14 and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002 | | | | |
| 31.2* | Certification of Chief Financial Officer pursuant to Exchange Act Rules 13a-14 and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002 | | | | |
| 32.1* | Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 | | | | |
| 32.2* | Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 | | | | |
| 101.INS | Inline XBRL Instance Document | | | | |
| 101.SCH | Inline XBRL Taxonomy Extension Schema Document | | | | |
| 101.CAL | Inline XBRL Taxonomy Extension Calculation Linkbase Document | | | | |
| 101.DEF | Inline XBRL Taxonomy Extension Definition Linkbase Document | | | | |
| 101.LAB | Inline XBRL Taxonomy Extension Label Linkbase Document | | | | |
| 101.PRE | Inline XBRL Taxonomy Extension Presentation Linkbase Document | | | | |
| 104 | Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101) | | | | |

* Filed or furnished, as applicable, herewith.

† Management contract or compensatory plan or arrangement required to be filed as an Exhibit to the Form 10-K.

†† In accordance with Item 601(b)(10) of Regulation S-K, portions of this exhibit have been omitted in order for them to remain confidential.

## Item 16. Form 10-K Summary.

Not applicable.

**SIGNATURES**

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized, on this 13th day of April 2023.

Aceragen, Inc.

By: /S/ JOHN TAYLOR
John Taylor
*President and*
*Chief Executive Officer*

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this report has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated.

| Signature | Title | Date |
|---|---|---|
| /S/ JOHN TAYLOR<br>John Taylor | President, Chief Executive Officer and Director (Principal Executive Officer) | April 13, 2023 |
| /S/ JOHN J. KIRBY<br>John J. Kirby | Chief Financial Officer (Principal Financial and Accounting Officer) | April 13, 2023 |
| /S/ VINCENT J. MILANO<br>Vincent J. Milano | Chairman of the Board of Directors | April 13, 2023 |
| /S/ MICHAEL DOUGHERTY<br>Michael Dougherty | Director | April 13, 2023 |
| /S/ CRISTINA CSIMMA<br>Cristina Csimma, Pharm. D., M.H.P. | Director | April 13, 2023 |
| /S/ MAXINE GOWEN<br>Maxine Gowen, Ph.D. | Director | April 13, 2023 |
| /S/ RONALD WOOTEN<br>Ronald Wooten | Director | April 13, 2023 |

**ACERAGEN, INC. AND SUBSIDIARIES**

**INDEX TO CONSOLIDATED FINANCIAL STATEMENTS**
**December 31, 2022**

## REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Aceragen, Inc.

**Opinion on the Financial Statements**

We have audited the accompanying consolidated balance sheets of Aceragen, Inc. (the Company) as of December 31, 2022 and 2021, the related consolidated statements of operations and comprehensive income (loss), redeemable convertible preferred stock and stockholders' equity (deficit) and cash flows for each of the two years in the period ended December 31, 2022, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2022 and 2021, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2022, in conformity with U.S. generally accepted accounting principles.

**The Company's Ability to Continue as a Going Concern**

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations, has a working capital deficiency, and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

**Basis for Opinion**

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

**Critical Audit Matters**

The critical audit matters communicated below are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matters below, providing separate opinions on the critical audit matters or on the accounts or disclosures to which they relate.

***Valuation of intangible assets acquired in a business combination***

*Description of the Matter*

As discussed in Notes 2 and 3 of the consolidated financial statements, the Company acquired Aceragen, Inc., a privately held, biotechnology company in a transaction accounted for as a business combination. In connection with the acquisition, the Company recognized $71.6 million of in-process research and development intangible assets.

Auditing the Company's accounting for its acquisition was especially complex due to the significant estimation and judgment required by management in determining the fair value of the identifiable intangible assets, which consisted principally of in-process research and development. The Company used the multi-period excess earnings method of the income approach to measure the fair value of the in-process research and development. The estimation uncertainty was primarily due to the subjective nature of the significant inputs to the valuation model, including the relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population, estimated payments (e.g., royalty) and discount rate. Given the preclinical nature of the assets acquired, these significant assumptions are forward-looking and could be affected by future economic and market conditions.

*How We Addressed the Matter in Our Audit*

To test the estimated fair value of the in-process research and development intangible assets, we performed audit procedures that included, among others, evaluating the Company's use of the income approach (the multi-period excess earnings method), testing the significant assumptions used in the model, including the relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population, estimated payments (e.g., royalty) and discount rate, and assessing the completeness and accuracy of the underlying data. We compared the significant assumptions to current industry and market data, and to related data from comparable companies within the same industry. We involved our valuation professionals to assist with our evaluation of the methodology used by the Company and significant assumptions included in the fair value estimate.

***Valuation of Series X preferred stock liability***

*Description of the Matter*

As discussed in Notes 2, 3 and 4 of the consolidated financial statements, the Company issued shares of Series X preferred stock (Series X) in connection with the acquisition of Aceragen, Inc. The Series X represents a liability for accounting purposes for which the Company has elected the fair value option. The fair value of the Series X as of the date of acquisition and at December 31, 2022 was $31.9 million and $34.3 million, respectively.

Auditing the Company's valuation for the Series X was especially complex due to the significant estimation and judgment required by management in determining the fair value of the Series X. The Company used an income approach and Monte Carlo simulation method to measure the fair value of the Series X. The estimation uncertainty was primarily due to the subjective nature of the significant inputs to the valuation model, including the estimated sales proceeds related to the priority review voucher, the relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population, sales by region, estimated royalty payments and discount rate. These significant assumptions are forward-looking and could be affected by future economic and market conditions.

*How We Addressed the Matter in Our Audit*

To test the estimated fair value of the Series X, we performed audit procedures that included, among others, evaluating the Company's use of the income approach and the Monte Carlo simulation approach, testing the significant assumptions used in the model, including the estimated sales proceeds related to the priority review voucher, the relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population, sales by region, estimated royalty payments and discount rate, and assessing the completeness and accuracy of the underlying data. We compared the significant assumptions to current industry and market

data, to related data from comparable companies within the same industry and to underlying contracts. We involved our valuation professionals to assist with our evaluation of the methodology used by the Company and significant assumptions included in the fair value estimate.

/s/ ERNST & YOUNG LLP

We have served as the Company's auditor since 2002.

Philadelphia, Pennsylvania
April 13, 2023

# ACERAGEN, INC.
## Consolidated Balance Sheets
## As of December 31, 2022 and 2021

| (In thousands, except share and per share amounts) | December 31, 2022 | December 31, 2021 |
|---|---|---|
| **ASSETS** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 12,044 | $ 32,545 |
| Accounts receivable | 4,208 | — |
| Prepaid expenses and other current assets | 1,611 | 1,493 |
| Total current assets | 17,863 | 34,038 |
| Property and equipment, net | 7 | 22 |
| Intangible assets | 71,600 | — |
| Goodwill | 11,100 | — |
| Operating lease right-of-use assets | 537 | 734 |
| Other assets | — | 70 |
| Total assets | $ 101,107 | $ 34,864 |
| | | |
| **LIABILITIES, CONVERTIBLE REDEEMABLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY** | | |
| Current liabilities: | | |
| Accounts payable | $ 5,200 | $ 565 |
| Accrued expenses | 9,911 | 4,088 |
| Acquisition obligation, net | 6,078 | — |
| Operating lease liability | 234 | 209 |
| Total current liabilities | 21,423 | 4,862 |
| Warrant liability | 2,819 | — |
| Series X preferred stock liability (includes 5 shares of Series X convertible preferred stock, $0.01 par value per share issued and outstanding as December 31, 2022 - Note 8) | 34,300 | — |
| Operating lease liability, net of current portion | 326 | 549 |
| Deferred tax liability | 3,283 | — |
| Other liabilities | 22 | — |
| Total liabilities | 62,173 | 5,411 |
| | | |
| Commitments and contingencies (Note 15) | | |
| Preferred stock, $0.01 par value, Authorized — 5,000,000 shares: | | |
| Series Z convertible redeemable preferred stock (Note 9); Designated — 150,000 shares, Issued and outstanding — 77,900 shares at December 31, 2022 | 27,108 | — |
| | | |
| Stockholders' equity*: | | |
| Preferred stock, $0.01 par value, Authorized — 5,000,000 shares: | | |
| Series A convertible preferred stock; Designated — 1,500,000 shares; Issued and outstanding — 655 shares | — | — |
| Series B preferred stock; Designated — 200,000 shares; Issued and outstanding — 62,355 shares | 1 | — |
| Common stock, $0.001 par value, Authorized — 140,000,000 shares; Issued and outstanding — 3,653,685 and 3,106,947 at December 31, 2022 and December 31, 2021, respectively | 4 | 3 |
| Additional paid-in capital | 770,663 | 764,911 |
| Accumulated deficit | (758,821) | (735,461) |
| Accumulated other comprehensive income (loss) | (21) | — |
| Total stockholders' equity | 11,826 | 29,453 |
| Total liabilities, convertible redeemable preferred stock, and stockholders' equity | $ 101,107 | $ 34,864 |

* Reflects effect of retroactive application of reverse stock split (Note 1).

The accompanying notes are an integral part of these consolidated financial statements.

# ACERAGEN, INC.

## Consolidated Statements of Operations and Comprehensive Income (Loss)
## for the Years ended December 31, 2022 and 2021

| (In thousands, except share and per share amounts) | Year Ended December 31, 2022 | 2021 |
|---|---|---|
| Government contracts revenue | $ 4,862 | $ — |
| Operating expenses: | | |
| Research and development | 12,188 | 16,375 |
| General and administrative | 12,213 | 9,976 |
| Acquisition-related costs | 4,566 | — |
| Restructuring and other costs | 3,713 | 1,322 |
| Total operating expenses | 32,680 | 27,673 |
| Loss from operations | (27,818) | (27,673) |
| Other income (expense): | | |
| Interest income (expense), net | 204 | 2 |
| Warrant revaluation gain | 361 | 6,983 |
| Series X preferred stock liability loss | (2,400) | — |
| Future tranche right revaluation gain | — | 118,803 |
| Foreign currency exchange and other gain (loss), net | (25) | (24) |
| (Loss) income before income tax benefit | $ (29,678) | $ 98,091 |
| Income tax benefit | 6,318 | — |
| Net (loss) income | $ (23,360) | $ 98,091 |
| Undistributed earnings to preferred stockholders | — | (1,150) |
| Net (loss) income applicable to common stockholders | $ (23,360) | $ 96,941 |
| | | |
| Net (loss) income applicable to common stockholders (Note 18)* | | |
| — Basic | $ (23,360) | $ 96,941 |
| — Diluted | $ (23,360) | $ (28,845) |
| Net (loss) income per share applicable to common stockholders (Note 18)* | | |
| — Basic | $ (7.18) | $ 33.49 |
| — Diluted | $ (7.18) | $ (9.78) |
| Weighted-average number of common shares used in computing net (loss) income per share applicable to common stockholders* | | |
| — Basic | 3,255,648 | 2,894,287 |
| — Diluted | 3,255,648 | 2,948,659 |
| | | |
| Comprehensive (loss) income: | | |
| Net (loss) income | $ (23,360) | $ 98,091 |
| Other comprehensive income (loss), net of tax: | | |
| Foreign currency translation | (21) | — |
| Total other comprehensive (loss) income, net of tax | (21) | — |
| Total comprehensive (loss) income | $ (23,381) | $ 98,091 |

* Reflects effect of retroactive application of reverse stock split (Note 1).

The accompanying notes are an integral part of these consolidated financial statements.

# ACERAGEN, INC.

## Consolidated Statements of Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)
## for the Years ended December 31, 2022 and 2021

| (In thousands, except share and per share amounts) | Series B1 Preferred Number of Shares | Series B1 Preferred $0.01 Par Value | Series Z Preferred Number of Shares | Series Z Preferred $0.01 Par Value | Series B Preferred Number of Shares | Series B Preferred $0.01 Par Value | Common Stock Number of Shares* | Common Stock $0.001 Par Value* | Additional Paid-In Capital* | Accumulated Deficit | Other Comprehensive Income (Loss) | Total Stockholders' Equity (Deficit) |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **Balance, December 31, 2020** | 23,684 | $ — | — | $ — | — | $ — | 2,252,390 | $ 2 | $742,378 | $ (833,552) | $ — | $ (91,172) |
| Sale of common stock, net of issuance costs | — | — | — | — | — | — | 348,079 | 1 | 19,514 | — | — | 19,515 |
| Conversion of Series B1 preferred stock | (23,684) | — | — | — | — | — | 139,317 | — | — | — | | — |
| Issuance of common stock under employee stock purchase plan | — | — | — | — | — | — | 2,889 | — | 59 | — | — | 59 |
| Issuance of common stock under equity incentive plan (vesting of restricted stock units) | — | — | — | — | — | — | 13,927 | — | — | — | — | — |
| Issuance of common stock upon exercise of common stock options and warrants | — | — | — | — | — | — | 345,332 | — | 271 | — | — | 271 |
| Issuance of common stock for services rendered | — | — | — | — | — | — | 5,013 | — | 152 | — | — | 152 |
| Stock-based compensation | — | — | — | — | — | — | — | — | 2,537 | — | — | 2,537 |
| Net income | — | — | — | — | — | — | — | — | — | 98,091 | — | 98,091 |
| **Balance, December 31, 2021** | — | $ — | — | $ — | — | $ — | 3,106,947 | $ 3 | $764,911 | $ (735,461) | $ — | $ 29,453 |
| Sale of common stock, net of issuance costs | — | — | — | — | — | — | — | — | (15) | — | — | (15) |
| Issuance of common stock, preferred stock, restricted stock, options and warrants upon Acquisition of Aceragen (Note 3) | — | — | 77,663 | 27,108 | — | — | 434,845 | 1 | 3,427 | — | — | 3,428 |
| Vesting of restricted stock awards | — | — | 237 | — | — | — | 1,331 | — | — | — | — | — |
| Issuance of common stock under employee stock purchase plan | — | — | — | — | — | — | 7,788 | — | 49 | — | — | 49 |
| Common stock dividend issued in the form of Series B Preferred Stock | — | — | — | — | 62,355 | 1 | — | — | — | — | — | 1 |
| Issuance of common stock under equity incentive plan (vesting of restricted stock units) | — | — | — | — | — | — | 1,600 | — | — | — | — | — |
| Issuance of common stock upon exercise of common stock warrants | — | — | — | — | — | — | 90,185 | — | 15 | — | — | 15 |
| Issuance of common stock for services rendered | — | — | — | — | — | — | 10,989 | — | 88 | — | — | 88 |
| Stock-based compensation | — | — | — | — | — | — | — | — | 2,188 | — | — | 2,188 |
| Foreign currency translation | — | — | — | — | — | — | — | — | — | — | (21) | (21) |
| Net loss | — | — | — | — | — | — | — | — | — | (23,360) | — | (23,360) |
| **Balance, December 31, 2022** | — | $ — | 77,900 | $27,108 | 62,355 | $ 1 | 3,653,685 | $ 4 | $770,663 | $ (758,821) | $ (21) | $ 11,826 |

* Reflects effect of retroactive application of reverse stock split (Note 1).

The accompanying notes are an integral part of these consolidated financial statements.

# ACERAGEN, INC.
## Consolidated Statements of Cash Flows
## for the Years ended December 31, 2022 and 2021

| (In thousands) | Year Ended December 31, 2022 | 2021 |
|---|---|---|
| **Cash Flows from Operating Activities:** | | |
| Net (loss) income | $ (23,360) | $ 98,091 |
| Adjustments to reconcile net (loss) income to net cash used in operating activities: | | |
| Stock-based compensation | 2,188 | 2,537 |
| Foreign currency translation | 6 | — |
| Warrant liability revaluation gain | (361) | (6,983) |
| Series X preferred stock liability loss | 2,400 | — |
| Future tranche right liability revaluation gain | — | (118,803) |
| Issuance of common stock for services rendered | 88 | 152 |
| Accretion of discounts on short-term investments | — | (1) |
| Accretion of discounts on acquisition obligation | 66 | — |
| Depreciation and amortization expense | 15 | 22 |
| Deferred tax benefit | (6,318) | — |
| Changes in operating assets and liabilities, net of effects from Acquisition: | | |
| Accounts receivable | (2,294) | — |
| Prepaid expenses and other assets | 506 | 2,134 |
| Accounts payable, accrued expenses, and other liabilities | 2,548 | (1,751) |
| Other | 21 | 5 |
| Net cash used in operating activities | (24,495) | (24,597) |
| | | |
| **Cash Flows from Investing Activities:** | | |
| Cash acquired in acquisition of Aceragen | 5,482 | — |
| Proceeds from maturity of available-for-sale securities | — | 4,500 |
| Net cash provided by investing activities | 5,482 | 4,500 |
| | | |
| **Cash Flows from Financing Activities:** | | |
| Proceeds from common stock financings, net | (15) | 19,518 |
| Proceeds from employee stock purchases | 49 | 59 |
| Proceeds from exercise of common stock options and warrants | 15 | 271 |
| Payment on Acquisition Obligation | (1,534) | — |
| Payments on seller-financed purchases | — | (435) |
| Other | (3) | — |
| Net cash (used in) provided by financing activities | (1,488) | 19,413 |
| | | |
| Net decrease in cash and cash equivalents | (20,501) | (684) |
| Cash and cash equivalent, beginning of period | 32,545 | 33,229 |
| Cash and cash equivalents, end of period | $ 12,044 | $ 32,545 |
| | | |
| **Supplemental disclosure of cash flow information:** | | |
| Cash paid for interest | $ — | $ 5 |
| **Supplemental disclosure of non-cash financing and investing activities:** | | |
| Offering costs in accrued expenses | $ — | $ 3 |
| Non-cash seller-financed purchases | $ — | $ 652 |

The accompanying notes are an integral part of these consolidated financial statements.

# ACERAGEN, INC.

**Notes to Consolidated Financial Statements**
**December 31, 2022**

## Note 1. Business and Organization

***Business Overview***

Aceragen, Inc. ("Aceragen" or the "Company") (f/k/a Idera Pharmaceuticals, Inc. ("Idera")), a Delaware corporation, is a clinical-stage biopharmaceutical company with a business strategy focused on the clinical development, and ultimately the commercialization, of drug candidates for rare disease indications characterized by small, well-defined patient populations with serious unmet medical needs. The Company's current focus is to develop and optimize commercial value of ACG-701 (patented formulation of sodium fusidate) and ACG-801 (recombinant human acid ceramidase (rhAC)) for appropriate patients. The Company has in the past and may in the future explore clinical funding arrangements and collaborative alliances to support development and commercialization of any of its drug candidates. The Company may also seek to identify and potentially acquire rights to novel development or commercial stage rare disease programs, through new business development opportunities, including additional strategic alternatives.

On September 28, 2022 (the "Effective Date"), Idera acquired Aceragen, Inc. and its wholly owned subsidiaries ("Legacy Aceragen"), in accordance with the terms of the Agreement and Plan of Merger, dated as of the Effective Date (the "Merger Agreement"). Legacy Aceragen was a privately-held biotechnology company addressing severe, rare, and orphan pulmonary and rheumatic diseases for which there are limited or no available treatments. The Company acquired Legacy Aceragen as a strategic extension of its rare disease business and focus with the primary objective of further developing Legacy Aceragen's portfolio of rare disease product candidates.

Following the Special Meeting of Stockholders held on January 12, 2023 (the "Special Meeting"), Idera's name was changed to Aceragen, Inc. (the "Merger" and, together with the other transactions contemplated by the Merger Agreement, the "Aceragen Acquisition"). See Note 3, "Business Acquisition," for additional information on the Aceragen Acquisition.

Prior to December 2021, the Company was developing a toll-like receptor agonist, tilsotolimod (IMO-2125), for oncology indications. In December 2021, all Company-sponsored development of tilsotolimod was discontinued and all study-related activities have subsequently been concluded. However, the Company is considering the potential for out-licensing arrangements so that tilsotolimod's full potential might continue to be explored on behalf of patients who did not respond to traditional immunotherapy, together with other alternatives.

***Liquidity and Financial Condition***

The Company follows the provisions of Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") 205-40, *Presentation of Financial Statements—Going Concern*, which requires management to assess the Company's ability to continue as a going concern within one year after the date the consolidated financial statements are issued.

The Company has incurred substantial losses and negative cash flows from operations since its inception and had an accumulated deficit of $758.8 million as of December 31, 2022. The Company's cash and cash equivalents balance of $12.0 million as of December 31, 2022 is not sufficient to fund its operations for the one-year period after the date the financial statements are issued. These factors raise substantial doubt about the Company's ability to continue as a going concern. The accompanying consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The consolidated financial statements do not include any adjustments related to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty.

The Company anticipates incurring additional losses until such time, if ever, that it can generate significant sales of its product candidates currently in development. The Company is subject to a number of risks and uncertainties

similar to those of other companies of the same size within the biotechnology industry, such as uncertainty of clinical trial outcomes, uncertainty of additional funding, and history of operating losses. Substantial additional financing will be needed by the Company to fund its operations and to commercially develop its product candidates. Management is currently evaluating different strategies to obtain the required funding for future operations. These strategies may include, but are not limited to: product development financing, private placements and/or public offerings of equity and/or debt securities, payments from potential strategic research and development collaborations and/or similar arrangements, and payments from the potential sale and/or licensing of technology assets. There can be no assurance that these future funding efforts will be successful. Accordingly, management has concluded that substantial doubt exists with respect to the Company's ability to continue as a going concern within one year after the date that these financial statements are issued.

***Reverse Stock Split***

As further described in Note 19, on January 17, 2023, the Company effected a 1-for-17 reverse stock split of the Company's outstanding shares of common stock, as approved by the Company's stockholders at the Special Meeting. All share and per share amounts of common stock, options, warrants, restricted stock, restricted stock units, and conversion ratio of convertible preferred stock and convertible preferred stock warrants in the accompanying consolidated financial statements and notes thereto have been retroactively adjusted for all periods presented to reflect the reverse stock split.

**Note 2. Summary of Significant Accounting Policies**

***Basis of Presentation***

The accompanying consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB"). The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiary, Aceragen, LLC, including Aceragen, LLC's wholly owned subsidiaries, Arrevus, Inc., a Delaware Corporation ("Arrevus"), and Aceragen GmbH, a limited liability company ("AGmbH"). All intercompany accounts and transactions have been eliminated in consolidation. The Company has determined the functional currency of AGmbH to be the Swiss Franc. The Company translates assets and liabilities of AGmbH's operations at exchange rates in effect at the balance sheet date with the resulting translation adjustments directly recorded as a separate component of accumulated other comprehensive income. Income and expense accounts are translated at average exchange rates for the period. Transactions which are not in the functional currency of AGmbH are remeasured into the functional currency and gains and losses resulting from the remeasurement are recorded in foreign currency exchange and other gain (loss), net.

***Use of Estimates***

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates, judgements, and assumptions that affect the reported amounts of assets and liabilities at the date of consolidated financial statements and reported amounts of revenues and expenses during the reporting period, and related disclosure of contingencies in the accompanying consolidated financial statements and these notes. In addition, management's assessment of the Company's ability to continue as a going concern involves the estimation of the amount and timing of future cash inflows and outflows. The Company bases its estimates and assumptions on historical experience when available and on various factors that it believes to be reasonable under the circumstances and are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from these estimates. Estimates that are critical to the accompanying consolidated financial statements include the estimated fair value of the net assets acquired in connection with the Aceragen Acquisition, the estimated fair value of the liability classified warrants issued to Legacy Aceragen warrant holders, Series X Preferred Stock (Note 8), and accrued clinical trial expenses.

***Segment Information***

Operating segments are defined as components of an enterprise in which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and assessing performance. The Company views its operations and manages its business as one operating segment, which is the business of developing novel therapeutics for rare diseases.

***Financial Instruments***

The fair value of the Company's financial instruments is determined and disclosed in accordance with the three-tier fair value hierarchy specified in Note 4. The Company is required to disclose the estimated fair values of its financial instruments. As of December 31, 2022, the Company's financial instruments included cash and cash equivalents, accounts receivable, accounts payable, Acquisition Obligation (defined below), and Series X Preferred Stock and Series Z Preferred Stock Warrant liabilities. As of December 31, 2021, the Company's financial instruments consisted of cash and cash equivalents. The carrying amount of cash and cash equivalents, accounts receivable, and accounts payable approximates fair value due to the short-term maturities of these instruments. The carrying values of the Acquisition Obligation (defined below), Series X Preferred Stock liability and Series Z Preferred Stock Warrants liability are recorded at their estimated fair values. As of December 31, 2022, the Company did not have any other derivatives, hedging instruments or other similar financial instruments.

***Concentration of Credit Risk***

Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash, which, at times, may exceed federally insured limits, and cash equivalents consisting of investments in money market funds managed by a variety of financial institutions. The Company's credit risk is managed by investing in only highly rated money market instruments. As a result, no significant additional credit risk is believed by management to be inherent in the Company's assets and the Company has not experienced any losses in such accounts and believes it is not exposed to any significant risk on such accounts. As of December 31, 2022, the Company's cash and cash equivalents were held at six financial institutions.

As more fully described in Note 19, "Subsequent Events" the Company had approximately 56% of its cash and cash equivalent balances in segregated custodial accounts held by a third-party custodian for which SVB (as defined below) was the Company's agent and/or SVB Asset Management, an affiliate of SVB, was the advisor at the time SVB was closed. The Company does not believe it will be impacted by the closure of SVB.

***Business Combinations***

The Company evaluates acquisitions of assets and other similar transactions to assess whether or not the transaction should be accounted for as a business combination or asset acquisition by first applying a screen to determine if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets. If the screen is met, the transaction is accounted for as an asset acquisition. If the screen is not met, further determination is required as to whether or not the Company has acquired inputs and processes that have the ability to create outputs, which would meet the requirements of a business. If determined to be a business combination, the Company accounts for the transaction under the acquisition method of accounting as indicated in ASU 2017-01, *Business Combinations (ASC 805)*, which requires the acquiring entity in a business combination to recognize the fair value of all assets acquired, liabilities assumed, and any non-controlling interest in the acquiree and establishes the acquisition date as the fair value measurement point. Accordingly, the Company recognizes assets acquired and liabilities assumed in business combinations based on the fair value estimates as of the date of acquisition. In accordance with ASC 805, *Business Combinations*, the Company recognizes and measures goodwill as of the acquisition date, as the excess of the fair value of the consideration paid over the fair value of the identified net assets acquired.

***Cash and Cash Equivalents***

The Company considers all highly liquid investments with maturities of 90 days or less when purchased to be "cash equivalents." Cash and cash equivalents at December 31, 2022 and 2021 consisted of cash and money market funds.

***Accounts Receivable***

The U.S. Government accounted for all of the Company's accounts receivable as of December 31, 2022. Accordingly, the Company does not expect any credit losses with respect to its accounts receivable and no credit losses have been incurred to date. Included in accounts receivable at December 31, 2022 is $0.4 million of unbilled receivables which relates to revenue recognized for work that has been performed but the invoicing has not yet occurred as of the reporting date.

***Property and Equipment***

Property and equipment are carried at acquisition cost less accumulated depreciation, subject to review for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be recoverable as described further under the heading "Impairment of Long-Lived Assets" below. The cost of normal, recurring, or periodic repairs and maintenance activities related to property and equipment are expensed as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if the repair will result in future economic benefits.

Depreciation and amortization are computed using the straight-line method based on the estimated useful lives of the related assets. Leasehold improvements are amortized over the remaining lease term or the related useful life, if shorter. Equipment and other long-lived assets are depreciated over three to five years.

When an asset is disposed of, the associated cost and accumulated depreciation is removed from the related accounts on the Company's balance sheet with any resulting gain or loss included in the Company's consolidated statement of operations.

***Indefinite-Lived Intangible Assets***

Indefinite-lived intangible assets consist of In-Process Research and Development ("IPR&D"). The fair values of IPR&D project assets acquired in business combinations are capitalized. The Company generally utilizes the Multi-Period Excess Earning Method to determine the estimated fair value of the IPR&D assets acquired in a business combination. The projections used in this valuation approach are based on many factors, such as relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population, and estimated payments (e.g., royalty). The estimated future net cash flows are then discounted to the present value using an appropriate discount rate. These assets are treated as indefinite-lived intangible assets until completion or abandonment of the projects, at which time the assets are amortized over the remaining useful life or written off, as appropriate.

Intangible assets with indefinite lives, including IPR&D, are tested for impairment if impairment indicators arise and, at a minimum, annually. However, an entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that an indefinite-lived intangible asset's fair value is less than its carrying amount. Otherwise, no further impairment testing is required. The indefinite-lived intangible asset impairment test consists of a one-step analysis that compares the fair value of the intangible asset with its carrying amount. If the carrying amount of an intangible asset exceeds its fair value, an impairment loss is recognized in an amount equal to that excess. The Company considers many factors in evaluating whether the value of our intangible assets with indefinite lives may not be recoverable, including, but not limited to, expected growth rates, the cost of equity and debt capital, general economic conditions, our outlook and market performance of our industry and recent and forecasted financial performance.

The Company evaluates indefinite-lived intangible assets for impairment at least annually on October 1 and whenever facts and circumstances indicate that their carrying amounts may not be recoverable. For the year ended December 31, 2022, the Company determined that there was no impairment to IPR&D.

***Goodwill***

Goodwill represents the amount of consideration paid in excess of the fair value of net assets acquired as a result of the Company's business acquisitions accounted for using the acquisition method of accounting. The intangible assets acquired represented the fair value of IPR&D which has been recorded on the accompanying consolidated balance sheet as indefinite-lived intangible assets. A deferred tax liability was recorded for the difference between the fair value of the acquired IPR&D and its tax basis which was recognized as goodwill in applying the purchase method of accounting. Goodwill is not amortized and is subject to impairment testing at a reporting unit level on an annual basis or when a triggering event occurs that may indicate the carrying value of the goodwill is impaired. An entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that the fair value of the reporting unit is less than its carrying amount.

The Company evaluates goodwill for impairment at least annually on October 1 and whenever facts and circumstances indicate that their carrying amounts may not be recoverable. For the year ended December 31, 2022, the Company determined that there was no impairment to goodwill.

***Operating Lease Right-of-use Asset and Lease Liability***

The Company accounts for leases under ASC 842, *Leases*. Operating leases are included in "Operating lease right-of-use assets" within the Company's consolidated balance sheets and represent the Company's right to use an underlying asset for the lease term. The Company's related obligation to make lease payments are included in "Operating lease liability" and "Operating lease liability, net of current portion" within the Company's consolidated balance sheets. Operating lease right-of-use ("ROU") assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term. As most of the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rates, which are the rates incurred to borrow on a collateralized basis over a similar term, an amount equal to the lease payments in a similar economic environment. Lease expense for lease payments is recognized on a straight-line basis over the lease term. The ROU assets are tested for impairment according to ASC 360, *Property, Plant, and Equipment* ("ASC 360"). Leases with an initial term of 12 months or less are not recorded on the balance sheet and are recognized as lease expense on a straight-line basis over the lease term.

As of December 31, 2022 and 2021, the Company's operating lease ROU assets and corresponding short-term and long-term lease liabilities primarily relate to its existing Exton, Pennsylvania facility operating lease, which expires on May 31, 2025. In connection with the Aceragen Acquisition, the Company acquired an operating lease for an office in Basel, Switzerland, which expired on March 31, 2023.

***Impairment of Long-Lived Assets***

In accordance with ASC 360-10-35, *Impairment or Disposal of Long-Lived Assets*, the Company reviews its long-lived assets for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable (i.e., impaired). Once an impairment is determined, the actual impairment recognized is the difference between the carrying amount and the fair value (less costs to sell for assets to be disposed of) as estimated using one of the following approaches: income, cost, and/or market. Fair value using the income approach is determined primarily using a discounted cash flow model that uses the estimated cash flows associated with the asset or asset group under review, discounted at a rate commensurate with the risk involved. Fair value utilizing the cost approach is determined based on the replacement cost of the asset reduced for, among other things, depreciation and obsolescence. Fair value, utilizing the market approach, benchmarks the fair value against the carrying amount.

***Warrant Liability***

The Company accounts for stock warrants as either equity instruments, liabilities or derivative liabilities in accordance with ASC 480, *Distinguishing Liabilities from Equity* ("ASC 480") and/or ASC 815, *Derivatives and Hedging* ("ASC 815"), depending on the specific terms of the warrant agreement. Freestanding warrants for shares that are potentially redeemable, whereby the Company may be required to transfer assets (e.g., cash or other assets) outside of its control, are classified as liabilities. Liability-classified warrants are recorded at their estimated fair values at each reporting period until they are exercised, terminated, reclassified or otherwise settled. Changes in the estimated fair value of liability-classified warrants are recorded in "warrant revaluation gain (loss)" in the Company's consolidated statements of operations. Equity classified warrants are recorded within additional paid-in capital at the time of issuance and not subject to remeasurement.

In connection with the Aceragen Acquisition, a portion of the consideration paid to Legacy Aceragen warrant holders was in the form of warrants to purchase shares of Series Z Preferred Stock ("Series Z Warrants"). Such warrants were classified as liabilities upon issuance and as of December 31, 2022 because the underlying Series Z Preferred Stock is contingently redeemable. The fair value of the Series Z Warrants on the date of issuance was recorded as a component to the carrying value of the shares Series Z Preferred Stock and as a long-term liability in the consolidated balance sheets. The Series Z Warrants are remeasured to fair value at each balance sheet date until the warrants are exercised, reclassified, expire, or otherwise settled. Changes in the fair values of the Series Z Warrants are recognized as other income or expense in the consolidated statements of operations and comprehensive loss. See Notes 3, 4, and 9 to these consolidated financial statements for further details.

***Redeemable Preferred Stock***

The Company applies ASC 480 when determining the classification and measurement of its preferred stock. Preferred shares subject to mandatory redemption are classified as liability instruments and are measured at fair value. Conditionally redeemable preferred shares (including preferred shares that feature redemption rights that are either within the control of the holder or subject to redemption upon the occurrence of uncertain events not solely within the Company's control) are classified as temporary equity. At all other times, preferred shares are classified as stockholders' equity. See Notes 8, 9 and 10 to these consolidated financial statements.

**Series X Preferred Stock Liability**

In conjunction with the Aceragen Acquisition, the Company evaluated the newly issued Series X Preferred Stock and determined its revised terms represents a sale of future revenues and is classified as a liability under ASC 470, *Debt* and the Company has elected to account for the Series X Preferred Stock liability under the fair value option. The fair value of the Series X Preferred Stock liability represents the present value of estimated future payments, including royalty payments, as well as estimated payments that are contingent upon the achievement of specified milestones. The fair value of the Series X Preferred Stock liability is based on the cumulative probability of the various estimated payments. The fair value measurement is based on significant Level 3 unobservable inputs which are further described in Note 4. Any changes in the fair value of the liability in each reporting period are recognized in the consolidated statements of operations until it is settled. See Note 8 to these consolidated financial statements for further discussion of the Series X Preferred Stock Liability.

***Future Tranche Right Liability and Revaluation Gain***

The December 2019 Securities Purchase Agreement (as defined in Note 9) contained call options on redeemable preferred shares with warrants (conditionally exercisable for shares that are puttable). The Company determined that these call options represent freestanding financial instruments and accounted for the options as liabilities under ASC 480, which required the measurement and recognition of the fair value of the liability at the time of issuance and at each reporting period until such call options were exercised or cancelled. During the year ended December 31, 2021, the liability-classified call options provided for under the December 2019 Securities Purchase Agreement terminated and, accordingly, the liability balance was derecognized resulting in a future tranche right revaluation gain recorded in the Company's statements of operations.

***Revenue Recognition***

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* ("ASC 606"), which applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. In accordance with ASC 606, the Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that the Company determines are within the scope of ASC 606, it performs the following five steps:

- identify the contract(s) with a customer;
- identify the performance obligations in the contract;
- determine the transaction price;
- allocate the transaction price to the performance obligations in the contract; and
- recognize revenue when (or as) the entity satisfies a performance obligation.

The Company only applies the five-step model to contracts when it determines that it is probable it will collect the consideration to which it is entitled in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Amounts received prior to satisfying the revenue recognition criteria are recognized as deferred revenue in the Company's balance sheet. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current portion of deferred revenue. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

*Government Contract Revenue*

Revenues from reimbursable contracts are recognized as costs are incurred, generally based on allowable direct costs incurred during the period, plus allocable overheads together with any recognizable earned fee. The Company uses this output method to measure progress as the customer has access to the development research under these projects and benefits incrementally as research and development activities occur.

See Note 11, "Government Contracts Revenue," of the notes to these consolidated financial statements for discussion of the Company's cost reimbursement contracts.

*Other Revenues*

Certain of the Company's collaborative research, development, and/or commercialization agreements may result in the recognition of revenue for one or more of the following: nonrefundable, up-front license fees; research, development, and commercial milestone payments; and other contingent payments due based on the activities of the counterparty or the reimbursement by licensees of costs associated with patent maintenance. See Note 12, "Clinical Funding, Collaboration and License Agreements," of the notes to these consolidated financial statements for additional details regarding the Company's collaboration and out-licensing arrangements.

*Customer Concentration Risk*

The U.S. Government accounted for all of the Company's revenues for the year ended December 31, 2022.

***Research and Development Prepayments, Accruals and Related Expenses***

All research and development expenses are expensed as incurred. Research and development expenses are comprised of costs incurred in performing research and development activities, including drug development trials and studies, research collaborations, drug manufacturing, laboratory supplies, external research, payroll including stock-based compensation and overhead. The Company is required to estimate our accrued and prepared expenses for research and development activities performed by third parties, including Clinical Research Organizations ("CROs") and clinical investigators. These estimates are made as of the reporting date of the work completed over the life of the individual study in accordance with agreements established with CROs and other clinical sites. Some CROs invoice the Company on a monthly basis, while others invoice upon the achievement of milestones. The Company determines the estimates of research and development activities incurred at the end of each reporting period through discussion with internal personnel, outside service providers, and research collaboration partners as to the progress or stage of completion of trials or services, as of the end of the reporting period, pursuant to contracts with clinical trial centers or CROs and the agreed upon fee to be paid for such services. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are accepted by the Company or the services are performed. As of December 31, 2022 and 2021, the Company recorded approximately $0.6 million and $0.9 million, respectively, as prepaid research and development, which is included within prepaid expenses and other current assets in the accompanying balance sheets.

***Acquisition-Related Costs***

Acquisition-related costs include direct expenses incurred in connection with the Aceragen Acquisition, as well as integration-related professional fees and other incremental costs directly associated to the Aceragen Acquisition.

***Stock-Based Compensation***

The Company accounts for stock-based compensation using ASC 718, *Compensation – Stock Compensation*, or ASC 505-50, *Equity – Equity Based Payments to Non-Employees*, as applicable. The Company accounts for stock-based awards to employees and non-employee directors using the fair value-based method to determine compensation expense for all arrangements where shares of stock or equity instruments are issued for compensation.

The Company recognizes all share-based payments to employees and directors as expense in the statements of operations based on their fair values. The Company records compensation expense on a straight-line basis over an award's requisite service period, or vesting period, based on the award's fair value at the date of grant. Vesting for time-based options and restricted stock units is generally four years for employees and one year for directors. The Company uses a Black-Scholes option-pricing model to determine the fair value of each option grant as of the date of grant for expense incurred. The Black-Scholes option pricing model requires inputs for risk-free interest rate, dividend yield, expected stock price volatility and expected term of the options. Forfeitures are accounted for as they occur. See Note 14, "Stock-based Compensation," for additional details.

***Income Taxes***

An asset and liability approach is used for financial accounting and reporting for income taxes. Deferred income taxes arise from temporary differences between income tax and financial reporting and principally relate to recognition of revenue and expenses in different periods for financial and tax accounting purposes and are measured using currently enacted tax rates and laws. In addition, a deferred tax asset can be generated by a net operating loss carryover. If it is more likely than not that some portion or all of a deferred tax asset will not be realized, a valuation allowance is recognized.

In the event the Company is charged interest or penalties related to income tax matters, the Company would record such interest as interest expense and would record such penalties as other expense in the Statements of Operations. No such charges have been incurred by the Company. For each of the years ended December 31, 2022 and 2021, the Company had no uncertain tax positions. See Note 16, "Income Taxes," for additional details.

***Net Income (Loss) per Common Share Applicable to Common Stockholders***

The Company uses the two-class method to compute net income per common share during periods the Company realizes net income and has securities outstanding (e.g., redeemable convertible preferred stock) that entitle the holder to participate in dividends and earnings of the Company. In addition, the Company analyzes the potential dilutive effect of outstanding redeemable convertible preferred stock under the "if-converted" method when calculating diluted earnings per share and reports the more dilutive of the approaches (two class or "if-converted"). The two-class method is not applicable during periods with a net loss, as the holders of the redeemable convertible preferred stock have no obligation to fund losses. The Company also analyzes the potential dilutive effect of outstanding stock options, unvested restricted stock and restricted stock units, warrants and shares underlying future tranche rights under the treasury stock method (as applicable), during periods of income, or during periods in which income is recognized related to changes in fair value of its liability-classified securities.

***New Accounting Pronouncements***

From time to time, new accounting pronouncements are issued by the FASB and rules are issued by the Securities and Exchange Commission (“SEC”) that the Company has or will adopt as of a specified date. Unless otherwise noted, management does not believe that any other recently issued accounting pronouncements issued by the FASB or guidance issued by the SEC had, or is expected to have, a material impact on the Company’s present or future financial statements.

**Note 3. Business Acquisition**

On the Effective Date, and in accordance with the terms of the Merger Agreement, the Company acquired 100% of the outstanding security interests of Legacy Aceragen in a “stock-for-stock” transaction whereby all Legacy Aceragen outstanding equity interests were exchanged for a combination of shares of Company common stock, shares of Series Z Preferred Stock, and shares of the newly designated Series X non-voting preferred stock, par value $0.01 per share (“Series X Preferred Stock”). Under the terms of the Merger Agreement, Legacy Aceragen stockholders received (i) 451,608 shares of the Company’s common stock (inclusive of unvested restricted common stock – see Note 14), (ii) 80,656 shares of Series Z Preferred Stock (inclusive of unvested restricted preferred stock – see Note 14) and (iii) five shares of Series X Preferred Stock. In addition, all outstanding options and warrants to purchase Legacy Aceragen common stock were assumed by the Company and converted into stock options and warrants to purchase shares of the Company’s common stock and Series Z Preferred Stock on terms substantially identical to those in effect prior to the Aceragen Acquisition, except for adjustments to the underlying number of shares and the exercise price based on the Merger Agreement exchange ratio. The Aceragen Acquisition was unanimously approved by the board of directors of the Company and the board of directors of Legacy Aceragen. The closing of the transaction was not subject to the approval of the Company’s stockholders.

Pursuant to the Merger Agreement, at the Special Meeting the Company’s stockholders approved, among other matters: (i) the conversion of Series Z Preferred Stock into shares of common stock in accordance with Nasdaq Listing Rule 5635(a) (the “Conversion Proposal”) and (ii) a proposal to amend our Restated Certificate of Incorporation to effect a reverse stock split of all of the Company’s issued and outstanding shares of common stock (the “Reverse Stock Split Proposal” and, together with the Conversion Proposal, the “Merger Agreement Meeting Proposals”).

The Company’s transaction costs of $4.6 million were expensed as incurred and included in the “Acquisition-related costs” financial statement line item in the Company’s consolidated statement of operations.

The transaction was accounted for under the acquisition method of accounting. Under the acquisition method, the total purchase price of the acquisition is allocated to the net tangible and identifiable intangible assets acquired and liabilities assumed based on the fair values as of the date of the acquisition. Consideration paid is comprised of the estimated fair value of various securities issued including the Series Z Preferred Stock, Series X Preferred Stock, stock options, restricted stock and warrants issued to Legacy Aceragen shareholders. In the fourth quarter of fiscal 2022, the preliminary purchase price allocation was updated, including the related determination of fair value of these securities issued as consideration, the allocation of consideration to the specific in-process research

and development programs acquired and the related income tax implications for the updates to the purchase price allocation. The fair value of the consideration totaled approximately $65.6 million, summarized as follows:

| (In thousands) | |
|---|---|
| Common stock issued to Aceragen stockholders | $ 2,809 |
| Series Z issued to Aceragen stockholders (Note 9) | 25,085 |
| Series X liability in connection with Aceragen Acquisition (Note 8) | 31,900 |
| Stock options, restricted stock and warrants allocated to consideration paid | 5,822 |
| Total Consideration paid | $ 65,616 |

The Company recorded the assets acquired and liabilities assumed as of the date of the Aceragen Acquisition based on the information available at that date. The following table presents the allocation of the purchase price to the estimated fair values of the assets acquired and liabilities assumed as of the Aceragen Acquisition date:

| (In thousands) | |
|---|---|
| Assets acquired: | |
| Cash and cash equivalents | $ 5,482 |
| Receivables | 1,914 |
| Prepaid expenses and other assets | 575 |
| In-process research and development assets | 71,600 |
| Goodwill | 11,100 |
| | $ 90,671 |
| Liabilities assumed: | |
| Accounts Payable and accrued expenses | $ 7,886 |
| Acquisition Obligation (Note 7) | 7,546 |
| Operating lease liabilities | 22 |
| Deferred tax liabilities | 9,601 |
| | $ 25,055 |
| Net assets acquired | $ 65,616 |

The fair value of IPR&D was capitalized as of the Aceragen Acquisition date and accounted for as indefinite-lived intangible assets until completion or disposition of the assets or abandonment of the associated research and development efforts. Upon successful completion of the development efforts, the useful lives of the IPR&D assets will be determined based on the anticipated period of regulatory exclusivity and will be amortized within operating expenses. Until that time, the IPR&D assets will be subject to impairment testing and will not be amortized. The goodwill recorded related to the acquisition is the excess of the fair value of the consideration transferred by the acquirer over the fair value of the net identifiable assets acquired and liabilities assumed at the date of the Aceragen Acquisition. The goodwill recorded is not deductible for tax purposes.

The following summarizes the Company's intangible assets acquired in connection with the Aceragen Acquisition and their carrying value as of December 31, 2022.

| (In thousands) | Acquisition Date Fair Value | Impairment | Carrying Value as of December 31, 2022 |
|---|---|---|---|
| ACG-701 for Cystic Fibrosis | $ 50,700 | $ — | $ 50,700 |
| ACG-701 for Melioidosis | 14,900 | — | 14,900 |
| ACG-801 for Farber Disease | 6,000 | — | 6,000 |
| Total in-process research and development costs (IPR&D) | $ 71,600 | $ — | $ 71,600 |

Intangible asset fair values for the three IPR&D programs were determined using the Multi-Period Excess Earnings Method ("MPEEM") which is a form of the income approach. Under the MPEEM, the fair value of an intangible asset is equal to the present value of the asset's incremental after-tax cash flows (excess earnings) remaining after deducting the market rates of return on the estimated value of contributory assets (contributory charge) over its remaining useful life. To calculate fair value of acquired IPR&D programs under the MPEEM, the Company uses probability-weighted cash flows discounted at a rate considered appropriate given the significant inherent risks associated with drug development by development-stage companies. Cash flows were calculated based on estimated projections of revenues and expenses related to each program and then reduced by a contributory charge on requisite assets employed. Contributory assets included debt-free working capital, net fixed assets and assembled workforce. Rates of return on the contributory assets were based on rates used for comparable market participants. Cash flows were assumed to extend through the market exclusivity period estimated to be provided by orphan drug designation. The resultant cash flows were then discounted to present value using a weighted-average cost of equity capital for companies with profiles substantially similar to that of each acquired IPR&D program, which the Company believes represents the rate that market participants would use to value the assets. The Company compensated for the phase of development of each program by probability-adjusting its estimation of the expected future cash flows. The projected cash flows were based on significant assumptions, such as the time and resources needed to complete the development and approval of each IPR&D program, estimates of revenue and operating profit related to the program considering its stage of development, the life of the potential commercialized product and associated risks, including the inherent difficulties and uncertainties in drug development, such as obtaining marketing approval from the FDA and other regulatory agencies, and risks related to the viability of and potential alternative treatments in any future target markets.

*Unaudited Pro Forma Financial Information*

The following unaudited pro forma financial information reflects the consolidated results of operations of the Company as if the Aceragen Acquisition had taken place on January 1, 2021. The unaudited pro forma financial information is not necessarily indicative of the results of operations as they would have been had the transactions been effected on the assumed date.

| | December 31, | |
|---|---|---|
| **(In thousands)** | **2022** | **2021** |
| Net revenues | $ 18,196 | $ 1,005 |
| Net (loss) income | $ (35,379) | $ 80,502 |

Nonrecurring pro forma transaction costs directly attributable to the Aceragen Acquisition was $11.2 million for the year ended December 31, 2022. There were no such costs for the year ending December 31, 2021. The costs deducted included success fees of $4.0 million in the aggregate incurred with financial advisors in connection with the Aceragen Acquisition. Additionally, the Company incurred $0.8 million in retention costs as a result of stay bonuses to employees immediately following the closing of the Aceragen Acquisition. The Company also incurred $3.7 million in restructuring costs related to the reduction-in-workforce during 2022 (see Note 13). These costs are excluded from the pro forma financial information for the year ended December 31, 2022. In addition, the Company recognized the $6.3 million income tax benefit for the year ended December 31, 2021 as if the transaction was completed on January 1, 2021.

**Note 4. Fair Value Measurements**

***Assets and Liabilities Measured at Fair Value on a Recurring Basis***

The Company applies the guidance in ASC 820, *Fair Value Measurement*, to account for financial assets and liabilities measured on a recurring basis. Fair value is measured at the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. As such, fair value is a market-based measurement that is determined based on assumptions that market participants would use in pricing an asset or liability.

The Company uses a fair value hierarchy, which distinguishes between assumptions based on market data (observable inputs) and an entity's own assumptions (unobservable inputs). The guidance requires that fair value measurements be classified and disclosed in one of the following three categories:

- Level 1: Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;
- Level 2: Quoted prices in markets that are not active or inputs which are observable, either directly or indirectly, for substantially the full term of the asset or liability; and
- Level 3: Prices or valuation techniques that require inputs that are both significant to the fair value measurement and unobservable (i.e., supported by little or no market activity).

Determining which category an asset or liability falls within the hierarchy requires significant judgment. The Company evaluates its hierarchy disclosures each reporting period. There were no transfers between Level 1, 2, and 3 during the year ended December 31, 2022.

The table below presents the assets and liabilities measured and recorded in the consolidated financial statements at fair value on a recurring basis at December 31, 2022 and 2021 categorized by the level of inputs used in the valuation of each asset and liability.

| | December 31, 2022 | | | |
|---|---|---|---|---|
| **(In thousands)** | **Total** | **Level 1** | **Level 2** | **Level 3** |
| **Assets** | | | | |
| Cash | $ 3,342 | $ 3,342 | $ — | $ — |
| Cash equivalents – money market funds | 8,702 | 8,702 | — | — |
| Total assets | $ 12,044 | $ 12,044 | $ — | $ — |
| **Liabilities** | | | | |
| Warrant liability | $ 2,819 | $ — | $ — | $ 2,819 |
| Series X Preferred Stock liability | 34,300 | — | — | 34,300 |
| Total liabilities | $ 37,119 | $ — | $ — | $ 37,119 |

| | December 31, 2021 | | | |
|---|---|---|---|---|
| **(In thousands)** | **Total** | **Level 1** | **Level 2** | **Level 3** |
| **Assets** | | | | |
| Cash | $ 250 | $ 250 | $ — | $ — |
| Cash equivalents – money market funds | 32,295 | 32,295 | — | — |
| Total assets | $ 32,545 | $ 32,545 | $ — | $ — |

The Level 1 assets consist of cash and money market funds, which are actively traded daily. The Level 3 liabilities include the Company’s warrant liability and Series X Preferred Stock liability.

***Changes in Level 3 Liabilities Measured at Fair Value on a Recurring Basis***

*Warrant Liability and Series X Preferred Stock Liability*

The reconciliation of the Company's warrant and Series X Preferred Stock liability measured at fair value on a recurring basis using unobservable inputs (Level 3) is as follows:

| (In thousands) | Warrant Liability | Series X Preferred Stock Liability |
|---|---|---|
| **Balance, December 31, 2021** | $ — | $ — |
| Issuance in connection with the Aceragen Acquisition | 3,180 | 31,900 |
| Change in fair value | (361) | 2,400 |
| **Balance, December 31, 2022** | $ 2,819 | $ 34,300 |

***Assumptions Used in Determining Fair Value of Liability-Classified Warrants***

The Company utilizes an option pricing model to value its liability-classified warrants. Inherent in the valuation model are assumptions related to volatility, risk-free interest rate, expected term, and dividend rate.

The fair value of the warrants has been estimated with the following weighted-average assumptions:

| | December 31, 2022 |
|---|---|
| Risk-free interest rate | 4.09% |
| Expected dividend yield | — |
| Expected term (years) | 4.1 |
| Expected volatility | 104% |
| Stock price (common stock) | $ 5.95 |
| Discount rate applied to preferred shares | 15% |
| Exercise price (per share) | $ 7.82 |

***Assumptions Used in Determining Fair Value of Liability-Classified Series X Preferred Stock***

The fair value of the Series X Preferred Stock represents the present value of estimated future payments that include royalty payments, as well as potential payments contingent upon the Company being awarded a priority review voucher (“PRV”). The Company utilized an income approach and Monte Carlo simulation method to determine the estimated fair value of the Series X Preferred Stock. The inputs used in the valuation approach are based on many factors such as estimated sales proceeds related to the PRV, the relevant market size, the estimated probability of regulatory success rates, anticipated patent protection, expected pricing, expected treated population, sales by region, estimated royalty payments and discount rate.

**Note 5. Property and Equipment**

At December 31, 2022 and 2021, net property and equipment at cost consisted of the following:

| ($ in thousands) | December 31, 2022 | December 31, 2021 |
|---|---|---|
| Leasehold improvements | $ 107 | $ 107 |
| Equipment and other | 712 | 712 |
| Total property and equipment, at cost | $ 819 | $ 819 |
| Less: Accumulated depreciation and amortization | 812 | 797 |
| Property and equipment, net | $ 7 | $ 22 |

Depreciation and amortization expense on property and equipment was less than $0.1 million for each of the years ended December 31, 2022 and 2021.

**Note 6. Accrued Expenses**

At December 31, 2022 and 2021, accrued expenses consisted of the following:

| ($ in thousands) | December 31, 2022 | December 31, 2021 |
|---|---|---|
| Payroll and related costs | $ 1,886 | $ 477 |
| Clinical and nonclinical trial expenses | 2,106 | 2,909 |
| Professional and consulting fees | 1,637 | 591 |
| Restructuring and other costs (Note 13) | 2,327 | — |
| Acquisition-related costs | 1,666 | — |
| Other | 289 | 111 |
| Total accrued expenses | $ 9,911 | $ 4,088 |

**Note 7. Acquisition Obligation**

As a result of the Aceragen Acquisition, the Company assumed an obligation pursuant to the Arrevus Merger Agreement (as defined below), whereby Legacy Aceragen was obligated to make an aggregate future payment of $7.5 million to the Former Stockholders (as defined below), $6.0 million and $1.5 million of which was originally due in October 2022 and January 2023, respectively (the “Acquisition Obligation”). The estimated fair value of the Acquisition Obligation on the Effective Date was $7.5 million. During the fourth quarter of 2022, $1.5 million of the $7.5 million obligation was paid.

In connection with the closing of the Aceragen Acquisition, Legacy Aceragen entered into a binding term sheet (the “Term Sheet”) with the representative of certain former stockholders of Arrevus (the “Former Stockholders”), pursuant to which Legacy Aceragen and the Former Stockholders agreed to defer certain payments owed by Legacy Aceragen to the Former Stockholders under that certain Agreement and Plan of Merger, dated October 18, 2021, by and among Legacy Aceragen, Arrevus, and their respective affiliates (the “Arrevus Merger Agreement”), in an aggregate amount of $6.0 million (the “Deferred Payments”) until October 24, 2023. The Deferred Payments bear interest at 12% per annum, paid quarterly beginning on April 1, 2023. The Company may prepay the Deferred Payments at any time, subject to payment in full in cash of the Deferred Payments, plus accrued interest up until the date of such prepayment. Any prepayment of the Deferred Payments must be made on a pro-rata basis among the holders of the Convertible Notes (as defined below) in proportion to their respective shares of the Deferred Payments; provided that prior to any such prepayment, the holder of each Convertible Note shall be given written notice thereof and the option to convert the principal balance into shares of common stock pursuant to the terms of the Convertible Note.

The Term Sheet provided that the Deferred Payments will be memorialized in 12% convertible unsecured promissory notes to be issued by the Company, pursuant to which each Former Stockholder will have the right to convert such Former Stockholder’s portion of its right to receive the Deferred Payments into shares of common stock (the “Convertible Notes”), The Term Sheet further provides that the Convertible Notes will provide the Former

Stockholders with customary registration rights covering the Common Stock issued following any conversion of the Convertible Notes. See Note 19 for discussion of Convertible Notes issued in January 2023.

During the period the Term Sheet was in effect, the Company imputed interest expense using the effective interest method based on the difference between the estimated fair value and the notional value. Interest expense for the year ended December 31, 2022 was immaterial.

**Note 8. Series X Preferred Stock Liability**

In connection with the Aceragen Acquisition, the Company issued five shares of Series X Preferred Stock. The shares of Series X Preferred Stock are non-convertible and non-voting and are entitled to discrete development and commercial milestone payments as well as royalty payments on net product sales of ACG-801 for Farber disease. The royalty rates range between low single digits to low double digits and expire, unless terminated earlier, upon the later of the expiration of the last valid claim in the licensed patent rights in such country covering such product and the expiration of data exclusivity in such country for such product. In addition, the payments due to the holders of the Series X shares are secured by substantially all of the assets related to ACG-801.

The Company concluded that the shares of Series X Preferred Stock do not represent a residual interest in the Company and are accounted for as debt. The liabilities associated with the shares of Series X Preferred Stock require the Company to make certain estimates and assumptions, particularly about the achievement of future development and regulatory milestones and future product sales. Such estimates and assumptions are utilized in determining the expected repayment term, accretion of interest expense and classification between current and long-term portions of amounts outstanding. The Company elected to carry the Series X Preferred Stock liability at fair value, and the debt instrument is outside the scope of ASC 480, *Distinguishing Liabilities from Equity*, and thus will be classified as a liability under ASC 470, *Debt*, in the Company's consolidated financial statements. Any changes in the fair value of the liability are recognized in the consolidated statement of operations until it is settled.

**Note 9. Redeemable Convertible Preferred Stock**

***Series B1 Preferred Stock***

On December 23, 2019, the Company entered into a Securities Purchase Agreement (the "December 2019 Securities Purchase Agreement") with institutional investors affiliated with Baker Brothers Advisors, LP (the "Purchasers"). Pursuant to the December 2019 Securities Purchase Agreement, the Company sold 23,684 shares of Series B1 convertible preferred stock ("Series B1 Preferred Stock") and warrants to purchase 139,318 shares of the Company's common stock at an exercise price of $25.84 per share (or, if the holder elected to exercise the warrants for shares of Series B1 Preferred Stock, 23,684 shares of Series B1 Preferred Stock at an exercise price of $2,584 per share) for aggregate gross proceeds of $3.9 million.

Due to the redeemable nature of the Series B1 Preferred Stock, the Series B1 Preferred Stock was classified as temporary equity and the carrying value was being accreted to its redemption value as of December 31, 2020 and while the Series B1 Preferred Stock was outstanding during 2021. During 2021, all the Company's 23,684 shares of Series B1 Preferred Stock outstanding were converted into shares of the Company's common stock.

The Series B1 warrants were classified as liabilities until their termination in March 2021 as the underlying shares were potentially redeemable and such redemption was deemed to be outside of the Company's control.

***Series B2, B3 and B4 Preferred Stock (Future Tranche Rights)***

Pursuant to the December 2019 Securities Purchase Agreement, the Company agreed to sell to the Purchasers, at their option and subject to certain conditions, (i) 98,685 shares of the Company's Series B2 convertible preferred stock ("Series B2 Preferred Stock") and 580,500 warrants to purchase common stock at an exercise price of $25.84 per share (or, at the election of the holder, 98,685 shares of Series B2 Preferred Stock at an price of $2,584.00 per share), for aggregate gross proceeds of $15 million (the "Series B2 Tranche"), (ii) 82,418 shares of Series B3 convertible preferred stock ("Series B3 Preferred Stock") and 387,849 warrants to purchase common stock at an exercise price of $30.94 per share (or, at the election of the holder, 65,934 shares of Series B3 Preferred Stock at a price of $3,094.00 per share), for aggregate gross proceeds of $15.0 million (the "Series B3

Tranche"), and (iii) 82,418 shares of Series B4 convertible preferred stock ("Series B4 Preferred Stock") and 387,849 warrants to purchase common stock at an exercise price of $30.94 per share (or, at the election of the holder, 65,934 shares of Series B3 Preferred Stock at a price of $3,094.00 per share), for aggregate gross proceeds of $15.0 million (the "Series B4 Tranche") (collectively, the "Future Tranche Rights") over a period of up to 21 months following the Company's 2020 Annual Meeting of Stockholders held on May 12, 2020. As consideration for the Future Tranche Rights, the Company received aggregate gross proceeds of $6.2 million in December 2019.

The purchase and sale of the securities issuable under the Series B2, B3, and B4 tranches described above were subject to three separate closings, each to be conducted at the purchasers' discretion. As a result of the Purchasers not exercising the Series B2 Tranche prior to expiration, all Future Tranche Rights and outstanding warrants previously issued pursuant to the December 2019 Securities Purchase Agreement were terminated during the year ended December 31, 2021. Accordingly, the Company is no longer eligible to receive additional proceeds pursuant to the December 2019 Securities Purchase Agreement.

The Future Tranche Rights were classified as liabilities until their termination in March 2021. Changes to the fair value of the future tranche right liability each reporting period, including the derecognition of the liability during the year ended December 31, 2021, is included in Future Tranche Right Liability Revaluation Gain in the Company's statements of operations.

***Series Z Redeemable Preferred Stock***

In connection with the Aceragen Acquisition, the Company issued 80,656 shares of Series Z Preferred Stock. The Series Z Preferred Stock did not have voting rights except for voting on specific corporate matters including (i) changes to the rights and preferences of the Series Z Preferred Stock, (ii) issuance of additional Series Z Preferred Stock, and (iii) enter into a fundamental transaction such as a sale of the Company. Certain provisions of the Series Z Preferred Stock are as follows:

- Conversion: Upon obtaining stockholder approval at the Special Meeting, each share of Series Z automatically converted into 58.82 shares of common stock, subject to beneficial ownership limitations.
- Dividends: Series Z Preferred Stock was eligible to participate in any dividends with common stockholders on an as-converted basis
- Liquidation: In the event of the liquidation, dissolution, or winding up of the affairs of the Company (a "Liquidity Event"), prior to stockholder approval at the Special Meeting, the holders of Series Z Preferred Stock would have been entitled to receive a liquidation preference prior to any payment to the holders of common stock.
- Redemption: In the event the Company would have been unable to obtain an affirmative stockholder vote at the Special Meeting to permit conversion, each holder of Series Z Preferred Stock would have been entitled to elect, at the holder's option, to have the shares of Series Z Preferred Stock be redeemed by the Company and equal to the estimated fair value of the Series Z Preferred Stock share at the time of redemption. Due to this redemption feature, as of December 31, 2022, the Series Z Preferred Stock was classified within temporary equity on the consolidated balance sheet.

The carrying value of the shares of Series Z is accreted to redemption value using the estimated fair value of the redemption value at each reporting period until the redeemable convertible preferred stock cease to be outstanding or the redemption right has expired. There was no accretion for the year ended December 31, 2022.

As more fully described in Note 19, "Subsequent Events", in January 2023, following shareholder approval of the Merger Agreement Proposals at the Special Meeting, all outstanding Series Z Preferred Stock converted into shares of common stock.

**Note 10. Stockholders' Equity (Deficit)**

***Preferred Stock***

The Restated Certificate of Incorporation, as amended, of the Company permits its Board of Directors to issue up to 5,000,000 shares of preferred stock, par value $0.01 per share, in one or more series, to designate the number of shares constituting such series, and fix by resolution, the powers, privileges, preferences and relative, optional or special rights thereof, including liquidation preferences and dividends, and conversion and redemption rights of each such series.

As of December 31, 2022, the Company has designated the following class of preferred stock:

- Series A: 1,500,000 authorized shares of Series A Convertible Preferred Stock
- Series B: 200,000 authorized shares of Series B Preferred Stock
- Series B1: 277,921 authorized shares of Series B1 Redeemable Convertible Preferred Stock
- Series B2: 98,685 authorized shares of Series B2 Redeemable Convertible Preferred Stock
- Series B3: 82,814 authorized shares of Series B3 Redeemable Convertible Preferred Stock
- Series B4: 82,814 authorized shares of Series B4 Redeemable Convertible Preferred Stock
- Series Z: 80,656 authorized shares of Series Z Redeemable Convertible Preferred Stock
- Series X: 5 authorized shares of Series X Preferred Stock

*Series A Convertible Preferred Stock*. The dividends on the Series A convertible preferred stock ("Series A Preferred Stock") are payable semi-annually in arrears at the rate of 1% per annum, at the election of the Company, either in cash or additional duly designated, fully paid and nonassessable shares of Series A Preferred Stock. In the event of liquidation, dissolution, or winding up of the Company, after payment of debts and other liabilities of the Company, the holders of the Series A Preferred Stock then outstanding will be entitled to a distribution of $1 per share out of any assets available to shareholders. The Series A Preferred Stock is non-voting. All remaining shares of Series A Preferred Stock rank, as to payment upon the occurrence of any liquidation event, senior to the Company's common stock. Shares of Series A Preferred Stock are convertible, in whole or in part, at the option of the holder into fully paid and nonassessable shares of common stock at $4,624.00 per share, subject to adjustment. As of December 31, 2022 and 2021, there were 655 shares of Series A Preferred Stock outstanding.

Series B Preferred Stock. On November 17, 2022, the Company's Board of Directors declared a dividend of one one-thousandth of a share of Series B Preferred Stock, par value $0.01 per share ("Series B Preferred Stock"), for each outstanding share of the Company's common stock to stockholders of record at 5:00 p.m. Eastern Time on November 28, 2022 (the "Record Date"). Each share of Series B Preferred Stock entitled the holder thereof to 1,000,000 votes per share, together with the outstanding shares of the Company's common stock as a single class, exclusively with respect to certain proposals at the Special Meeting. The holders of the Series B Preferred Stock were not entitled to receive dividends of any kind. All outstanding shares of Series B Preferred Stock were redeemed immediately prior to, or concurrently with, the approval of the Reverse Stock Split Proposal at the Special Meeting.

*Series B1, B2, B3 and B4 Convertible Preferred Stock.* No shares outstanding at December 31, 2022 and 2021.

Series Z Preferred Stock. In connection with the Aceragen Acquisition, the Company issued Series Z Preferred Stock. See Note 9 for details on rights and preferences of holders of the Series Z Preferred Stock.

Series X Preferred Stock. In connection with the Aceragen Acquisition, the Company issued five shares of Series X Preferred Stock. Holders of shares of Series X Preferred Stock are entitled to receive distributions on shares of Series X Preferred Stock as set forth in (a) that certain the Stock and Warrant Purchase Agreement, dated as of March 24, 2021, by and between Legacy Aceragen and NovaQuest, as amended by that Amendment, dated

October 25, 2021, and as such agreement may be amended from time to time (the “Purchase Agreement”), and (b) that certain Sales Distribution and PRV Agreement dated as of October 25, 2021 (the “PRV Agreement”).
Such distributions include tiered royalty payments on net sales of ACG-801 for Farber disease based on a mid-double-digit percentage which drops to mid-single digits after reaching a predetermined milestone cap, and a required 35% share of the proceeds from the possible sale of a priority review voucher (“PRV”), which may be awarded by the FDA upon regulatory approval in the U.S. for ACG-801.

***Common Stock***

*Common Stock Authorized*

As of December 31, 2022, the Company had 140,000,000 shares of common stock authorized, of which 7,861,082 shares of common stock were reserved for issuance upon the exercise of outstanding warrants and options to purchase common stock, outstanding restricted stock units, the conversion of Series A Preferred Stock, the conversion of Series Z Preferred Stock, and shares available for grant under the Company’s equity incentive and employee stock purchase plans, each more fully described in Note 14.

*Put Shares*

Pursuant to the terms of a unit purchase agreement dated as of May 5, 1998, the Company issued and sold a total of 8,821 shares of common stock (the “Put Shares”) at a price of $2,176.00 per share. Under the terms of the unit purchase agreement, the initial purchasers (the “Put Holders”) of the Put Shares have the right (the “Put Right”) to require the Company to repurchase the Put Shares. The Put Right may not be exercised by any Put Holder unless: (1) the Company liquidates, dissolves or winds up its affairs pursuant to applicable bankruptcy law, whether voluntarily or involuntarily; (2) all of the Company’s indebtedness and obligations, including without limitation the indebtedness under the Company’s then outstanding notes, has been paid in full; and (3) all rights of the holders of any series or class of capital stock ranking prior and senior to the common stock with respect to liquidation, including without limitation the Series A convertible preferred stock, have been satisfied in full. The Company may terminate the Put Right upon written notice to the Put Holders if the closing sales price of its common stock exceeds $4,352.00 per share for the twenty consecutive trading days prior to the date of notice of termination. Because the Put Right is not transferable, in the event that a Put Holder has transferred Put Shares since May 5, 1998, the Put Right with respect to those shares has terminated. As a consequence of the Put Right, in the event the Company is liquidated, holders of shares of common stock that do not have Put Rights with respect to such shares may receive smaller distributions per share upon the liquidation than if there were no Put Rights outstanding.

As of December 31, 2022, the Company had repurchased or received documentation of the transfer of 2,941 Put Shares and 263 of the Put Shares continued to be held in the name of Put Holders. The Company cannot determine at this time what portion of the Put Rights of the remaining 5,617 Put Shares have terminated.

***Equity Financings***

*Common Stock Purchase Agreement*

On March 4, 2019, the Company entered into a Purchase Agreement with Lincoln Park Capital Fund, LLC (“Lincoln Park”), which was amended on September 2, 2020 (as amended to date, the “LPC Purchase Agreement”), pursuant to which, upon the terms and subject to the conditions and limitations set forth therein, Lincoln Park committed to purchase an aggregate of $35.0 million of shares of Company common stock from time to time at the Company’s sole discretion over a 36-month period, which expired on March 4, 2022. As consideration for entering into the LPC Purchase Agreement, the Company issued 15,867 shares of Company common stock to Lincoln Park as a commitment fee (the “Commitment Shares”). The closing price of the Company’s common stock on March 4, 2019 was $48.28 and the Company did not receive any cash proceeds from the issuance of the Commitment Shares.

During the year ended December 31, 2021, the Company sold 47,059 shares pursuant to the LPC Purchase Agreement, resulting in net proceeds of $4.2 million. No shares were sold during the year ended December 31, 2022, prior to the March 4, 2022 expiration of the LPC Purchase Agreement.

*"At-The-Market" Equity Program*

In November 2018, the Company entered into an Equity Distribution Agreement (the "ATM Agreement") with JMP Securities LLC ("JMP"), pursuant to which the Company may issue and sell shares of its common stock having an aggregate offering price of up to $50.0 million (the "Shares") through JMP as its agent. Subject to the terms and conditions of the ATM Agreement, JMP will use its commercially reasonable efforts to sell the Shares from time to time, based upon the Company's instructions, by methods deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended, or if specified by the Company, by any other method permitted by law, including but not limited to in negotiated transactions. The Company has no obligation to sell any of the Shares, and the Company or JMP may at any time suspend sales under the ATM Agreement or terminate the ATM Agreement. JMP is entitled to a fixed commission of 3.0% of the gross proceeds from Shares sold.

During the year ended December 31, 2021, the Company sold 301,021 Shares pursuant to the ATM Agreement resulting in net proceeds, after deduction of commissions and other offering expenses, of $15.3 million. No Shares were sold during the year ended December 31, 2022. As of December 31, 2022, the Company may sell up to an additional $19.5 million of shares under the ATM Agreement.

***Common and Preferred Stock Warrants***

In connection with various financing transactions, the Company has issued warrants to purchase shares of the Company's common stock and preferred stock. The Company accounts for common stock and preferred stock warrants as equity instruments or liabilities, depending on the specific terms of the warrant agreement. See Note 2 for further details on accounting policies related to the Company's warrants.

In connection with the Aceragen Acquisition, the Company issued warrants to Legacy Aceragen warrant holders to purchase shares of its common stock and Series Z Preferred Stock. The Series Z Preferred Stock warrants are liability classified and remeasured at each reporting period.

The following table summarizes outstanding warrants to purchase shares of the Company's common stock and/or preferred stock as of December 31, 2022 and 2021:

| | Number of Warrants | | | |
|---|---|---|---|---|
| Description | December 31, 2022 | December 31, 2021 | Weighted-Average Exercise Price | Expiration Date |
| **Equity-classified warrants:** | | | | |
| May 2013 warrants | 908 | 908 | $ 1.36 | None |
| September 2013 warrants | 241 | 241 | $ 1.36 | None |
| February 2014 warrants | 128 | 128 | $ 1.36 | None |
| April 2020 Private Placement first closing warrants | 178,794 | 178,794 | $ 38.76 | Apr 2023 |
| April 2020 Private Placement second closing warrants | 80,801 | 80,801 | $ 46.07 | Dec 2023 |
| April 2020 Private Placement second closing warrants | — | 67,260 | $ 0.17 | None |
| July 2020 Private Placement first closing warrants | — | 22,925 | $ 0.17 | None |
| July 2020 Private Placement first closing warrants | 162,601 | 162,601 | $ 43.86 | Jul 2023 |
| Assumed Legacy Aceragen common stock warrants | 79,596 | — | $ 7.82 | Mar 2031 |
| | 503,069 | 513,658 | | |
| **Liability-classified warrants:** | | | | |
| Assumed Legacy Aceragen Series Z Warrants (1) | 14,215 | — | $ 460.00 | Mar 2031 |
| | 14,215 | — | | |
| **Total outstanding** | 517,284 | 513,658 | | |

The table below is a summary of the Company's warrant activity for the year ended December 31, 2022.

| | Number of Warrants | | | Weighted-Average Exercise Price (1) |
|---|---|---|---|---|
| | Common Warrants | Series Z Warrants | Total | |
| **Outstanding at December 31, 2021** | 513,658 | — | 513,658 | $ 21.76 |
| Issued (2) | 79,596 | 14,215 | 93,811 | 7.82 |
| Exercised | (90,185) | — | (90,185) | 0.17 |
| Expired | — | — | — | — |
| **Outstanding at December 31, 2022** | 503,069 | 14,215 | 517,284 | $ 17.58 |

(1) Weighted-average exercise price for Series Z Warrants is calculated based on the common stock equivalent shares and exercise price as all Series Z Warrants were automatically converted into warrants to purchase common stock on January 12, 2023. See Note 19.

(2) Represents warrants issued in connection with the Aceragen Acquisition. See Note 3.

**Note 11. Government Contracts Revenue**

Government contracts revenue for the years ended December 31, 2022 consists of revenue from contracts with customers (U.S. government agencies) accounted for in accordance with ASC Topic 606, as more fully described in Note 2.

As of December 31, 2022, the Company had three in-process contracts with various agencies of the U.S. government with a total aggregate contract value of $46.3 million, of which $16.0 million has been used as of December 31, 2022. Of the $30.3 million total contractual value remaining as of December 31, 2022, $30.0 million is related to a contract awarded by Defense Threat Reduction Agency (“DTRA”) to develop ACG-701 as a potential medical countermeasure against the pathogen that causes melioidosis, B. Pseudomallei (the “DTRA Award”). The DTRA Award was granted pursuant to an agreement with a consortium management firm (“CMF”) with a contractual term through December 2026. While the contractual arrangement is with a CMF, the Company has determined that DTRA is the customer in the arrangement and the contract contains a single performance obligation (ACG-801 development services) which meet the criteria to be recognized over time. Other government contracts are not currently material.

During the year ended December 31, 2022, the Company recognized government contract revenues of $4.9 million, of which $4.6 million related to the DTRA Award. No such revenues were recognized during the year ended December 31, 2021. As of December 31, 2022, there were no material amounts of remaining performance obligations that are required to be disclosed.

**Note 12. Clinical Funding, Collaboration and License Agreements**

**Clinical Funding Agreements**

*Cystic Fibrosis Foundation Award*

In December 2021, the Cystic Fibrosis Foundation (“CFF”) provided Legacy Aceragen a Therapeutic Development Award Agreement (the “CFF Award”) in the amount of $3.5 million, of which $1.0 million had been received as of December 31, 2022. The CFF Award is intended to support the Company’s clinical trial for cystic fibrosis pulmonary exacerbations. The CFF Awards will provide the Company with $2.5 million of additional funding to be paid in line with certain development program milestones anticipated to begin in 2023 and go through 2024.

*U.S. Government Clinical Funding Contracts*

The Company is party to contracts with various agencies of the U.S. government which provide funding for the development of certain product candidates as more fully discussed in Note 11.

**Collaboration Agreement with Scriptr**

In February 2021, the Company entered into a collaboration and option agreement with Scriptr Global, Inc. ("Scriptr"), pursuant to which (i) Scriptr and the Company will conduct a research collaboration utilizing Scriptr Platform Technology ("SPT") to identify, research and develop gene therapy candidates (each, a "Collaboration Candidate") for the treatment, palliation, diagnosis or prevention of (a) myotonic dystrophy type 1 ("DM1 Field") and (b) Friedreich's Ataxia ("FA Field") on a Research Program-by-Research Program basis, as applicable, and (ii) the Company was granted an exclusive option, in its sole discretion, to make effective the Scriptr License Agreement (as defined below) for a given Research Program, as defined below, to make use of Collaboration Candidates and related intellectual property (collectively, the "Scriptr Agreement").

Pursuant to the Scriptr Agreement, Scriptr will use commercially reasonable efforts to carry out research activities set forth in accordance with the applicable DM1 Field and FA Field research plans, including certain pre-clinical proof of concept studies, to identify research Collaboration Candidates utilizing SPT (each, a "Research Program"). Following the completion of activities under a given Research Program, Scriptr will prepare and submit to the Company a comprehensive data package (each, a "Data Package") that summarizes, on a Research Program-by-Research Program basis, any Collaboration Candidates researched under the Research Program, including any data and results. Upon receipt of a Data Package, the Company has, in its sole discretion, up to two-hundred seventy (270) calendar days to make effective the exclusive license agreement entered into by and between Scriptr and the Company, pursuant to which, among other things, Scriptr grants us exclusive rights and licenses with respect to the development, manufacture and commercialization of licensed candidates and products, subject to certain conditions and limitations (the "Scriptr License Agreement"), for a given Research Program (each licensed Research Program, a "Licensed Program"). The Scriptr License Agreement provides for customary development milestones on candidates developed under a Licensed Program and royalties on licensed products, if any.

In partial consideration of the rights granted by Scriptr to Idera under the Scriptr Agreement, the Company made a one-time, non-creditable and non-refundable payment to Scriptr during the first quarter of 2021. In order to fund the Research Programs, the Company will reimburse Scriptr for costs incurred by or on behalf of Scriptr in connection with the conduct of each Research Program during the research term in accordance with the applicable Research Program budget and payment schedule. The Company incurred research and development expenses under the Scriptr Agreement of $0.5 million and $2.1 million during the years ended December 31, 2022 and 2021, respectively.

**Option and License Agreement with Licensee**

In April 2019, the Company entered into an amended and restated option and license agreement with a privately-held biopharmaceutical company ("Licensee"), pursuant to which the Company granted Licensee (i) exclusive worldwide rights to develop and market IMO-8400 for the treatment, palliation, and diagnosis of all diseases, conditions, or indications in humans (the "IMO-8400 License"), (ii) an exclusive right and license to develop IMO-9200 in accordance with certain IMO-9200 pre-option exercise protocols (the "IMO-9200 Option Period License"), and (iii) an exclusive one-year option, exercisable at Licensee's discretion, to obtain the exclusive worldwide rights to develop and market IMO-9200 for the treatment, palliation and diagnosis of all diseases, conditions, or indications in humans (the "IMO-9200 Option") (collectively, the "Licensee Agreement").

Under the terms of the Licensee Agreement, the Company received upfront, non-refundable fees totaling approximately $1.4 million and ownership of 10% of Licensee's outstanding common stock, subject to future adjustment, for granting Licensee the IMO-8400 License, the IMO-9200 Option Period License and transfer of related drug materials in 2019. In 2020, the IMO-9200 Option expired and, in 2022, the Licensee Agreement was terminated in its entirety. Accordingly, the Company is no longer eligible to receive any development and sales-based milestone payments and royalties pursuant to the Licensee Agreement.

As disclosed above, in connection with the Licensee Agreement, the Company acquired 10% of Licensee's outstanding common stock, subject to future adjustment. The Company accounted for the investment in accordance with ASC 321, *Investments-Equity Securities*. In connection with the termination of the Licensee Agreement, the Company determined that the value of the investment in License was *de minimis* and wrote off the carrying value at the date of termination, of less than $0.1 million.

**Note 13. Restructuring and Other Costs**

On September 28, 2022, in connection with the Aceragen Acquisition, the Company determined to restructure its operations and reduce its workforce which resulted in seven positions being eliminated, representing approximately 54% of the Company's pre-Aceragen Acquisition employees, of which five were eliminated on or before September 28, 2022. All seven of the positions were eliminated by December 31, 2022.

In April 2021, in order to align the Company's workforce with its needs in light of clinical trial outcomes and related shift in focus to business development activities aimed on identifying new portfolio opportunities, the Company determined to restructure its operations and reduce its workforce which resulted in 16 positions being eliminated, representing approximately 50% of the Company's pre-restructuring employees.

As a result of the above restructuring initiatives, the Company incurred restructuring-related charges of $3.7 million and $1.3 million for the years ended December 31, 2022 and 2021, respectively. Restructuring-related charges for both periods which were comprised of one-time termination costs in connection with the reduction-in-workforce, including severance, benefits, and related costs.

As of December 31, 2022, the short-term portion of the accrued restructuring balance, or $2.3 million, is included in "Accrued expenses" in the accompanying consolidated balance sheets. The long-term portion of less than $0.1 million is included within "Other liabilities" in the accompanying consolidated balance sheets.

**Note 14. Stock-based Compensation**

As of December 31, 2022, the only equity compensation plans from which the Company was permitted to issue new awards from was the Company's 2013 Stock Incentive Plan (as amended to date, the "2013 Plan") and 2017 Employee Stock Purchase Plan (the "2017 ESPP"), each as more fully described below. Subsequent to December 31, 2022, the Company's board of directors adopted the 2022 Equity Plan (as defined in Note 19), which was approved by the Company's stockholders at the Special Meeting on January 12, 2023.

***Equity Incentive and Employee Stock Purchase Plans***

*2013 Stock Incentive Plan*

The Company's board of directors adopted the 2013 Plan, which was approved by the Company's stockholders effective July 26, 2013. Amendments to the 2013 Plan were approved by the Company's stockholders in June 2014, June 2015, June 2017, June 2019, and June 2022. The 2013 Plan was intended to further align the interests of the Company and its stockholders with its employees, including its officers, non-employee directors, consultants, and advisers by providing equity-based incentives. The 2013 Plan allows for the issuance of incentive stock options intended to qualify under Section 422 of the Internal Revenue Code of 1986, as amended, non-statutory stock options, stock appreciation rights, restricted stock awards, restricted stock units ("RSUs"), other stock-based awards and performance awards. The total number of shares of common stock authorized for issuance under the 2013 Plan is 603,121 shares of the Company's common stock, plus such additional number of shares of common stock (up to 9,174 shares) as is equal to the number of shares of common stock subject to awards granted under the Company's 2008 Stock Incentive Plan (the "2008 Plan"), to the extent such awards expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right.

As of December 31, 2022, options to purchase a total of 284,017 shares of common stock and 48,910 unvested RSUs were outstanding, and up to 252,527 shares of common stock remained available for grant under the 2013 Plan. However, on the effective date of the 2022 Equity Plan (as defined in Note 19), all shares remaining available for grant under the 2013 Plan were rolled into the 2022 Equity Plan (as defined in Note 19).

*Legacy Aceragen 2021 Stock Incentive Plan*

In accordance with the Merger Agreement, the Company assumed and became the sponsor of the Legacy Aceragen's 2021 Stock Incentive Plan, as amended (the "Legacy Aceragen Plan"). Under the Merger Agreement,

each Legacy Aceragen option that was outstanding and unexercised immediately prior to the effective time of the Aceragen Acquisition was assumed and converted into and became an option to purchase (i) shares of the Company's common stock (the "Legacy Aceragen Common Options") and (ii) shares of the Company's Series Z Preferred Stock (the "Legacy Aceragen Preferred Options"), each on the same terms and conditions as applied to such options immediately prior to the Aceragen Acquisition as adjusted by the exchange ratio pursuant to the Merger Agreement. No additional awards were permitted to be issued from the Legacy Aceragen Plan as of the effective time of the Aceragen Acquisition.

Following stockholder approval of the Conversion Proposal, and pursuant to the terms of the Merger Agreement, in January 2023, each Legacy Aceragen Preferred Option became exercisable solely for shares of the Company's common stock. See Note 19.

*Other Awards and Inducement Grants*

The Company has not made any awards pursuant to other equity incentive plans, including the 2008 Plan, since the Company's stockholders approved the 2013 Plan. As of December 31, 2022, options to purchase a total of 4,908 shares of common stock were outstanding under the 2008 Plan. In addition, as of December 31, 2022, non-statutory stock options to purchase an aggregate of 19,116 shares of common stock were outstanding that were issued outside of the 2013 Plan to certain employees in 2015 and 2014 pursuant to the Nasdaq inducement grant exception as a material component of new hires' employment compensation.

2017 *Employee Stock Purchase Plan*

The Company's board of directors adopted the 2017 ESPP which was approved by the Company's stockholders and became effective June 7, 2017. Amendments to the 2017 ESPP were approved by the Company's stockholders in June 2019 and June 2022. The 2017 ESPP is intended to qualify as an "employee stock purchase plan" as defined in Section 423 of the Internal Revenue Code of 1986, as amended, and is intended to encourage our employees to become stockholders of ours, to stimulate increased interest in our affairs and success, to afford employees the opportunity to share in our earnings and growth and to promote systematic savings by them. The total number of shares of common stock authorized for issuance under the 2017 ESPP is 59,558 shares of common stock, subject to adjustment as described in the 2017 ESPP. Participation is limited to employees that would not own 5% or more of the total combined voting power or value of the stock of the Company after the grant. As of December 31, 2022, 39,048 shares remained available for issuance under the 2017 ESPP, however, future offering periods have been suspended until further notice.

*Stock Purchase Plan Administration*

The 2017 ESPP provides for offerings to employees to purchase common stock with offerings beginning on dates determined by the compensation committee of the board of directors or on the first business day thereafter. Each offering begins a "plan period" during which payroll deductions are to be made and held for the purchase of common stock at the end of the plan period. The compensation committee may, at its discretion, choose a plan period of 12 months or less for subsequent offerings and/or choose a different commencement date for offerings. During each plan period participating employees may elect to have a portion of their compensation, ranging from 1% to 10% of compensation as defined by the plan, withheld and used for the purchase of common stock at the end of each plan period. The purchase price is equal to 85% of the lower of the fair market value of a share of common stock on the first trading date of each plan period or the fair market value of a share of common stock on the last trading day of the plan period, and is limited by participant to $25,000 in fair value of common stock per year as well as other quarterly plan limitations as defined by each plan.

For the years ended December 31, 2022 and 2021, the Company issued 7,788 and 2,889 shares of common stock, respectively, under the 2017 ESPP and received proceeds of less than $0.1 million for each year, as a result of stock purchases.

***Accounting for Stock-based Compensation***

The Company recognizes non-cash compensation expense for stock-based awards under the Company's equity incentive plans and employee stock purchases under the Company's 2017 ESPP as follows:

- Stock Options: Compensation cost is recognized over an award's requisite service period, or vesting period, using the straight-line attribution method, based on the grant date fair value determined using the Black-Scholes option-pricing model.
- RSUs: Compensation cost for time-based RSUs, which vest over time based only on continued service, is recognized on a straight-line basis over the requisite service period based on the fair value of the Company's common stock on the date of grant. Compensation cost for awards that are subject to market considerations is recognized on a straight-line basis over the implied requisite service period, based on the grant date fair value estimated using a Monte Carlo simulation. Compensation cost for awards that are subject to performance conditions is recognized over the period of time commencing when the performance condition is deemed probable of achievement based on the fair value of the Company's common stock on the date of grant.
- Employee Stock Purchases: Compensation cost is recognized over each plan period based on the fair value of the look-back provision, calculated using the Black-Scholes option-pricing model, considering a 15% discount on shares purchased.

Total stock-based compensation expense attributable to stock-based payments made to employees and directors and employee stock purchases included in operating expenses in the Company's statements of operations for the years ended December 31, 2022 and 2021 was as follows:

| (in thousands) | Year Ended December 31, 2022 | 2021 |
|---|---|---|
| Stock-based compensation: | | |
| Research and development | | |
| Employee Stock Purchase Plan | $ 24 | $ 28 |
| Equity Incentive Plans | 258 | 546 |
| | $ 282 | $ 574 |
| General and administrative | | |
| Employee Stock Purchase Plan | $ 5 | $ 3 |
| Equity Incentive Plans | 1,901 | 1,960 |
| | $ 1,906 | $ 1,963 |
| Total stock-based compensation expense | $ 2,188 | $ 2,537 |

During the years ended December 31, 2022 and 2021, the weighted average fair market value of stock options granted was $5.88 and $26.18, respectively.

*Assumptions Used in Determining Fair Value of Stock Options*

Inherent in the Black-Scholes option-pricing model are the following assumptions:

- *Volatility*. The Company estimates stock price volatility based on the Company's historical stock price performance over a period of time that matches the expected term of the stock options.
- *Risk-free interest rate*. The risk-free interest rate is based on the U.S. Treasury yield curve in effect at the time of grant commensurate with the expected term assumption.
- *Expected term*. The expected term of stock options granted is based on an estimate of when options will be exercised or cancelled in the future.
- *Dividend rate.* The dividend rate is based on the historical rate, which the Company anticipates will remain at zero.

The fair value of each option award at the date of grant was estimated using the Black-Scholes option pricing model. All options granted during the years ended December 31, 2022 and 2021 were granted at exercise prices equal to the fair market value of the common stock on the dates of grant.

The following weighted average assumptions apply to the options to purchase 68,796 and 79,784 shares of common stock granted to employees and directors during the years ended December 31, 2022 and 2021, respectively:

| | 2022 | 2021 |
|---|---|---|
| Average risk-free interest rate | 2.6% | 0.4% |
| Expected dividend yield | — | — |
| Expected lives (years) | 3.8 | 3.6 |
| Expected volatility | 104% | 94% |
| Weighted average exercise price (per share) | $ 8.34 | $ 45.56 |

All options granted during the years ended December 31, 2022 were granted at exercise prices equal to the fair market value of the common stock on the dates of grant**.**

***Stock Option Activity***

The following table summarizes stock option activity for the year ended December 31, 2022.

| | Common Stock Options | | | |
|---|---|---|---|---|
| **($ in thousands, except per share data)** | **Number of Options** | **Weighted-Average Exercise Price** | **Weighted-Average Remaining Contractual Life (in years)** | **Aggregate Intrinsic Value** |
| **Outstanding at December 31, 2021** | 305,838 | $ 137.08 | 5.9 | $ — |
| Granted | 68,796 | 8.34 | | |
| Assumed in connection with Aceragen Acquisition | 111,038 | 6.22 | | |
| Exercised | — | — | | |
| Forfeited | (15,963) | 3.27 | | |
| Expired | (63,535) | 148.30 | | |
| **Outstanding at December 31, 2022 (1)** | 406,174 | $ 83.00 | 6.1 | $ 102 |
| **Exercisable at December 31, 2022** | 239,866 | $ 132.26 | 4.1 | $ 54 |

| | Preferred Stock Options | | | |
|---|---|---|---|---|
| **($ in thousands, except per share data)** | **Number of Options** | **Weighted-Average Exercise Price** | **Weighted-Average Remaining Contractual Life (in years)** | **Aggregate Intrinsic Value** |
| **Outstanding at December 31, 2021** | — | $ — | — | — |
| Assumed in connection with Aceragen Acquisition | 19,826 | 365.96 | | |
| Forfeited | (2,304) | 130.00 | | |
| **Outstanding at December 31, 2022 (1)** | 17,522 | $ 397.02 | 9.1 | $ 1,073 |
| **Exercisable at December 31, 2022** | 5,229 | $ 317.43 | 8.9 | $ 568 |

(1) Includes both vested stock options as well as unvested stock options for which the requisite service period has not been rendered but that are expected to vest based on achievement of a service condition.

In March 2021, the Company accelerated the vesting of 90,328 options, which were previously granted from 2019 to 2021. The modification resulted in an insignificant incremental stock-based compensation charge.

As of December 31, 2022, there was $3.9 million of unrecognized compensation cost related to unvested options, which the Company expects to recognize over a weighted average period of 2.7 years.

***Restricted Stock Unit Activity***

The following table summarizes restricted stock unit activity for the year ended December 31, 2022:

| | Time-based Awards | | Market/Performance-based Awards | |
|---|---|---|---|---|
| | Number of Shares | Weighted-Average Grant Date Fair Value | Number of Shares | Weighted-Average Grant Date Fair Value |
| **Nonvested shares at December 31, 2021** | 4,039 | $ 39.10 | 29,814 | $ 26.14 |
| Granted | 16,657 | 6.79 | — | — |
| Cancelled | — | — | — | — |
| Vested | (1,600) | 41.36 | — | — |
| **Nonvested shares at December 31, 2022** | 19,096 | $ 10.73 | 29,814 | $ 26.14 |

*Time-based Restricted Stock Units*

In March 2021, the Company accelerated the vesting of 8,110 unvested time-based RSUs which were previously granted in 2019 and 2020. The modification resulted in an insignificant incremental stock-based compensation charge on the modification date. During the years ended December 31, 2022 and 2021, the Company recognized $0.7 million and $0.3 million of compensation expense related to modified time-based RSUs that would have vested under the original terms of the award.

As of December 31, 2022, there was less than $0.1 million of unrecognized compensation cost related to the Company's time-based RSUs, which is expected to be recognized over a weighted average period of 0.8 years.

*Market/Performance-based Restricted Stock Units*

In July 2020, the Company granted RSUs to certain employees, including executive officers, under the 2013 Plan, with vesting that may occur upon a combination of specific performance and/or market conditions. Accordingly, the Company views these RSUs as two separate awards: (i) an award that vests if the market condition is achieved, and (ii) an award that vests whether or not the market condition is achieved, so long as the performance condition is achieved.

The Company recognized compensation expense for these awards over the estimated requisite service period of 2.36 years based on the estimated fair value when considering the market condition of the award, which was determined using a Monte Carlo simulation. During the year ended December 31, 2022, the Company recognized $0.3 million of compensation expense related to these awards. As of December 31, 2022, there was no remaining unrecognized compensation cost for the market-based component of these awards. However, should the performance condition be achieved, the Company would recognize an additional $0.3 million of compensation expense.

***Restricted Stock Activity***

The following table summarizes restricted stock activity for the year ended December 31, 2022:

| | Number of Shares | |
|---|---|---|
| | Common Stock | Series Z |
| **Nonvested shares at December 31, 2021** | — | — |
| Issued in connection with Aceragen Acquisition [(1)] | 16,763 | 2,993 |
| Cancelled | — | — |
| Vested | (1,331) | (237) |
| **Nonvested shares at December 31, 2022** | 15,432 | 2,756 |

(1) Issued in connection with Aceragen Acquisition to Legacy Aceragen shareholders as part of the merger consideration which include time-based vesting restrictions.

**Note 15. Commitments and Contingencies**

***Lease Commitments***

As of December 31, 2022, the Company's leased assets primarily consisted of its office headquarters in Exton, Pennsylvania. During each of the years ended December 31, 2022 and 2021, rent expense, including real estate taxes, totaled approximately $0.4 million. The leases are classified as operating leases.

Future minimum commitments as of December 31, 2022 under the Company's lease agreements are approximately:

| December 31, | Operating Leases *(in thousands)* |
|---|---|
| 2023 | $ 285 |
| 2024 | 240 |
| 2025 | 101 |
| Total lease payments | $ 626 |
| Less: imputed interest | (66) |
| Total present value of lease liabilities | $ 560 |

The Company entered into the Exton, Pennsylvania facility lease on April 1, 2015, which was subsequently amended on September 23, 2015 to include additional space. The Company currently leases approximately 11,000 square feet of office space at its Exton facility. The lease expires on May 31, 2025.

***Employee Benefit Plans***

Through December 31, 2022, the Company had an employee benefit plan under Section 401(k) of the Internal Revenue Code of 1986, as amended, which allowed eligible employees to make contributions up to a specified percentage of their compensation. Under the plan, the Company matched up to 5% of base salary, by matching 100% of the first 5% of base salary contributed by each employee.

Additionally, in connection with the Aceragen Acquisition, the Company assumed Legacy Aceragen's pooled employer benefit plan under Section 401(k) of the Internal Revenue Code of 1986, as amended ("Aceragen Pooled Plan"), which allows employees to make contributions up to a specified percentage of their compensation. Under the Aceragen Pooled Plan, the Company matches 100% of the first 3% of employee eligible compensation, as defined, contributed to the Aceragen Pooled Plan and 50% of the next 2% of the employee eligible compensation contributed to the Aceragen Pooled Plan. The Aceragen Pooled Plan was effective for Legacy Aceragen employees only through December 31, 2022. Effective January 1, 2023, the Company adopted the Aceragen Pooled Plan for all of its employees.

Total matching contributions for the years ended December 31, 2022 and 2021 was approximately $0.2 million and $0.3 million, respectively.

***Contingent Severance and Retention Payments***

In connection with the Aceragen Acquisition, the Company entered into transition and separation agreements with two former executives and retention agreements with three retained executives. These arrangements include certain compensation totaling $2.7 million in the aggregate to be paid to such executives in the form of stock and/or cash contingent on certain events occurring, including obtaining certain shareholder approvals at the Special Meeting for terminated executives and termination of employment within six months from the date of the Special Meeting for retained employees. As none of these contingencies were probable of occurring as of December 31, 2022, no expenses have been recognized in the consolidated statements of operations.

**Note 16. Income Taxes**

As of December 31, 2022 and 2021, the significant components of the Company's deferred tax assets and liabilities after applying the enacted corporate tax rates are approximately as follows:

| (in thousands) | 2022 | 2021 |
|---|---|---|
| Deferred tax assets: | | |
| Operating loss carryforwards | $ 94,356 | $ 90,550 |
| Tax credit carryforwards | 29,988 | 28,226 |
| Stock-based compensation | 4,959 | 6,902 |
| Capitalized research and development | 11,287 | 7,818 |
| Lease liabilities | 141 | 220 |
| Other | 459 | 70 |
| Total deferred tax assets | $ 141,190 | $ 133,786 |
| Deferred tax liabilities: | | |
| Right-of-use asset | $ (134) | $ (213) |
| In-process research and development intangible assets | (15,312) | — |
| Total deferred tax liabilities | $ (15,446) | $ (213) |
| Valuation allowance | $ (129,027) | $ (133,573) |
| Net deferred tax assets (liabilities) | $ (3,283) | $ — |

The Company has provided a full valuation allowance for its deferred tax asset as of December 31, 2021 due to the uncertainty surrounding the ability to realize these assets. At December 31, 2022, the Company evaluated the realizability of its deferred tax assets and determined that the valuation allowance should be decreased by approximately $6.3 million primarily for consideration of the acquired in-process research and development intangible assets. An income tax benefit for the year ended December 31, 2022 is reflected in the consolidated statement of operations.

The difference between the U.S. federal corporate tax rate and the Company's effective tax rate for the years ended December 31, 2022 and 2021 are as follows:

| | 2022 | 2021 |
|---|---|---|
| Expected federal income tax rate | (21.0)% | (21.0)% |
| State income taxes, net of federal benefit | 1.7 | 2.1 |
| Federal and state credits | (4.2) | 1.7 |
| Reduction of state income tax rate | 14.9 | — |
| Warrant and future tranche right revaluation gain | (0.3) | 26.9 |
| Series X revaluation loss | 1.7 | — |
| Stock-based compensation | 4.6 | — |
| Other | 1.7 | (0.4) |
| Change in valuation allowance | (20.4) | (9.3) |
| Effective tax rate | (21.3)% | 0.0 % |

The components of income tax benefit are as follows:

| (in thousands) | 2022 | 2021 |
|---|---|---|
| Current: | | |
| Federal | $ — | $ — |
| State | — | — |
| | $ — | $ — |
| Deferred: | | |
| Federal | $ (6,318) | $ — |
| State | — | — |
| | $ (6,318) | $ — |
| | | |
| Total Income Tax Benefit | $ (6,318) | $ — |

As of December 31, 2022, the Company had cumulative federal, various state, and Switzerland net operating loss carryforwards ("NOLs") of approximately $355.8 million, $362.7 million, and $0.9 million, respectively, available to reduce federal, state and foreign taxable income, respectively. As a result of the Tax Cuts and Jobs Act of 2017, federal net operating losses incurred for taxable years beginning after January 1, 2018 have an unlimited carryforward period, but can only be utilized to offset 80% of taxable income in future taxable periods. Of the $355.8 million of federal NOLs, $158.4 million have an unlimited carryforward and the remaining NOLs are subject to expiration through 2037. In addition, at December 31, 2022, the Company had cumulative federal and state tax credit carryforwards of $28.3 million and $1.9 million, respectively. The federal credits expire through 2042 and the state credits expire through 2033.

Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, prescribe limitations on the amount of NOLs and tax credit carryforwards that may be utilized in any one year. Under Internal Revenue Code Section 382, if a corporation undergoes an "ownership change," the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income may be limited. In December 2017, the Company completed a study which determined that ownership changes had occurred. The ownership changes have and will continue to subject the Company's pre-ownership change NOL carryforwards to an annual limitation, which will significantly restrict the Company's ability to use them to offset taxable income in periods following the ownership change. The federal and state net operating loss and tax credit carryforwards and related deferred tax assets shown in the table below have been adjusted to reflect the limitations that resulted from this study. As no study has been completed subsequent to 2017, additional ownership change limitations may result from ownership changes that have occurred, or may occur in the future. In conjunction with the Aceragen Acquisition, the Company acquired Legacy Aceragen's federal, various state, and Switzerland NOL's of $8.1 million, $19.1 million, and $0.8 million, respectively.

The Company applies ASC 740-10, *Accounting for Uncertainty in Income Taxes, an interpretation of ASC 740*. ASC 740-10 clarifies the accounting for uncertainty in income taxes recognized in financial statements and requires the impact of a tax position to be recognized in the financial statements if that position is more likely than not of being sustained by the taxing authority. The Company had no unrecognized tax benefits resulting from uncertain tax positions at December 31, 2022 and 2021.

The Company files income tax returns in the U.S., various states, and Switzerland and is subject to examination in each of these jurisdictions. The Company's tax years in the US are open under statute from inception to present. All open years may be examined to the extent that tax credits or net operating loss carry forwards are used in future periods. The Company does not expect any material increase or decrease in its income tax expense, in the next twelve months, related to examinations or changes in uncertain tax positions. The Company's policy is to record interest and penalties on uncertain tax positions as general and administrative expense.

**Note 17. Related Party Transactions**

***Pillar Investment Entities***

Youssef El Zein, a member of the Company's Board of Directors until his resignation in October 2017, is a director and controlling stockholder of Pillar Invest Corporation ("Pillar Invest"), which is the general partner of Pillar Pharmaceuticals I, L.P., Pillar Pharmaceuticals II, L.P., Pillar Pharmaceuticals III, L.P., Pillar Pharmaceuticals IV, L.P., Pillar Pharmaceuticals V, L.P., Pillar 6, Pillar 7 and Pillar Partners (collectively, the "Pillar Investment Entities"). As of December 31, 2022, the Pillar Investment Entities beneficially owned 985,204 shares of the Company's common stock.

During the year ended December 31, 2021, certain of the Pillar Investment Entities exercised warrants to purchase 185,787 shares of the Company's common stock at an exercise price of $0.17 per share for a total exercise price of less than $0.1 million. A total of 1,121 shares were used as cashless shares to cover the exercise costs.

During the year ended December 31, 2022, certain of the Pillar Investment Entities exercised warrants to purchase 90,186 shares of the Company's common stock at an exercise price of $0.17 per share for a total exercise price of less than $0.1 million.

As of December 31, 2022, the Pillar Investment Entities held (i) warrants to purchase up to 178,794 shares of the Company's common stock at an exercise price of $38.76 per share, (ii) warrants to purchase up to 162,601 shares of the Company's common stock at an exercise price of $43.86 per share, and (iii) warrants to purchase up to 80,801 shares of the Company's common stock at an exercise price of $46.07 per share.

**NovaQuest**

Ron Wooten, a member of the Company's Board of Directors, is a member of the investment committee of NQ POF V GP, Ltd. ("NovaQuest GP"), which is the general partner of NovaQuest Co-Investment Fund XV, L.P. ("NovaQuest").

In connection with the Aceragen Acquisition, NovaQuest was issued five shares of Series X Preferred Stock and is entitled to receive distributions on shares of Series Z Preferred Stock, as more fully described in Note 10. In addition, all outstanding warrants to purchase Legacy Aceragen common stock held by NovaQuest immediately prior to the Aceragen Acquisition were assumed by the Company and converted into warrants to purchase shares of the Company's common stock and Series Z Preferred Stock on terms substantially identical to those in effect prior to the Aceragen Acquisition, except for adjustments to the underlying number of shares and the exercise price based on the Merger Agreement exchange ratio.

As of December 31, 2022, NovaQuest held five shares of Series X Preferred Stock, warrants to purchase 79,032 shares of the Company's common stock, and warrants to purchase 14,115 shares of Series Z Preferred Stock.

**Agreement with Dr. Atul Chopra**

In March 2021, Legacy Aceragen entered into a consulting agreement with Dr. Atul Chopra, a founder and a member of Legacy Aceragen's board of directors, pursuant to which Dr. Chopra provides consulting and advisory services in exchange for (i) $16,667 per month and (ii) a right to purchase 1,000,000 fully vested shares of Legacy Aceragen's common stock at a price equivalent to par value $0.001 per share. Subsequent to the executed consulting agreement, Dr. Chopra purchased all 1,000,000 shares, which were converted into shares of the Company's common stock and Series Z Preferred Stock in connection with the Aceragen Acquisition based on the Merger Agreement exchange ratio. The term of the consulting agreement was to remain in effect for a period of one year and automatically renew for successive one-year terms until terminated. At the effective time of the Aceragen Acquisition, the consulting agreement was terminated. Since March 2021 (inception of consulting agreement) through the termination of the agreement, Dr. Chopra received $0.3 million in consulting fees pursuant to the agreement.

As of December 31, 2022, Dr. Chopra owned 127,718 shares of the Company's common stock and 22,810 shares of Series Z Preferred Stock, which in January automatically converted into 1,341,764 shares of the Company's common stock. Following the conversion of the Series Z Preferred Stock, Dr. Chopra owned 1,469,482 shares of the Company's common stock.

***Board Fees Paid in Stock***

Pursuant to the Company's director compensation program, in lieu of director board and committee fees of $0.1 million during each of the years ended December 31, 2022 and 2021, the Company issued 10,781 and 16,221 shares of common stock, respectively, to certain of its directors. Director board and committee fees are paid in arrears and the number of shares issued was calculated based on the market closing price of the Company's common stock on the issuance date.

**Note 18. Net Income (Loss) per Common Share Applicable to Common Stockholders**

Details in the computation of basic and diluted net income (loss) per common share were as follows:

| | Year Ended December 31, | |
|---|---|---|
| **($ in thousands except share and per share data)** | **2022** | **2021** |
| **Net (loss) income per share — Basic:** | | |
| Net (loss) income | $ (23,360) | $ 98,091 |
| Less: Undistributed earnings to preferred stockholders | — | (1,150) |
| Net (loss) income applicable to common stockholders - basic | $ (23,360) | $ 96,941 |
| | | |
| Numerator for basic net (loss) income applicable to common stockholders | $ (23,360) | $ 96,941 |
| Denominator for basic net (loss) income applicable to common stockholders | 3,255,648 | 2,894,287 |
| Net (loss) income applicable to common stockholders - basic | $ (7.18) | $ 33.49 |
| | | |
| **Net (loss) income per share — Diluted:** | | |
| Net (loss) income applicable to common holders - basic | $ (23,360) | $ 96,941 |
| Less: Warrant revaluation gain applicable to dilutive liability-classified warrants | — | (6,983) |
| Less: Future tranche right revaluation gain applicable to dilutive liability-classified future tranche rights | — | (118,803) |
| Numerator for diluted net (loss) income applicable to common stockholders | $ (23,360) | $ (28,845) |
| | | |
| Denominator for basic net (loss) income applicable to common stockholders | 3,255,648 | 2,894,287 |
| Plus: Incremental shares underlying “in the money” liability-classified warrants outstanding | — | 5,492 |
| Plus: Incremental shares underlying “in the money” liability-classified future tranche rights outstanding | — | 48,880 |
| Denominator for diluted net income (loss) applicable to common stockholders | 3,255,648 | 2,948,659 |
| Net (loss) income applicable to common stockholders - diluted | $ (7.18) | $ (9.78) |

Total antidilutive securities (or their common stock equivalent, where applicable) excluded from the calculation of diluted net loss per share for the years ended December 31, 2022 and 2021 were as follows:

| | Year Ended December 31, | |
|---|---|---|
| | **2022** | **2021** |
| Common stock options | 406,174 | 306,000 |
| Preferred stock options | 1,030,693 | — |
| Restricted stock units and restricted stock awards | 226,460 | 33,865 |
| Common stock warrants | 503,069 | 513,661 |
| Preferred stock warrants | 836,176 | — |
| Convertible preferred stock | 4,582,364 | 14 |
| Total | 7,584,936 | 853,540 |

**Note 19. Subsequent Events**

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. In some instances, such subsequent events may require retroactive adjustment to information reported at the balance sheet date.

***Conversion of Series Z Preferred Stock***

On January 12, 2023, at the Special Meeting, the Company's stockholders approved the issuance of shares of the Company's common stock upon conversion of the Series Z Preferred Stock in accordance with Nasdaq Listing Rule 5635(a). Following approval, effective January 17, 2023 at 5:00 p.m. Eastern Time, all 80,656 outstanding shares of Series Z Preferred Stock were automatically converted into 4,744,467 shares of the Company's common stock pursuant to the terms of the Series Z Preferred Stock. In addition, all Legacy Aceragen Preferred Options were automatically converted into Legacy Aceragen Common Options.

***Reverse Stock Split***

On January 12, 2023, at the Special Meeting, the Company's stockholders approved an amendment to the Company's Restated Certificate of Incorporation, as amended, to effect a reverse stock split of the Company's issued and outstanding common stock by a whole number ratio to be determined by the Company board of directors within a range of one-for-seventeen (1:17) and one-for-twenty-three (1:23) (or any number in between), to be effected in the sole discretion of the Company's board of directors at any time within one year of the date of the Special Meeting.

On January 17, 2023, the Company implemented a one-for-seventeen (1:17) reverse split of its issued and outstanding shares of common stock (the "Reverse Split"). The Reverse Split became effective on January 17, 2023 at 5:00 p.m., Eastern Time, and the Company's common stock began trading on the Nasdaq Capital Market on a Reverse Split-adjusted basis at the opening of trading on January 18, 2023. As of a result of the Reverse Split, every seventeen (17) shares of the Company's issued and outstanding common stock were combined into one share of its common stock, except to the extent that the Reverse Split resulted in any of the Company's stockholders owning a fractional share, which was settled in cash. The Reverse Split did not change the number of authorized shares or par value of the Company's common or preferred stock.

***2022 Equity Plan***

On January 12, 2023, at the Special Meeting, the Company's stockholders approved the Idera Pharmaceuticals, Inc. 2022 Stock Incentive Plan (the "2022 Equity Plan"). The 2022 Equity Plan provides for the issuance of incentive stock options, non-qualified stock options, stock awards, stock units, stock appreciation rights, and other stock-based awards. The 2022 Equity Plan was adopted principally to serve as a successor plan to the 2013 Plan and to increase the number of shares of the Company's common stock reserved for equity-based awards by an amount equal to the sum of: (i) 1,388,235 shares of Company common stock, plus (ii) 194,456 shares of Company common stock, which is the number of shares of Company common stock reserved for issuance under the 2013 Plan that remained available for grant under the 2013 Plan as of the effective date of the 2022 Equity Plan. In addition, shares of the Company's common stock underlying any outstanding award granted under the 2013 Plan that, following the 2022 Equity Plan effective date, expire, or are terminated, surrendered, or forfeited for any reason without issuance of such shares shall be available for new grants under the 2022 Equity Plan.

***January 2023 Convertible Notes***

Pursuant to the terms of the Term Sheet, on January 31, 2023, the Company issued 12% convertible unsecured promissory notes (the "Convertible Notes") to certain of the Former Stockholders in an aggregate amount of approximately $5.9 million. The Convertible Notes bear annual interest at 12%. Under the terms of the Convertible Notes, at the holder's election, any or all of the outstanding principal and accrued interest may be converted into shares of Company's common stock using a conversion price determined by the VWAP (as defined in the Convertible Notes) on the applicable trading market for the fifteen consecutive trading days ending prior to the date the holder provides notice of their intent to convert. The terms of the Convertible Notes provide the Former

Stockholders with customary registration rights covering the Common Stock issued following any conversion of the Convertible Notes.

***Silicon Valley Bank Closure***

Silicon Valley Bank ("SVB") was closed on March 10, 2023 by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation ("FDIC") as receiver. At the time of closing, the Company had approximately 56% of its cash and cash equivalent balances in segregated custodial accounts held by a third-party custodian for which SVB was the Company's agent and/or SVB Asset Management, an affiliate of SVB, is the advisor. The Company's investment portfolio currently does not contain any securities of SVB. On March 12, 2023, the U.S. Treasury, Federal Reserve, and FDIC announced that SVB depositors would have access to all of their money starting March 13, 2023 and the Company has received such access. The Company does not believe it will be impacted by the closure of SVB and will continue to monitor the situation as it evolves.

***Cost-reduction Plan Implementation***

On April 13, 2023, the Board approved certain cost-cutting measures with a view to preserving capital to support the Company's continuing operations. As part of this plan, the Company has commenced the furlough of 12 employees, representing approximately 46% of its workforce. Additionally, certain of the Company's employees and executive officers will defer portions of their respective base salaries in amounts that exceed $200,000, with such deferrals having a retroactive effective date of April 5, 2023. The Company will continue continue to review operations for other opportunities to reduce costs and pursue financing opportunities. For more information, please see Item 9B of this Form 10-K.

**THIS AMENDED AND RESTATED WARRANT AND THE UNDERLYING SECURITIES HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE "ACT"). THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED OR HYPOTHECATED IN THE ABSENCE OF AN EFFECTIVE REGISTRATION STATEMENT AS TO SUCH SECURITIES UNDER THE ACT OR AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY THAT SUCH REGISTRATION IS NOT REQUIRED.**

**ACERAGEN, INC.**

**AMENDED AND RESTATED**

**WARRANT TO PURCHASE COMMON STOCK**

**No. CS-1/A** **Original Issuance Date: March 24, 2021**

**Amended and Restated as of: March 30, 2023**

**Void After March 23, 2031 (the "Expiration Date")**

**THIS CERTIFIES THAT**, for value received, **NOVAQUEST CO-INVESTMENT FUND XV, L.P.**, having an address at 4208 Six Forks Road, Suite 920 Raleigh, NC 27609, or assigns (the **"Holder"**), is entitled to subscribe for and purchase at the Exercise Price (defined below) from **ACERAGEN, INC.** (f/k/a Idera Pharmaceuticals, Inc.), a Delaware corporation (the **"Company"**), having an address at 15 T.W. Alexander Drive, Suite 418, Research Triangle Park, NC 22709, shares of the common stock, par value $0.001 per share, of the Company (the **"Common Stock"**) on the terms and subject to the conditions set forth below.

**RECITALS**

Aceragen, LLC (f/k/a Aceragen, Inc.) ("**Old Aceragen**") has previously issued a warrant to Holder to purchase shares of Old Aceragen common stock ("**Old Aceragen Shares**") pursuant to the terms of the Stock and Warrant Purchase Agreement, dated March 24, 2021 by and among the Old Aceragen and the Holder (the "**Original Warrant**");

Pursuant to that certain Agreement and Plan of Merger, dated as of September 28, 2022 by and among the Company, Old Aceragen, Bell Merger Sub I, Inc., a Delaware corporation, and Bell Merger Sub II, LLC, a Delaware limited liability company (the "**Merger Agreement**"), the Original Warrant was assumed by the Company and converted into a warrant to purchase Common Stock, and all rights of the Holder with respect to the Old Aceragen Shares were converted into rights with respect to Common Stock, in each case pursuant to the applicable provisions of the Merger Agreement;

Following the closing of the transactions contemplated by the Merger Agreement, the Company's Board of Directors approved a one-for-seventeen (1:17) reverse split of the Company's issued and outstanding Common Stock (the "**Reverse Stock Split**"), which became effective as of 4:59 p.m. Eastern Time on January 17, 2023; and

The Company and Holder desire to amend and restate the Original Warrant in its entirety to reflect the assumption of the Original Warrant by the Company in connection with the Merger Agreement, and effectiveness of the Reverse Stock Split.

**AGREEMENT**

The Original Warrant is hereby superseded in its entirety by the terms hereof and is no longer in effect.

The number of shares of Common Stock that the Holder may purchase by exercising this warrant (the "**Warrant**") is 909,326 subject to adjustment pursuant to the terms herein, including but not limited to adjustment pursuant to Section 6 below.

**1. DEFINITIONS.** As used herein, the following terms shall have the following respective meanings:

**(a) "Exercise Period"** shall mean the period commencing with the date hereof and ending ten (10) years later, unless sooner terminated as provided below.

**(b) "Exercise Price"** shall mean $7.82 per share, subject to adjustment pursuant to Section 6 below.

**(c) "Exercise Shares"** shall mean the shares of the Common Stock issuable upon exercise of the Warrant, subject to adjustment pursuant to the terms herein, including but not limited to adjustment pursuant to Section 6 below.

**2. EXERCISE OF WARRANT.** The rights represented by the Warrant may be exercised in whole or in part at any time during the Exercise Period, by delivery of the following to the Company at its address set forth above (or at such other address as it may designate by notice in writing to the Holder):

**(a)** An executed Notice of Exercise in the form attached hereto;

**(b)** Payment of the Exercise Price in cash or by check; and

**(c)** The Warrant.

Upon the exercise of the rights represented by the Warrant, a certificate or certificates for the Exercise Shares so purchased, registered in the name of the Holder or persons affiliated with the Holder, if the Holder so designates, shall be issued and delivered to the Holder within a reasonable time after the rights represented by the Warrant shall have been so exercised.

The person in whose name any certificate or certificates for Exercise Shares are to be issued upon exercise of the Warrant shall be deemed to have become the holder of record of such shares on the date on which the Warrant was surrendered and payment of the Exercise Price was made, irrespective of the date of delivery of such certificate or certificates, except that, if the date of such surrender and payment is a date when the stock transfer books of the Company are closed, such

person shall be deemed to have become the holder of such shares at the close of business on the next succeeding date on which the stock transfer books are open.

**2.1 Net Exercise.** Notwithstanding any provisions herein to the contrary, if the fair market value of one share of the Common Stock is greater than the Exercise Price (at the date of calculation as set forth below), in lieu of exercising the Warrant by payment of cash, the Holder may elect to receive shares equal to the value (as determined below) of the Warrant (or the portion thereof being canceled) by surrender of the Warrant at the principal office of the Company together with the properly endorsed Notice of Exercise in which event the Company shall issue to the Holder a number of shares of Common Stock computed using the following formula:

$$X = \frac{Y\ (A-B)}{A}$$

Where X = the number of shares of Common Stock to be issued to the Holder

Y = the number of shares of Common Stock purchasable under the Warrant or, if only a portion of the Warrant is being exercised, the portion of the Warrant being canceled (at the date of such calculation)

A = the fair market value of one share of the Common Stock (at the date of such calculation)

B = Exercise Price (as adjusted to the date of such calculation)

For purposes of the above calculation, the fair market value of one share of Common Stock shall be determined by the Company's Board of Directors in good faith; *provided*, *however,* that in the event that the Warrant is exercised pursuant to this Section 2.1 in connection with the Company's initial public offering of its Common Stock, the fair market value per share shall be the per share offering price to the public of the Company's initial public offering.

**3. AUTOMATIC EXERCISE UPON EXPIRATION DATE**. If, on the Expiration Date, the fair market value, calculated as set forth in Section 2.1, of one share of Common Stock is greater than the Exercise Price, then this Warrant shall automatically be deemed on and as of such date to be exercised as to all shares of Common Stock for which it has not previously been exercised, in which event the Company shall issue to the Holder a number of shares of Common Stock computed using the formula set forth in Section 2.1. The Company shall deliver to Holder a certificate representing such shares within a reasonable period time thereafter.

**4. COVENANTS OF THE COMPANY**.

**4.1 Covenants as to Exercise Shares.** The Company covenants and agrees that all Exercise Shares that may be issued upon the exercise of the rights represented by the Warrant will, upon issuance, be validly issued and outstanding, fully paid and nonassessable, and free from all taxes, liens and charges with respect to the issuance thereof. The Company further covenants and agrees that the Company will at all times during the Exercise Period, have authorized and reserved, free from preemptive rights, a sufficient number of shares of its Common Stock to provide for the exercise of the rights represented by the Warrant. If at any time during the Exercise

Period the number of authorized but unissued shares of Common Stock shall not be sufficient to permit exercise of the Warrant, the Company will take such corporate action as may, in the opinion of its counsel, be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes.

**4.2 Notices of Record Date.** In the event of any taking by the Company of a record of the holders of any class of securities for the purpose of determining the holders thereof who are entitled to receive any dividend (other than a cash dividend which is the same as cash dividends paid in previous quarters) or other distribution, the Company shall mail to the Holder, at least ten (10) days prior to the date specified herein, a notice specifying the date on which any such record is to be taken for the purpose of such dividend or distribution.

**5. REPRESENTATIONS OF HOLDER.**

**5.1 Acquisition of Warrant for Personal Account.** The Holder represents and warrants that it is acquiring the Warrant and the Exercise Shares solely for its account for investment and not with a view to or for sale or distribution of said Warrant or Exercise Shares or any part thereof. The Holder also represents that the entire legal and beneficial interests of the Warrant and Exercise Shares the Holder is acquiring is being acquired for, and will be held for, its account only.

**5.2 Securities Are Not Registered.**

**(a)** The Holder understands that the Warrant and the Exercise Shares have not been registered under the Securities Act of 1933, as amended (the **"Act"**) on the basis that no distribution or public offering of the stock of the Company is to be effected. The Holder realizes that the basis for the exemption may not be present if, notwithstanding its representations, the Holder has a present intention of acquiring the securities for a fixed or determinable period in the future, selling (in connection with a distribution or otherwise), granting any participation in, or otherwise distributing the securities. The Holder has no such present intention.

**(b)** The Holder recognizes that the Warrant and the Exercise Shares must be held indefinitely unless they are subsequently registered under the Act or an exemption from such registration is available. The Holder recognizes that the Company has no obligation to register the Warrant or the Exercise Shares of the Company, or to comply with any exemption from such registration.

**(c)** The Holder is aware that neither the Warrant nor the Exercise Shares may be sold pursuant to Rule 144 adopted under the Act unless certain conditions are met, including, among other things, the existence of a public market for the shares, the availability of certain current public information about the Company, the resale following the required holding period under Rule 144 and the number of shares being sold during any three month period not exceeding specified limitations. Holder is aware that the conditions for resale set forth in Rule 144 have not been satisfied and that the Company presently has no plans to satisfy these conditions in the foreseeable future.

**5.3 Disposition of Warrant and Exercise Shares.**

**(a)** The Holder further agrees not to make any disposition of all or any part of the Warrant or Exercise Shares in any event unless and until:

(i) The Company shall have received a letter secured by the Holder from the Securities and Exchange Commission stating that no action will be recommended to the Commission with respect to the proposed disposition;

(ii) There is then in effect a registration statement under the Act covering such proposed disposition and such disposition is made in accordance with said registration statement; or

(iii) The Holder shall have notified the Company of the proposed disposition and shall have furnished the Company with a detailed statement of the circumstances surrounding the proposed disposition, and if reasonably requested by the Company, the Holder shall have furnished the Company with an opinion of counsel, reasonably satisfactory to the Company, for the Holder to the effect that such disposition will not require registration of such Warrant or Exercise Shares under the Act or any applicable state securities laws.

**(b)** The Holder understands and agrees that all certificates evidencing the shares to be issued to the Holder may bear the following legend:

> THESE SECURITIES HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE "ACT"). THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED OR HYPOTHECATED IN THE ABSENCE OF AN EFFECTIVE REGISTRATION STATEMENT AS TO THE SECURITIES UNDER THE ACT OR AN OPINION OF COUNSEL REASONABLY SATISFACTORY TO THE COMPANY THAT SUCH REGISTRATION IS NOT REQUIRED.

**5.4 Accredited Investor.** The Holder is an accredited investor as defined in Rule 501(a) of Regulation D promulgated under the Act.

**6. ADJUSTMENT OF EXERCISE PRICE.** In the event of changes in the outstanding Common Stock by reason of stock dividends, split-ups, recapitalizations, reclassifications, combinations or exchanges of shares, separations, reorganizations, liquidations, or the like, the number and class of shares available under the Warrant in the aggregate and the Exercise Price shall be correspondingly adjusted to give the Holder of the Warrant, on exercise for the same aggregate Exercise Price, the total number, class, and kind of shares as the Holder would have owned had the Warrant been exercised prior to the event and had the Holder continued to hold such shares until after the event requiring adjustment. The form of the Warrant need not be changed because of any adjustment in the number of Exercise Shares subject to the Warrant.

**7. FRACTIONAL SHARES.** No fractional shares shall be issued upon the exercise of the Warrant as a consequence of any adjustment pursuant hereto. All Exercise Shares (including fractions) issuable upon exercise of the Warrant may be aggregated for purposes of determining whether the exercise would result in the issuance of any fractional share. If, after aggregation, the exercise would result in the issuance of a fractional share, the Company shall, in lieu of issuance of any fractional share, pay the Holder otherwise entitled to such fraction a sum in cash equal to

the product resulting from multiplying the then current fair market value of an Exercise Share by such fraction.

**8. CORPORATE TRANSACTIONS.** In the event of, at any time during the Exercise Period, an initial public offering of securities of the Company registered under the Act, or any capital reorganization, or any reclassification of the capital stock of the Company (other than a change in par value or from par value to no par value or no par value to par value or as a result of a stock dividend or subdivision, split-up or combination of shares), or the consolidation or merger of the Company with or into another corporation (other than a merger solely to effect a reincorporation of the Company into another state), or the sale or other disposition of all or substantially all the properties and assets of the Company in its entirety to any other person, the Company shall provide to the Holder twenty (20) days advance written notice of such public offering, reorganization, reclassification, consolidation, merger or sale or other disposition of the Company's assets.

**9. No STOCKHOLDER RIGHTS.** The Warrant in and of itself shall not entitle the Holder to any voting rights or other rights as a stockholder of the Company.

**10. TRANSFER OF WARRANT.** Subject to applicable laws and the restriction on transfer set forth on the first page of the Warrant, the Warrant and all rights hereunder are transferable, by the Holder in person or by duly authorized attorney, upon delivery of the Warrant and the form of assignment attached hereto to any transferee designated by Holder; provided the transferee signs an investment letter in form and substance reasonably satisfactory to the Company.

**11. LOST, STOLEN, MUTILATED OR DESTROYED WARRANT.** If the Warrant is lost, stolen, mutilated or destroyed, the Company may, on such terms as to indemnity or otherwise as it may reasonably impose (which shall, in the case of a mutilated Warrant, include the surrender thereof), issue a new Warrant of like denomination and tenor as the Warrant so lost, stolen, mutilated or destroyed. Any such new Warrant shall constitute an original contractual obligation of the Company, whether or not the allegedly lost, stolen, mutilated or destroyed Warrant shall be at any time enforceable by anyone.

**12. NOTICES, ETC.** All notices required or permitted hereunder shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified, (b) when sent by confirmed facsimile if sent during normal business hours of the recipient, if not, then on the next business day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid, or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent to the Company and the Holder at their respective addresses set forth above or at such other address as the Company or Holder may designate by ten (10) days advance written notice to the other.

**13. GOVERNING LAW.** The Warrant and all rights, obligations and liabilities hereunder shall be governed by the laws of the State of Delaware.

**[SIGNATURE PAGE FOLLOWS]**

**IN WITNESS WHEREOF,** the Company and the Holder have caused the Warrant to be executed as of the date first written above.

**ACERAGEN, INC**.

By: /s/ John Taylor

Name: John Taylor

Title: Chief Executive Officer

**IN WITNESS WHEREOF**, the Company and the Holder have caused the Warrant to be executed as of the date first written above.

**HOLDER:**

**NovaQuest Co-Investment Fund XV, L.P.**

**By: NQ POF VGP, Ltd., its general partner**

By: /s/ John L. Bradley, Jr.
Name: John L. Bradley, Jr.
Title: Director

**NOTICE OF EXERCISE**

**TO: ACERAGEN, INC.**

**(1)** ❑ The undersigned hereby elects to purchase shares of the Common Stock of **ACERAGEN, INC.** (the **"Company"**) pursuant to the terms of the attached Warrant, and tenders herewith payment of the exercise price in full, together with all applicable transfer taxes, if any.

❑ The undersigned hereby elects to purchase shares of the Common Stock of **ACERAGEN, INC.** (the **"Company"**) pursuant to the terms of the net exercise provisions set forth in Section 2.1 of the attached Warrant, and shall tender payment of all applicable transfer taxes, if any.

**(2)** Please issue a certificate or certificates representing said shares of Common Stock in the name of the undersigned or in such other name as is specified below:

_____

(Name)

_____

(Address)

**(3)** The undersigned represents that (i) the aforesaid shares of Common Stock are being acquired for the account of the undersigned for investment and not with a view to, or for resale in connection with, the distribution thereof and that the undersigned has no present intention of distributing or reselling such shares; (ii) the undersigned is aware of the Company's business affairs and financial condition and has acquired sufficient information about the Company to reach an informed and knowledgeable decision regarding its investment in the Company; (iii) the undersigned is experienced in making investments of this type and has such knowledge and background in financial and business matters that the undersigned is capable of evaluating the merits and risks of this investment and protecting the undersigned's own interests; (iv) the undersigned understands that the shares of Common Stock issuable upon exercise of this Warrant have not been registered under the Securities Act of 1933, as amended (the **"Act"**), by reason of a specific exemption from the registration provisions of the Act, which exemption depends upon, among other things, the bona fide nature of the investment intent as expressed herein, and, because such securities have not been registered under the Act, they must be held indefinitely unless subsequently registered under the Act or an exemption from such registration is available; (v) the undersigned is aware that the aforesaid shares of Common Stock may not be sold pursuant to Rule 144 adopted under the Act unless certain conditions are met and until the undersigned has held the shares for the number of years prescribed by Rule 144, that among the conditions for use of the Rule is the availability of current information to the public about the Company and the Company has not made such information available and has no present plans to do so; and (vi) the undersigned agrees not to make any disposition of all or any part of the aforesaid shares of Common Stock unless and until there is then in effect a registration statement under the Act covering such proposed disposition and such disposition is made in accordance with said registration statement, or the

undersigned has provided the Company with an opinion of counsel satisfactory to the Company, stating that such registration is not required.

| | |
|---|---|
| (Date) | (Signature) |
| | (Print name) |

**ASSIGNMENT FORM**

(To assign the foregoing Warrant, execute this form and supply required information. Do not use this form to purchase shares.)

**FOR VALUE RECEIVED,** the foregoing Warrant and all rights evidenced thereby are hereby assigned to

Name: _____
(Please Print)

Address: _____
(Please Print)

Dated: _____ , 20 _____

Holder's Signature: _____

Holder's Address: _____

**NOTE:** The signature to this Assignment Form must correspond with the name as it appears on the face of the Warrant, without alteration or enlargement or any change whatever. Officers of corporations and those acting in a fiduciary or other representative capacity should file proper evidence of authority to assign the foregoing Warrant.

# DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

The following description sets forth certain material terms and provisions of Aceragen, Inc.'s ("Aceragen," "we," "us," and "our") securities that are registered under Section 12 of the Securities Exchange Act of 1934, as amended.

The following description is a summary and does not purport to be complete. It is subject to, and qualified in its entirety by reference to, Aceragen's Restated Certificate of Incorporation, as amended (the "Certificate of Incorporation") and Aceragen's Second Amended and Restated Bylaws (the "Bylaws"), each of which are incorporated by reference as an exhibit to the Annual Report on Form 10-K of which this Exhibit 4.23 is a part. The terms of these securities also may be affected by the Delaware General Corporation Law ("DGCL").

Unless otherwise indicated, any share and per share amounts included in the description of our securities, reflect, as applicable, the occurrence of a 1-for-8 reverse split of our common stock that occurred on June 29, 2006, a 1-for-8 reverse split of our common stock that occurred on July 27, 2018 and a 1-for-17 reverse split of our common stock that occurred on January 17, 2023.

## Authorized Capital Stock

We are authorized to issue a total of 145,000,000 shares of capital stock consisting of 140,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.01 per share. Our common stock is listed on the Nasdaq Capital Market under the trading symbol "ACGN."

## Description of Common Stock

*Voting*

Each outstanding share of common stock is entitled to one vote per share on all matters submitted to a vote of our stockholders, except as set forth in the Certificate of Incorporation, Bylaws or DGCL. Holders of common stock do not have cumulative voting rights.

*Dividends; Liquidation and Dissolution*

Subject to the preferences that may be applicable to any then outstanding shares of preferred stock, holders of common stock are entitled to receive ratably on a per share basis such dividends and other distributions in cash, stock or property of Aceragen as may be declared by our Board of Directors (the "Board") from time to time out of the legally available assets or funds of Aceragen. Upon our voluntary or involuntary liquidation, dissolution or winding up, holders of common stock are entitled to receive ratably all assets of Aceragen available for distribution to its stockholders after payment of any amounts due to creditors and any amounts due to the holders of our preferred stock.

*Other Rights and Restrictions*

Holders of our common stock have no preemptive rights and no right to convert their common stock into any other securities. There are no redemption or sinking fund provisions applicable to our common stock. The Certificate of Incorporation and Bylaws do not restrict the ability of holders of common stock to transfer their shares of common stock. Our Board may authorize the issuance of preferred stock with voting, conversion, dividend, liquidation and other rights that may adversely affect the rights of the holder of our common stock.

*Put Right*

Pursuant to the terms of that certain Unit Purchase Agreement, dated May 5, 1998 (the "UPA") we issued and sold a total of 8,821 shares of common stock (the "Put Shares") at a price of $2,176.00 per share. Under the UPA, the initial purchasers of the Put Shares (the "Put Holders") have the right to require us to repurchase the put shares (the "Put Right"). In order for the Put Right to be exercised by any Put Holder all of the following must occur: (1) we

liquidate, dissolve or wind up our affairs pursuant to applicable bankruptcy law, whether voluntarily or involuntarily; (2) all of our indebtedness and obligations, including without limitation the indebtedness under our outstanding notes, has been paid in full; and (3) all rights of the holders of any series or class of capital stick raking prior and senior to the common stock with respect to liquidation have been satisfied in full. We may terminate the Put Right upon written notice to the Put Holders if the closing sales price of our common stock exceeds $4,352.00 per share for the 20 consecutive trading days prior to the date of notice of termination. Because the Put Right is not transferable, in the event that a Put Holder has transferred Put Shares since May 5, 1998, the Put Right with respect to those Put Shares has terminated. As a consequence of the Put Right, in the event we are liquidated, holders of shares of common stock that do not have a Put Right with respect to such shares may receive smaller distributions per share upon our liquidation than if there was no Put Right outstanding. As of December 31, 2022, we had repurchased or received documentation of the transfer of 2,941 Put Shares and 263 of the Put Shares continued to be held in the name of the Put Holders. We cannot determine at this time what portion of the Put Rights of the remaining 5,617 Put Shares have terminated.

As of December 31, 2022, 3,669,117 shares of common stock were issued and outstanding (including 15,432 shares of restricted common stock) and 7,861,082 shares of common stock were reserved for issuance upon the exercise of outstanding warrants and options to purchase common stock, outstanding restricted stock units, the conversion of Series A Convertible Preferred Stock (“Series A”), the conversion of Series Z Redeemable Convertible Preferred Stock (“Series Z”), and shares available for grant under the Idera Pharmaceuticals, Inc. 2013 Stock Incentive Plan, the Idera Pharmaceuticals, Inc. 2022 Stock Incentive Plan, and the assumed Aceragen, Inc. 2021 Stock Incentive Plan, and shares available for purchase under the Idera Pharmaceuticals, Inc. 2017 Employee Stock Purchase Plan.

**Preferred Stock**

We are authorized to issue 5,000,000 shares of preferred stock, of which 1,500,000 shares have been designated Series A, 200,000 shares have been designated Series B Preferred Stock (“Series B”), 277,921 shares have been designated Series B1 Redeemable Convertible Preferred Stock (“Series B1”), 98,685 shares have been designated Series B2 Redeemable Convertible Preferred Stock (“Series B2”), 82,814 shares have been designated Series B3 Redeemable Convertible Preferred Stock (“Series B3”), 82,814 shares have been designated Series B4 Redeemable Convertible Preferred Stock (“Series B4”), 80,656 shares have been designated Series Z, and 5 shares have been designated Series X Preferred Stock (“Series X”).

Shares of Series A, in whole or in part, at the option of the holder, are convertible into fully paid and nonassessable shares of common stock at $4,624.00 per share, subject to adjustment. Shares of Series B are not convertible into, or exchangeable for, shares of any other class or series of stock or other securities of the Company. Each share of Series B1, Series B2, Series B3 and Series B4 is initially convertible into 5 shares of common stock. Shares of Series B1 and Series B2 are convertible, in whole or in part, at the option of the holder into fully paid and nonassessable shares of common stock at $25.84 per share, subject to adjustment. Shares of Series B3 and Series B4 are convertible, in whole or in part, at the option of the holder into fully paid and nonassessable shares of common stock at $30.94 per share, subject to adjustment.

As of December 31, 2022, there were 655 shares of Series A outstanding, 62,355 shares of Series B outstanding, 80,656 shares of Series Z outstanding (including 2,756 shares of restricted Series Z), 5 shares of Series X outstanding, and no shares of Series B1, Series B2, Series B3 and Series B4 outstanding. All outstanding shares of Series B were redeemed immediately prior to, or concurrently with, the approval of the 1-for-17 reverse split of our common stock that occurred on January 17, 2023. Following the approval of the issuance of shares of the Company’s common stock upon conversion of Series Z, all outstanding shares of Series Z were converted into shares of common stock on January 17, 2023.

**Common Stock Issuable Upon Exercise of Warrants**

In connection with various financing transactions, we have issued warrants to purchase shares of our common stock and preferred stock. As of December 31, 2022, there were 517,284 warrants outstanding, of which 503,069 were common stock warrants and 14,215 were Series Z warrants. Of the 503,069 common stock warrants outstanding, 501,792 warrants have expiration dates ranging between April 7, 2023 and March 23, 2031, and 1,277 warrants have an indefinite exercise period. Following the approval of the issuance of shares of the Company’s

common stock upon conversion of Series Z, all Series Z warrants were converted into common stock warrants on January 17, 2023 and are exercisable through March 23, 2031.

**Certain Anti-Takeover Provisions of Our Certificate Incorporation and Bylaws**

The following is a summary of certain provisions of our Certificate of Incorporation and Bylaws that may have the effect of delaying, deterring or preventing hostile takeovers or changes in control or the management of Aceragen. Such provisions could deprive our stockholders of opportunities to realize a premium on their stock. At the same time, these provisions may have the effect of inducing any persons seeking to acquire or control us to negotiate terms acceptable to our Board.

*Undesignated Preferred Stock*

Our Certificate of Incorporation authorizes our Board to issue shares of preferred stock and set the voting powers, designations, preferences, and other rights related to that preferred stock without stockholder approval. Any such designation and issuance of shares of preferred stock could delay, defer or prevent any attempt to acquire or control us.

*Staggered Board*

Our Certificate of Incorporation and Bylaws provide for the division of our Board into three classes as nearly equal in size as possible with staggered three-year terms. The classification of the Board could have the effect of making it more difficult for a third party to acquire, or of discouraging a third party from acquiring, control of us. Our Certificate of Incorporation and Bylaws require the affirmative vote of the holders of at least 75% of the shares of our capital stock issued and outstanding and entitled to vote to amend or repeal this provision.

*Vacancies on the Board of Directors; Removal of Directors*

Our Certificate of Incorporation and our Bylaws provide that, subject to any rights of holders of our preferred stock, any vacancies in our Board for any reason will be filled only by a majority of our directors remaining in office, and directors so elected will hold office until the next election of directors. The inability of our stockholders to fill vacancies on the Board may make it more difficult to change the composition of our Board. Additionally, our Certificate of Incorporation and Bylaws provide that a director may be removed from office by our stockholders only for cause and by the affirmative vote of at least two-thirds of our outstanding voting stock. Our Certificate of Incorporation and Bylaws require the affirmative vote of the holders of at least 75% of the shares of our capital stock issued and outstanding and entitled to vote to amend or repeal these provisions.

*Cumulative Voting*

Our Certificate of Incorporation and Bylaws do not provide for cumulative voting. Accordingly, the holders of a majority of the shares of common stock entitled to vote in any election of directors may elect all of the directors standing for election. As a result, subject to the voting rights, of which there currently are none, of any outstanding preferred stock, persons who hold more than 50% of the outstanding common stock entitled to elect members of our Board can elect all of the directors who are up for election in a particular year.

*Business Combinations*

We are subject to Section 203 of the DGCL. Subject to certain exceptions, Section 203 prevents a publicly-held Delaware corporation from engaging in a “business combination” with any “interested stockholder” for three years following the date that such person became an interested stockholder, unless either the interested stockholder attained such status with the approval of our Board, the business combination is approved by our Board and stockholders in a prescribed manner or the interested stockholder acquired at least 85% of our outstanding voting stock in the transaction in which such person became an interested stockholder. A “business combination” includes, among other things, a merger or consolidation involving us and the “interested stockholder” and the sale of more than 10% of our assets. In general, an “interested stockholder” is any entity or person beneficially owning 15% or more of

our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person.

*No Stockholder Action by Written Consent; Special Meeting of Stockholders*

Our Certificate of Incorporation and our Bylaws provide do not provide for action by written consent, which may require our stockholders to wait for a regularly scheduled annual meeting to change the composition of our Board. Our Certificate of Incorporation and our Bylaws also provide that special meetings of our stockholders may be called only by the Board or by our chief executive officer or, if the office the chief executive officer is vacant, our president. In no event may our stockholders call a special meeting of stockholders. Our Certificate of Incorporation and Bylaws require the affirmative vote of the holders of at least 75% of the shares of our capital stock issued and outstanding and entitled to vote to amend or repeal these provisions.

*Advance Notification of Stockholder Nominations and Proposals*

Our Bylaws provide that stockholders seeking to bring business before an annual meeting of stockholders, or to nominate candidates for election as directors at an annual meeting of stockholders, must meet specified procedural requirements. These provisions may preclude stockholders from bringing matters before an annual meeting of stockholders or from making nominations for directors at an annual or special meeting of stockholders.

**ACERAGEN, INC.**

**INCENTIVE STOCK OPTION GRANT AGREEMENT**
**GRANTED PURSUANT TO THE 2022 EQUITY INCENTIVE PLAN**

This INCENTIVE STOCK OPTION GRANT AGREEMENT (the "***Agreement***"), dated as of [●] (the "***Date of Grant***"), is delivered by Aceragen, Inc. (formerly known as Idera Pharmaceuticals, Inc.) (the "***Company***") to [●] (the "***Participant***").

RECITALS

The Idera Pharmaceuticals, Inc. 2022 Equity Incentive Plan (as may be amended from time to time, the "***Plan***") provides for the grant of stock options to purchase shares of Company common stock, par value $0.001 per share ("***Company Stock***"). The Committee has decided to make this incentive stock option grant as an inducement for the Participant to promote the best interests of the Company and its stockholders. This Agreement is made pursuant to the Plan and is subject in its entirety to all applicable provisions of the Plan. Capitalized terms used herein and not otherwise defined will have the meanings set forth in the Plan.

The Participant must accept the terms and conditions of this Agreement electronically through the electronic acceptance procedure established by the Company. In no event shall this option be exercised in the absence of such acceptance.

1. Grant of Option.

(a) Subject to the terms and conditions set forth in this Agreement and in the Plan, the Company hereby grants to the Participant an incentive stock option (the "***Option***") to purchase [●] shares of Company Stock (each a "***Share***", and together the "***Shares***") at an Exercise Price of $[●] per Share. The Option shall become exercisable according to Section 2 below.

(b) The Option is designated as an incentive stock option, as described in Section 5 below. However, if and to the extent the Option exceeds the limits for an incentive stock option, as described in Section 5, the Option shall be a nonqualified stock option.

2. Exercisability of Option.

(a) Subject to the terms of this Section 2, the Option shall become vested according to the following dates (each a "***Vesting Date***"), provided that the Participant continues to be employed by, or provide service to, the Employer from the Date of Grant until the applicable Vesting Date: twenty-five percent (25%) of the Shares subject to the Option shall vest on the first anniversary of the Date of Grant, and the Option shall become vested and exercisable with respect to the remaining Shares subject to the Option in equal monthly installments over the thirty-six (36)-month period following such anniversary. Notwithstanding the foregoing, (i) the vesting terms set forth herein shall be subject to any special vesting terms set forth in any employment agreement between the Participant and the Company; and (ii) vesting of the Option shall cease to occur upon the Participant's death or Disability.

(b) The vesting and exercisability of the Option is cumulative, but shall not exceed 100% of the Shares subject to the Option. If the terms set forth in Section 2(a) would produce fractional Shares, the number of Shares for which the Option becomes vested and exercisable shall be rounded down to the nearest

whole Share and the fractional Shares will be accumulated so that the resulting whole Shares will be included in the number of Shares for which the Option becomes vested and exercisable on the last Vesting Date.

(c) In the event of a Change of Control before the Option is fully vested and exercisable, the provisions of the Plan applicable to a Change of Control shall apply to the Option, and, in the event of a Change of Control, the Committee may take such actions with respect to the vesting and exercisability of the Option as it deems appropriate pursuant to the Plan.

3. Term of Option.

(a) The Option shall have a term of ten years from the Date of Grant and shall terminate at the expiration of that period, unless it is terminated at an earlier date pursuant to the provisions of this Agreement or the Plan. Notwithstanding the foregoing, in the event that on the last business day of the term of the Option, the exercise of the Option is prohibited by applicable law, including a prohibition on purchases or sales of Company Stock under the Company's insider trading policy, the term of the Option shall be extended for a period of 30 days following the end of the legal prohibition, unless the Committee determines otherwise.

(b) The Option shall automatically terminate upon the happening of the first of the following events:

(i) The expiration of the 90-day period after the Participant ceases to be employed by, or provide service to, the Employer, if the termination is for any reason other than Disability, death or Cause.

(ii) The expiration of the one-year period after the Participant ceases to be employed by, or provide service to, the Employer on account of the Participant's Disability.

(iii) The expiration of the one-year period after the Participant ceases to be employed by, or provide service to, the Employer, if the Participant dies while employed by, or providing service to, the Employer or the Participant dies within 90 days after the Participant ceases to be so employed or to provide services to the Employer for any reason other than Disability, death or Cause.

(iv) The date on which the Participant ceases to be employed by, or provide service to, the Employer for Cause. In addition, notwithstanding the prior provisions of this Section 3, if the Participant engages in conduct that constitutes Cause after the Participant's employment or service terminates, the Option shall immediately terminate, and the Participant shall automatically forfeit all Shares underlying any exercised portion of the Option for which the Company has not yet delivered the Share certificates, upon refund by the Company of the Exercise Price paid by the Participant for such Shares.

Notwithstanding the foregoing, in no event may the Option be exercised after the date that is immediately before the tenth anniversary of the Date of Grant, except as provided under Section 3(a) above. Any portion of the Option that is not exercisable at the time the Participant ceases to be employed by, or provide service to, the Employer shall immediately terminate.

4. Exercise Procedures.

(a) Subject to the provisions of Sections 2 and 3 above, the Participant may exercise part or all of the exercisable Option by giving the Company or its delegate either (i) written notice of intent to exercise or (ii) electronic notice of exercise via the Company's online equity management platform (e.g., UBS OneSource), specifying the number of shares of Company Stock as to which the Option is to be exercised and, in each case, such other information as the Company or its delegate may require.

(b) At such time as the Committee shall determine, the Participant shall pay the Exercise Price (i) in cash, (ii) unless the Committee determines otherwise, by delivering shares of Company Stock owned by the Participant, which shall be valued at their Fair Market Value on the date of exercise, or by attestation (in accordance with procedures prescribed by the Company) to ownership of shares of Company Stock having a Fair Market Value on the date of exercise at least equal to the Exercise Price, (iii) by payment through a broker in accordance with procedures permitted by Regulation T of the Federal Reserve Board, (iv) if permitted by the Committee, by withholding shares of Company Stock subject to the exercisable Option, which have a Fair Market Value on the date of exercise equal to the Exercise Price, or (v) by such other method as the Committee may approve, to the extent permitted by applicable law. The Committee may impose from time to time such limitations as it deems appropriate on the use of shares of Company Stock to exercise the Option.

(c) The obligation of the Company to deliver Shares upon exercise of the Option shall be subject to all applicable laws, rules, and regulations and such approvals by governmental agencies as may be deemed appropriate by the Committee, including such actions as Company counsel shall deem necessary or appropriate to comply with relevant securities laws and regulations.

(d) All obligations of the Company under this Agreement shall be subject to the rights of the Employer as set forth in the Plan to withhold amounts required to be withheld for any taxes, if applicable. The Participant shall be required to pay to the Employer, or make other arrangements satisfactory to the Employer to provide for the payment of, any federal, state, local or other taxes that the Employer is required to withhold with respect to the Option. At such time as the Committee may determine, the Participant may elect to satisfy any tax withholding obligation of the Employer with respect to the Option by having Shares withheld to satisfy the applicable withholding tax rate for FICA, federal, state, local and other tax liabilities.

(e) Upon exercise of the Option (or portion thereof), the Option (or portion thereof) will terminate and cease to be outstanding.

5. Designation as Incentive Stock Option.

(a) This Option is designated an incentive stock option under Section 422 of the Code. If the aggregate Fair Market Value of the stock on the date of the grant with respect to which incentive stock options are exercisable for the first time by the Participant during any calendar year, under the Plan or any other stock option plan of the Company or a parent or subsidiary, exceeds $100,000, then the Option, as to the excess, shall be treated as a nonqualified stock option that does not meet the requirements of Section 422. If and to the extent that the Option fails to qualify as an incentive stock option under the Code, the Option shall remain outstanding according to its terms as a nonqualified stock option.

(b) The Participant understands that favorable incentive stock option tax treatment is available only if the Option is exercised while the Participant is an employee of the Company or a parent or subsidiary of the Company or within a period of time specified in the Code after the Participant ceases to be an employee. The Participant understands that the Participant is responsible for the income tax consequences of the Option, and, among other tax consequences, the Participant understands that he or

she may be subject to the alternative minimum tax under the Code in the year in which the Option is exercised. The Participant will consult with his or her tax adviser regarding the tax consequences of the Option.

(c) The Participant agrees that the Participant shall immediately notify the Company in writing if the Participant sells or otherwise disposes of any Shares acquired upon the exercise of the Option and such sale or other disposition occurs on or before the later of (i) two years after the Date of Grant or (ii) one year after the exercise of the Option. The Participant also agrees to provide the Company with any information requested by the Company with respect to such sale or other disposition.

6. Restrictions on Exercise. Except as the Committee may otherwise permit pursuant to the Plan, only the Participant may exercise the Option during the Participant's lifetime and, after the Participant's death, the Option shall be exercisable (subject to the limitations specified in the Plan) solely by the legal representatives of the Participant, or by the Person who acquires the right to exercise the Option by will or by the laws of descent and distribution, to the extent that the Option is exercisable pursuant to this Agreement.

7. Grant Subject to Plan Provisions. This grant is made pursuant to the Plan, the terms of which are incorporated herein by reference, and in all respects shall be interpreted in accordance with the Plan. The grant and exercise of the Option are subject to the provisions of the Plan and to interpretations, regulations and determinations concerning the Plan established from time to time by the Committee in accordance with the provisions of the Plan, including, but not limited to, provisions pertaining to (a) rights and obligations with respect to withholding taxes, (b) the registration, qualification or listing of the Shares, (c) changes in capitalization of the Company, (d) other requirements of applicable law, and (e) governing law for the Plan and this Agreement. The Committee shall have the authority to interpret and construe the Option pursuant to the terms of the Plan, and its decisions shall be conclusive as to any questions arising hereunder.

8. No Employment or Other Rights. The grant of the Option shall not confer upon the Participant any right to be retained by or in the employ or service of the Employer and shall not interfere in any way with the right of the Employer to terminate the Participant's employment or service at any time. The right of the Employer to terminate at will the Participant's employment or service at any time for any reason is specifically reserved.

9. No Stockholder Rights. Neither the Participant, nor any Person entitled to exercise the Participant's rights in the event of the Participant's death, shall have any of the rights and privileges of a stockholder with respect to the Shares subject to the Option, until certificates for Shares have been issued upon the exercise of the Option.

10. Assignment and Transfers. Except as the Committee may otherwise permit pursuant to the Plan, the rights and interests of the Participant under this Agreement may not be sold, assigned, encumbered or otherwise transferred except, in the event of the death of the Participant, by will or by the laws of descent and distribution. In the event of any attempt by the Participant to alienate, assign, pledge, hypothecate, or otherwise dispose of the Option or any right hereunder, except as provided for in this Agreement, or in the event of the levy or any attachment, execution or similar process upon the rights or interests hereby conferred, the Company may terminate the Option by notice to the Participant, and the Option and all rights hereunder shall thereupon become null and void. The rights and protections of the Company hereunder shall extend to any successors or assigns of the Company and to the Company's parents, subsidiaries, and Affiliates. This Agreement may be assigned by the Company without the Participant's consent.

11. Applicable Law. The validity, construction, interpretation and effect of this Agreement shall be governed by and construed in accordance with the laws of the State of Delaware, without giving effect to the conflicts of laws provisions thereof.

12. Notice. Any notice to the Company provided for in this instrument shall be addressed to the Company in care of its General Counsel or Chief Financial Officer and any notice to the Participant shall be addressed to such Participant at the current address shown on the payroll of the Company. Any notice shall be delivered by hand or enclosed in a properly sealed envelope addressed as stated above, registered and deposited, postage prepaid, in a post office regularly maintained by the United States Postal Service or by the postal authority of the country in which the Participant resides or to an internationally recognized expedited mail courier.

13. Company Policies. The Participant acknowledges and agrees that the Option and the right to receive and retain any Shares purchased in connection therewith shall be subject to any applicable clawback or recoupment policies, share trading policies and other policies that may be approved or implemented by the Board or the Committee from time to time, whether or not approved before or after the Date of Grant. Accordingly, the Participant agrees that, subject to the requirements of applicable law, the Option, and the right to receive and retain any Shares, or the amount of any gain realized or payment received as a result of any sale or other disposition of the Shares, covered by this Agreement, shall be subject to rescission, cancellation or recoupment, in whole or part, if and to the extent so provided under any "clawback" or similar policy of the Company in effect on the Date of Grant or that may be established thereafter that is applicable to the Participant.

14. Entire Agreement. This Agreement contains the entire understanding between the Company and Participant with respect to the matter set forth herein, and shall supersede all prior and contemporaneous agreements and understandings, inducements or conditions, express or implied, oral or written.

15. Application of Section 409A of the Code. This Agreement is intended to be exempt from Section 409A of the Code and to the extent this Agreement is subject to Section 409A of the Code, it will in all respects be administered in accordance with Section 409A of the Code.

[***Signature Page Follows***]

IN WITNESS WHEREOF, the Company has caused an officer to execute this Agreement, and the Participant has executed this Agreement, effective as of the Date of Grant.

**ACERAGEN, INC.**

By: _____
Name:
Title:

I hereby accept the Option described in this Agreement, and I agree to be bound by the terms of the Plan and this Agreement. I hereby further agree that all decisions and determinations of the Committee shall be final and binding.

**Participant**

By: _____
Name:
Date:

***By clicking "I Agree" or "Accepted" or words to that effect on the online award acceptance tool made available to the Participant by the Company or its stock plan administrator, the Participant is acknowledging that they have read this Agreement, the Plan and all of the documents referred to herein and is agreeing to abide by all of their terms.***

**ACERAGEN, INC.**

**NONQUALIFIED STOCK OPTION GRANT AGREEMENT**
**GRANTED PURSUANT TO THE 2022 EQUITY INCENTIVE PLAN**

This NONQUALIFIED STOCK OPTION GRANT AGREEMENT (the "***Agreement***"), dated as of [●] (the "***Date of Grant***"), is delivered by Aceragen, Inc. (formerly known as Idera Pharmaceuticals, Inc.) (the "***Company***") to [●] (the "***Participant***").

RECITALS

The Idera Pharmaceuticals, Inc. 2022 Equity Incentive Plan (as may be amended from time to time, the "***Plan***") provides for the grant of stock options to purchase shares of Company common stock, par value $0.001 per share ("***Company Stock***"). The Committee has decided to make this nonqualified stock option grant as an inducement for the Participant to promote the best interests of the Company and its stockholders. This Agreement is made pursuant to the Plan and is subject in its entirety to all applicable provisions of the Plan. Capitalized terms used herein and not otherwise defined will have the meanings set forth in the Plan.

The Participant must accept the terms and conditions of this Agreement electronically through the electronic acceptance procedure established by the Company. In no event shall this option be exercised in the absence of such acceptance.

1. Grant of Option. Subject to the terms and conditions set forth in this Agreement and in the Plan, the Company hereby grants to the Participant a nonqualified stock option (the "***Option***") to purchase [●] shares of Company Stock (each a "***Share***", and together the "***Shares***") at an Exercise Price of $[●] per Share. The Option shall become exercisable according to Section 2 below.

2. Exercisability of Option.

(a) Subject to the terms of this Section 2, the Option shall become vested according to the following dates (each a "***Vesting Date***"), provided that the Participant continues to be employed by, or provide service to, the Employer from the Date of Grant until the applicable Vesting Date: twenty-five percent (25%) of the Shares subject to the Option shall vest on the first anniversary of the Date of Grant, and the Option shall become vested and exercisable with respect to the remaining Shares subject to the Option in equal monthly installments over the thirty-six (36)-month period following such anniversary. Notwithstanding the foregoing, (i) the vesting terms set forth herein shall be subject to any special vesting terms set forth in any employment agreement between the Participant and the Company; and (ii) vesting of the Option shall cease to occur upon the Participant's death or Disability.

(b) The vesting and exercisability of the Option is cumulative, but shall not exceed 100% of the Shares subject to the Option. If the terms set forth in Section 2(a) would produce fractional Shares, the number of Shares for which the Option becomes vested and exercisable shall be rounded down to the nearest whole Share and the fractional Shares will be accumulated so that the resulting whole Shares will be included in the number of Shares for which the Option becomes vested and exercisable on the last Vesting Date.

(c) In the event of a Change of Control before the Option is fully vested and exercisable, the provisions of the Plan applicable to a Change of Control shall apply to the Option, and, in the event of a Change of Control, the Committee may take such actions with respect to the vesting and exercisability of the Option as it deems appropriate pursuant to the Plan.

3. Term of Option.

(a) The Option shall have a term of ten years from the Date of Grant and shall terminate at the expiration of that period, unless it is terminated at an earlier date pursuant to the provisions of this Agreement or the Plan. Notwithstanding the foregoing, in the event that on the last business day of the term of the Option, the exercise of the Option is prohibited by applicable law, including a prohibition on purchases or sales of Company Stock under the Company's insider trading policy, the term of the Option shall be extended for a period of 30 days following the end of the legal prohibition, unless the Committee determines otherwise.

(b) The Option shall automatically terminate upon the happening of the first of the following events:

(i) The expiration of the 90-day period after the Participant ceases to be employed by, or provide service to, the Employer, if the termination is for any reason other than Disability, death or Cause.

(ii) The expiration of the one-year period after the Participant ceases to be employed by, or provide service to, the Employer on account of the Participant's Disability.

(iii) The expiration of the one-year period after the Participant ceases to be employed by, or provide service to, the Employer, if the Participant dies while employed by, or providing service to, the Employer or the Participant dies within 90 days after the Participant ceases to be so employed or to provide services to the Employer for any reason other than Disability, death or Cause.

(iv) The date on which the Participant ceases to be employed by, or provide service to, the Employer for Cause. In addition, notwithstanding the prior provisions of this Section 3, if the Participant engages in conduct that constitutes Cause after the Participant's employment or service terminates, the Option shall immediately terminate, and the Participant shall automatically forfeit all Shares underlying any exercised portion of the Option for which the Company has not yet delivered the Share certificates, upon refund by the Company of the Exercise Price paid by the Participant for such Shares.

Notwithstanding the foregoing, in no event may the Option be exercised after the date that is immediately before the tenth anniversary of the Date of Grant, except as provided under Section 3(a) above. Any portion of the Option that is not exercisable at the time the Participant ceases to be employed by, or provide service to, the Employer shall immediately terminate.

4. Exercise Procedures.

(a) Subject to the provisions of Sections 2 and 3 above, the Participant may exercise part or all of the exercisable Option by giving the Company or its delegate either (i) written notice of intent to exercise or (ii) electronic notice of exercise via the Company's online equity management platform (e.g., UBS

OneSource), specifying the number of shares of Company Stock as to which the Option is to be exercised and, in each case, such other information as the Company or its delegate may require.

(b) At such time as the Committee shall determine, the Participant shall pay the Exercise Price (i) in cash, (ii) unless the Committee determines otherwise, by delivering shares of Company Stock owned by the Participant, which shall be valued at their Fair Market Value on the date of exercise, or by attestation (in accordance with procedures prescribed by the Company) to ownership of shares of Company Stock having a Fair Market Value on the date of exercise at least equal to the Exercise Price, (iii) by payment through a broker in accordance with procedures permitted by Regulation T of the Federal Reserve Board, (iv) if permitted by the Committee, by withholding shares of Company Stock subject to the exercisable Option, which have a Fair Market Value on the date of exercise equal to the Exercise Price, or (v) by such other method as the Committee may approve, to the extent permitted by applicable law. The Committee may impose from time to time such limitations as it deems appropriate on the use of shares of Company Stock to exercise the Option.

(c) The obligation of the Company to deliver Shares upon exercise of the Option shall be subject to all applicable laws, rules, and regulations and such approvals by governmental agencies as may be deemed appropriate by the Committee, including such actions as Company counsel shall deem necessary or appropriate to comply with relevant securities laws and regulations.

(d) All obligations of the Employer under this Agreement shall be subject to the rights of the Employer as set forth in the Plan to withhold amounts required to be withheld for any taxes, if applicable. The Participant shall be required to pay to the Company, or make other arrangements satisfactory to the Employer to provide for the payment of, any federal, state, local or other taxes that the Employer is required to withhold with respect to the Option. At such time as the Committee may determine, the Participant may elect to satisfy any tax withholding obligation of the Employer with respect to the Option by having Shares withheld to satisfy the applicable withholding tax rate for FICA, federal, state, local and other tax liabilities.

(e) Upon exercise of the Option (or portion thereof), the Option (or portion thereof) will terminate and cease to be outstanding.

5. Restrictions on Exercise. Except as the Committee may otherwise permit pursuant to the Plan, only the Participant may exercise the Option during the Participant's lifetime and, after the Participant's death, the Option shall be exercisable (subject to the limitations specified in the Plan) solely by the legal representatives of the Participant, or by the Person who acquires the right to exercise the Option by will or by the laws of descent and distribution, to the extent that the Option is exercisable pursuant to this Agreement.

6. Grant Subject to Plan Provisions. This grant is made pursuant to the Plan, the terms of which are incorporated herein by reference, and in all respects shall be interpreted in accordance with the Plan. The grant and exercise of the Option are subject to the provisions of the Plan and to interpretations, regulations and determinations concerning the Plan established from time to time by the Committee in accordance with the provisions of the Plan, including, but not limited to, provisions pertaining to (a) rights and obligations with respect to withholding taxes, (b) the registration, qualification or listing of the Shares, (c) changes in capitalization of the Company, (d) other requirements of applicable law, and (e) governing law for the Plan and this Agreement. The Committee shall have the authority to interpret and construe the Option pursuant to the terms of the Plan, and its decisions shall be conclusive as to any questions arising hereunder.

7. No Employment or Other Rights. The grant of the Option shall not confer upon the Participant any right to be retained by or in the employ or service of the Employer and shall not interfere in any way with the right of the Employer to terminate the Participant's employment or service at any time. The right of the

Employer to terminate at will the Participant's employment or service at any time for any reason is specifically reserved.

8. No Stockholder Rights. Neither the Participant, nor any Person entitled to exercise the Participant's rights in the event of the Participant's death, shall have any of the rights and privileges of a stockholder with respect to the Shares subject to the Option, until certificates for Shares have been issued upon the exercise of the Option.

9. Assignment and Transfers. Except as the Committee may otherwise permit pursuant to the Plan, the rights and interests of the Participant under this Agreement may not be sold, assigned, encumbered or otherwise transferred except, in the event of the death of the Participant, by will or by the laws of descent and distribution. In the event of any attempt by the Participant to alienate, assign, pledge, hypothecate, or otherwise dispose of the Option or any right hereunder, except as provided for in this Agreement, or in the event of the levy or any attachment, execution or similar process upon the rights or interests hereby conferred, the Company may terminate the Option by notice to the Participant, and the Option and all rights hereunder shall thereupon become null and void. The rights and protections of the Company hereunder shall extend to any successors or assigns of the Company and to the Company's parents, subsidiaries, and Affiliates. This Agreement may be assigned by the Company without the Participant's consent.

10. Applicable Law. The validity, construction, interpretation and effect of this Agreement shall be governed by and construed in accordance with the laws of the State of Delaware, without giving effect to the conflicts of laws provisions thereof.

11. Notice. Any notice to the Company provided for in this instrument shall be addressed to the Company in care of its General Counsel or Chief Financial Officer and any notice to the Participant shall be addressed to such Participant at the current address shown on the payroll of the Employer. Any notice shall be delivered by hand or enclosed in a properly sealed envelope addressed as stated above, registered and deposited, postage prepaid, in a post office regularly maintained by the United States Postal Service or by the postal authority of the country in which the Participant resides or to an internationally recognized expedited mail courier.

12. Company Policies. The Participant acknowledges and agrees that the Option and the right to receive and retain any Shares purchased in connection therewith shall be subject to any applicable clawback or recoupment policies, share trading policies and other policies that may be approved or implemented by the Board or the Committee from time to time, whether or not approved before or after the Date of Grant. Accordingly, the Participant agrees that, subject to the requirements of applicable law, the Option, and the right to receive and retain any Shares, or the amount of any gain realized or payment received as a result of any sale or other disposition of the Shares, covered by this Agreement, shall be subject to rescission, cancellation or recoupment, in whole or part, if and to the extent so provided under any "clawback" or similar policy of the Company in effect on the Date of Grant or that may be established thereafter that is applicable to the Participant.

13. Entire Agreement. This Agreement contains the entire understanding between the Company and Participant with respect to the matter set forth herein, and shall supersede all prior and contemporaneous agreements and understandings, inducements or conditions, express or implied, oral or written.

14. Application of Section 409A of the Code. This Agreement is intended to be exempt from Section 409A of the Code and to the extent this Agreement is subject to Section 409A of the Code, it will in all respects be administered in accordance with Section 409A of the Code.

[***Signature Page Follows***]

IN WITNESS WHEREOF, the Company has caused an officer to execute this Agreement, and the Participant has executed this Agreement, effective as of the Date of Grant.

**ACERAGEN, INC.**

By: _____
Name:
Title:

I hereby accept the Option described in this Agreement, and I agree to be bound by the terms of the Plan and this Agreement. I hereby further agree that all decisions and determinations of the Committee shall be final and binding.

**Participant**

By: _____
Name:
Date:

***By clicking "I Agree" or "Accepted" or words to that effect on the online award acceptance tool made available to the Participant by the Company or its stock plan administrator, the Participant is acknowledging that they have read this Agreement, the Plan and all of the documents referred to herein and is agreeing to abide by all of their terms.***

**ACERAGEN, INC.**

**NONQUALIFIED STOCK OPTION GRANT AGREEMENT**
**GRANTED PURSUANT TO THE 2022 EQUITY INCENTIVE PLAN**
**FOR DIRECTORS OF THE COMPANY**

This NONQUALIFIED STOCK OPTION GRANT AGREEMENT (the "***Agreement***"), dated as of [●] (the "***Date of Grant***"), is delivered by Aceragen, Inc. (formerly known as Idera Pharmaceuticals, Inc.) (the "***Company***") to [●] (the "***Participant***").

RECITALS

The Idera Pharmaceuticals, Inc. 2022 Equity Incentive Plan (as may be amended from time to time, the "***Plan***") provides for the grant of stock options to purchase shares of Company common stock, par value $0.001 per share ("***Company Stock***"). The Committee has decided to make this nonqualified stock option grant as an inducement for the Participant to promote the best interests of the Company and its stockholders. This Agreement is made pursuant to the Plan and is subject in its entirety to all applicable provisions of the Plan. Capitalized terms used herein and not otherwise defined will have the meanings set forth in the Plan.

The Participant must accept the terms and conditions of this Agreement electronically through the electronic acceptance procedure established by the Company. In no event shall this option be exercised in the absence of such acceptance.

1. Grant of Option. Subject to the terms and conditions set forth in this Agreement and in the Plan, the Company hereby grants to the Participant a nonqualified stock option (the "***Option***") to purchase [●] shares of Company Stock (each a "***Share***", and together the "***Shares***") at an Exercise Price of $[●] per Share. The Option shall become exercisable according to Section 2 below.

2. Exercisability of Option.

(a) Subject to the terms of this Section 2, the Option shall become vested according to the following date(s) (each, a "***Vesting Date***"), provided that the Participant continues to provide services to the Company from the Date of Grant until the applicable Vesting Date: [one-third of the Shares underlying the Option on the first anniversary of the Date of Grant and the remaining Shares underlying the Option vest in eight equal quarterly installments following the first anniversary of the Date of Grant until all Shares underlying the Option have vested on the third anniversary of the Date of Grant.] [100% of the Shares underlying the Option shall vest on the first anniversary of the Date of Grant.]

(b) The vesting and exercisability of the Option is cumulative, but shall not exceed 100% of the Shares subject to the Option. If the terms set forth in Section 2(a) would produce fractional Shares, the number of Shares for which the Option becomes vested and exercisable shall be rounded down to the nearest whole Share and the fractional Shares will be accumulated so that the resulting whole Shares will be included in the number of Shares for which the Option becomes vested and exercisable on the last Vesting Date.

(c) In the event of a Change of Control before the Option is fully vested and exercisable, the provisions of the Plan applicable to a Change of Control shall apply to the Option, and, in the event of a

Change of Control, the Committee may take such actions with respect to the vesting and exercisability of the Option as it deems appropriate pursuant to the Plan.

3. Term of Option.

(a) The Option shall have a term of ten years from the Date of Grant and shall terminate at the expiration of that period, unless it is terminated at an earlier date pursuant to the provisions of this Agreement or the Plan. Notwithstanding the foregoing, in the event that on the last business day of the term of the Option, the exercise of the Option is prohibited by applicable law, including a prohibition on purchases or sales of Company Stock under the Company's insider trading policy, the term of the Option shall be extended for a period of 30 days following the end of the legal prohibition, unless the Committee determines otherwise.

(b) The Option shall automatically terminate upon the happening of the first of the following events:

(i) The expiration of the 90-day period after the Participant ceases to provide service to the Company, if the termination is for any reason other than Disability, death or Cause.

(ii) The expiration of the one-year period after the Participant ceases to provide service to the Company on account of the Participant's Disability.

(iii) The expiration of the one-year period after the Participant ceases to provide service to the Company, if the Participant dies while providing service to the Company or the Participant dies within 90 days after the Participant ceases to provide services to the Company for any reason other than Disability, death or Cause.

(iv) The date on which the Participant ceases to provide service to the Company for Cause. In addition, notwithstanding the prior provisions of this Section 3, if the Participant engages in conduct that constitutes Cause after the Participant's service terminates, the Option shall immediately terminate, and the Participant shall automatically forfeit all Shares underlying any exercised portion of the Option for which the Company has not yet delivered the Share certificates, upon refund by the Company of the Exercise Price paid by the Participant for such Shares.

Notwithstanding the foregoing, in no event may the Option be exercised after the date that is immediately before the tenth anniversary of the Date of Grant, except as provided under Section 3(a) above. Any portion of the Option that is not exercisable at the time the Participant ceases to provide services to the Company shall immediately terminate.

4. Exercise Procedures.

(a) Subject to the provisions of Sections 2 and 3 above, the Participant may exercise part or all of the exercisable Option by giving the Company or its delegate either (i) written notice of intent to exercise or (ii) electronic notice of exercise via the Company's online equity management platform (e.g., UBS OneSource), specifying the number of shares of Company Stock as to which the Option is to be exercised and, in each case, such other information as the Company or its delegate may require.

(b) At such time as the Committee shall determine, the Participant shall pay the Exercise Price (i) in cash, (ii) unless the Committee determines otherwise, by delivering shares of Company Stock owned by the Participant, which shall be valued at their Fair Market Value on the date of exercise, or by attestation

(in accordance with procedures prescribed by the Company) to ownership of shares of Company Stock having a Fair Market Value on the date of exercise at least equal to the Exercise Price, (iii) by payment through a broker in accordance with procedures permitted by Regulation T of the Federal Reserve Board, (iv) if permitted by the Committee, by withholding shares of Company Stock subject to the exercisable Option, which have a Fair Market Value on the date of exercise equal to the Exercise Price, or (v) by such other method as the Committee may approve, to the extent permitted by applicable law. The Committee may impose from time to time such limitations as it deems appropriate on the use of shares of Company Stock to exercise the Option.

(c) The obligation of the Company to deliver Shares upon exercise of the Option shall be subject to all applicable laws, rules, and regulations and such approvals by governmental agencies as may be deemed appropriate by the Committee, including such actions as Company counsel shall deem necessary or appropriate to comply with relevant securities laws and regulations.

(d) All obligations of the Company under this Agreement shall be subject to the rights of the Company as set forth in the Plan to withhold amounts required to be withheld for any taxes, if applicable. The Participant shall be required to pay to the Company, or make other arrangements satisfactory to the Company to provide for the payment of, any federal, state, local or other taxes that the Company is required to withhold with respect to the Option. At such time as the Committee may determine, the Participant may elect to satisfy any tax withholding obligation of the Company with respect to the Option by having Shares withheld to satisfy the applicable withholding tax rate for FICA, federal, state, local and other tax liabilities.

(e) Upon exercise of the Option (or portion thereof), the Option (or portion thereof) will terminate and cease to be outstanding.

5. Restrictions on Exercise. Except as the Committee may otherwise permit pursuant to the Plan, only the Participant may exercise the Option during the Participant's lifetime and, after the Participant's death, the Option shall be exercisable (subject to the limitations specified in the Plan) solely by the legal representatives of the Participant, or by the Person who acquires the right to exercise the Option by will or by the laws of descent and distribution, to the extent that the Option is exercisable pursuant to this Agreement.

6. Grant Subject to Plan Provisions. This grant is made pursuant to the Plan, the terms of which are incorporated herein by reference, and in all respects shall be interpreted in accordance with the Plan. The grant and exercise of the Option are subject to the provisions of the Plan and to interpretations, regulations and determinations concerning the Plan established from time to time by the Committee in accordance with the provisions of the Plan, including, but not limited to, provisions pertaining to (a) rights and obligations with respect to withholding taxes, (b) the registration, qualification or listing of the Shares, (c) changes in capitalization of the Company, (d) other requirements of applicable law, and (e) governing law for the Plan and this Agreement. The Committee shall have the authority to interpret and construe the Option pursuant to the terms of the Plan, and its decisions shall be conclusive as to any questions arising hereunder.

7. No Employment or Other Rights. The grant of the Option shall not confer upon the Participant any right to be retained by or in the service of the Company and shall not interfere in any way with the right of the Company to terminate the Participant's service at any time. The right of the Company to terminate at will the Participant's service at any time for any reason is specifically reserved.

8. No Stockholder Rights. Neither the Participant, nor any Person entitled to exercise the Participant's rights in the event of the Participant's death, shall have any of the rights and privileges of a stockholder with respect to the Shares subject to the Option, until certificates for Shares have been issued upon the exercise of the Option.

9. Assignment and Transfers. Except as the Committee may otherwise permit pursuant to the Plan, the rights and interests of the Participant under this Agreement may not be sold, assigned, encumbered or otherwise transferred except, in the event of the death of the Participant, by will or by the laws of descent and distribution. In the event of any attempt by the Participant to alienate, assign, pledge, hypothecate, or otherwise dispose of the Option or any right hereunder, except as provided for in this Agreement, or in the event of the levy or any attachment, execution or similar process upon the rights or interests hereby conferred, the Company may terminate the Option by notice to the Participant, and the Option and all rights hereunder shall thereupon become null and void. The rights and protections of the Company hereunder shall extend to any successors or assigns of the Company and to the Company's parents, subsidiaries, and Affiliates. This Agreement may be assigned by the Company without the Participant's consent.

10. Applicable Law. The validity, construction, interpretation and effect of this Agreement shall be governed by and construed in accordance with the laws of the State of Delaware, without giving effect to the conflicts of laws provisions thereof.

11. Notice. Any notice to the Company provided for in this instrument shall be addressed to the Company in care of its General Counsel or Chief Financial Officer and any notice to the Participant shall be addressed to such Participant at the current address shown on the records of the Company. Any notice shall be delivered by hand or enclosed in a properly sealed envelope addressed as stated above, registered and deposited, postage prepaid, in a post office regularly maintained by the United States Postal Service or by the postal authority of the country in which the Participant resides or to an internationally recognized expedited mail courier.

12. Company Policies. The Participant acknowledges and agrees that the Option and the right to receive and retain any Shares purchased in connection therewith shall be subject to any applicable clawback or recoupment policies, share trading policies and other policies that may be approved or implemented by the Board or the Committee from time to time, whether or not approved before or after the Date of Grant. Accordingly, the Participant agrees that, subject to the requirements of applicable law, the Option, and the right to receive and retain any Shares, or the amount of any gain realized or payment received as a result of any sale or other disposition of the Shares, covered by this Agreement, shall be subject to rescission, cancellation or recoupment, in whole or part, if and to the extent so provided under any "clawback" or similar policy of the Company in effect on the Date of Grant or that may be established thereafter that is applicable to the Participant.

13. Entire Agreement. This Agreement contains the entire understanding between the Company and Participant with respect to the matter set forth herein, and shall supersede all prior and contemporaneous agreements and understandings, inducements or conditions, express or implied, oral or written.

14. Application of Section 409A of the Code. This Agreement is intended to be exempt from Section 409A of the Code and to the extent this Agreement is subject to Section 409A of the Code, it will in all respects be administered in accordance with Section 409A of the Code.

[***Signature Page Follows***]

IN WITNESS WHEREOF, the Company has caused an officer to execute this Agreement, and the Participant has executed this Agreement, effective as of the Date of Grant.

**ACERAGEN, INC.**

By: _____
Name:
Title:

I hereby accept the Option described in this Agreement, and I agree to be bound by the terms of the Plan and this Agreement. I hereby further agree that all decisions and determinations of the Committee shall be final and binding.

**Participant**

By: _____
Name:
Date:

***By clicking "I Agree" or "Accepted" or words to that effect on the online award acceptance tool made available to the Participant by the Company or its stock plan administrator, the Participant is acknowledging that they have read this Agreement, the Plan and all of the documents referred to herein and is agreeing to abide by all of their terms.***

**AMENDMENT NO. 1 TO**
**EXECUTIVE TRANSITION AND SEPARATION AGREEMENT**

This Amendment No. 1 to Executive Transition and Separation Agreement (this "Agreement"), is entered into as of the date set forth on the signature page below (the "Execution Date"), by and between Vincent Milano ("You") and Aceragen, Inc. (formerly known as Idera Pharmaceuticals, Inc.), a Delaware corporation (together with its wholly owned subsidiaries and affiliates, the "Company"). You and the Company are collectively referred to as the "Parties" throughout this Agreement.

**BACKGROUND**

WHEREAS, You and the Company are parties to that certain Executive Transition and Separation Agreement dated September 28, 2022 (the "Executive Services Agreement") which set forth the terms and conditions of the termination of your employment with the Company, including the severance payable to you following the Termination Date and the Approval, each as defined in the Executive Services Agreement;

WHEREAS, Section 3(iii) of the Executive Services Agreement provides for severance payable following the Approval in the form of fully vested shares of common stock of the Company ("Common Stock") equal to a number of shares, calculated by dividing $800,000 by the volume-weighted average price per share of Common Stock based on the twenty (20) trading days prior to the date of grant, rounded down to the nearest full share (the "Stock Consideration") and the Stock Consideration was to be granted as soon as practicable, but in no event more than thirty (30) days, following the Approval; and

WHEREAS, the Parties desire to amend the Executive Services Agreement in certain respects as described herein.

NOW THEREFORE, in consideration of the mutual promises set forth in this Agreement and of other good and valuable consideration, the sufficiency of which you acknowledge, and intending to be legally bound hereby, you and the Company agree as follows:

1. Amendments. Section 3(iii) of the Executive Services Agreement shall be replaced in its entirety with the following:

> "You will receive fully vested shares of common stock of the Company ("Common Stock") equal to a number of shares, calculated by dividing $800,000 by the volume-weighted average price per share of Common Stock based on the twenty (20) trading days prior to the date of grant, rounded down to the nearest full share (the "Stock Consideration"). The Stock Consideration will be granted as soon as practicable following the date the Company consummates an equity financing pursuant to which it sells shares of Common Stock in exchange for the payment of the purchase price of such stock in cash which results in net proceeds of at least five million dollars ($5,000,000) (the "Equity Financing Date"), but in no event later than the earlier of (i) thirty (30) days following the Equity Financing Date and (ii) June 30, 2023.

2. Governing Law. This Agreement will be governed by and construed in accordance with the laws of the Commonwealth of Pennsylvania.

3. Counterparts. This Amendment may be executed in counterparts with the same effect as if both Parties had signed the same documents. All such counterparts shall be deemed an original, shall be construed together, and shall constitute one and the same instrument.

4. Executive Services Agreement Remains in Effect. Except as provided herein, all provisions, terms and conditions of the Executive Services Agreement shall remain in full force and effect. As amended hereby, the Executive Services Agreement is ratified and confirmed in all respects.

[Signature page follows]

IN WITNESS WHEREOF, and intending to be legally bound hereby, you and the Company hereby execute the foregoing Amendment No. 1 to Executive Transition and Separation Agreement as of the Execution Date set forth below.

| **VINCENT MILANO** | **ACERAGEN, INC.** |
| --- | --- |
| /s/ Vincent Milano | /s/ Bryant D. Lim |
| | By: Bryant D. Lim |
| | Title: Chief Business Officer and General Counsel |
| Date: February 10, 2023 | Date: February 10, 2023 |

[Signature Page to Agreement]

**AMENDMENT NO. 1 TO**
**EXECUTIVE TRANSITION AND SEPARATION AGREEMENT**

This Amendment No. 1 to Executive Transition and Separation Agreement (this "Agreement"), is entered into as of the date set forth on the signature page below (the "Execution Date"), by and between Daniel Soland ("You") and Aceragen, Inc. (formerly known as Idera Pharmaceuticals, Inc.), a Delaware corporation (together with its wholly owned subsidiaries and affiliates, the "Company"). You and the Company are collectively referred to as the "Parties" throughout this Agreement.

**BACKGROUND**

WHEREAS, You and the Company are parties to that certain Executive Transition and Separation Agreement dated September 28, 2022 (the "Executive Services Agreement") which set forth the terms and conditions of the termination of your employment with the Company, including the severance payable to you following the Termination Date and the Approval, each as defined in the Executive Services Agreement;

WHEREAS, pursuant to Section 2(b) of the Executive Services Agreement, You agreed to provide certain advisory and transition services to the Company from the Termination Date, as defined in the Executive Services Agreement, through the thirtieth (30th) day following the Approval, as defined in the Executive Services Agreement (the "Advisory Services Agreement");

WHEREAS, Section 3(iii) of the Executive Services Agreement provides for severance payable following the Approval in the form of fully vested shares of common stock of the Company ("Common Stock") equal to a number of shares, calculated by dividing $500,000 by the volume-weighted average price per share of Common Stock based on the twenty (20) trading days prior to the date of grant, rounded down to the nearest full share (the "Stock Consideration") and the Stock Consideration was to be granted as soon as practicable, but in no event more than thirty (30) days, following the Approval; and

WHEREAS, the Parties desire to amend the Executive Services Agreement and the Advisory Services Agreement set forth therein, in certain respects as described herein.

NOW THEREFORE, in consideration of the mutual promises set forth in this Agreement and of other good and valuable consideration, the sufficiency of which you acknowledge, and intending to be legally bound hereby, you and the Company agree as follows:

1. Amendments.

(a) Section 2(b) of the Executive Services Agreement, shall be replaced in its entirety with the following:

> "In entering into this Agreement, you agree to the Advisory Services Agreement set forth herein and agree to provide certain advisory and transition services to the Company on an as needed basis as requested by the Company from time to time from the Termination Date through June 30, 2023 (the "Services"). You will perform the Services in a professional manner, consistent with industry standards and in compliance with all applicable laws and regulations. In all respects, you will be providing the Services as an independent contractor, not an employee. You will not hold yourself out as an employee, partner, co-venturer, agent or representative of the Company. You may not make any promise or representation, or execute any contract, for the Company."

(b) Section 3(iii) of the Executive Services Agreement shall be replaced in its entirety with the following:

> "You will receive fully vested shares of common stock of the Company ("Common Stock") equal to a number of shares, calculated by dividing $500,000 by the volume-weighted average price per share of Common Stock based on the twenty (20) trading days prior to the date of grant, rounded down to the nearest full share (the "Stock Consideration"). The Stock Consideration will be granted as soon

as practicable following the date the Company consummates an equity financing pursuant to which it sells shares of Common Stock in exchange for the payment of the purchase price of such stock in cash which results in net proceeds of at least five million dollars ($5,000,000) (the "Equity Financing Date"), but in no event later than the earlier of (i) thirty (30) days following the Equity Financing Date and (ii) June 30, 2023.

2. Governing Law. This Agreement will be governed by and construed in accordance with the laws of the Commonwealth of Pennsylvania.

3. Counterparts. This Amendment may be executed in counterparts with the same effect as if both Parties had signed the same documents. All such counterparts shall be deemed an original, shall be construed together, and shall constitute one and the same instrument.

4. Executive Services Agreement Remains in Effect. Except as provided herein, all provisions, terms and conditions of the Executive Services Agreement shall remain in full force and effect. As amended hereby, the Executive Services Agreement is ratified and confirmed in all respects.

[Signature page follows]

IN WITNESS WHEREOF, and intending to be legally bound hereby, you and the Company hereby execute the foregoing Amendment No. 1 to Executive Transition and Separation Agreement as of the Execution Date set forth below.

| | |
|---|---|
| **DANIEL SOLAND** | **ACERAGEN, INC.** |
| /s/ Daniel Soland | /s/ Bryant D. Lim |
| | By: Bryant D. Lim |
| | Title: Chief Business Officer and General Counsel |
| Date: February 10, 2023 | Date: February 10, 2023 |

[Signature Page to Agreement]

**Exhibit 23.1**

**Consent of Independent Registered Public Accounting Firm**

We consent to the incorporation by reference in the following Registration Statements:

(1) Registration Statement (Form S-8 No. 333-152669) pertaining to the 2008 Stock Incentive Plan of Idera Pharmaceuticals, Inc.

(2) Registration Statement (Form S-8 No. 333-176067) pertaining to the 2008 Stock Incentive Plan and 1995 Employee Stock Purchase Plan of Idera Pharmaceuticals, Inc.

(3) Registration Statement (Form S-8 No. 333-191076) pertaining to the 2013 Stock Incentive Plan of Idera Pharmaceuticals, Inc.

(4) Registration Statement (Form S-8 No. 333-197062) pertaining to the 2013 Stock Incentive Plan of Idera Pharmaceuticals, Inc.

(5) Registration Statement (Form S-8 No. 333-202691) pertaining to Inducement Stock Option Awards of Idera Pharmaceuticals, Inc.

(6) Registration Statement (Form S-8 No. 333-206129) pertaining to the 2013 Stock Incentive Plan, as amended, of Idera Pharmaceuticals, Inc.

(7) Registration Statement (Form S-8 No. 333-210090) pertaining to an Inducement Stock Option Award of Idera Pharmaceuticals, Inc.

(8) Registration Statement (Form S-1 as amended by Form S-3/A No. 333-136610) of Idera Pharmaceuticals, Inc.

(9) Registration Statement (Form S-1 as amended by Form S-3/A No. 333-187155) of Idera Pharmaceuticals, Inc.

(10) Registration Statement (Form S-2 as amended by Form S-3/A No. 333-109630) of Idera Pharmaceuticals, Inc.

(11) Registration Statement (Form S-3 No. 333-119943) of Idera Pharmaceuticals, Inc.

(12) Registration Statement (Form S-3 No. 333-126634) of Idera Pharmaceuticals, Inc.

(13) Registration Statement (Form S-3 No. 333-131804) of Idera Pharmaceuticals, Inc.

(14) Registration Statement (Form S-3 No. 333-133455) of Idera Pharmaceuticals, Inc.

(15) Registration Statement (Form S-3 No. 333-133456) of Idera Pharmaceuticals, Inc.

(16) Registration Statement (Form S-3 No. 333-139830) of Idera Pharmaceuticals, Inc.

(17) Registration Statement (Form S-3 as amended by Form S-3/A No. 333-185392) of Idera Pharmaceuticals, Inc.

(18) Registration Statement (Form S-3 No. 333-186312) of Idera Pharmaceuticals, Inc.

(19) Registration Statement (Form S-3 No. 333-189700) of Idera Pharmaceuticals, Inc.

(20) Registration Statement (Form S-3 No. 333-210140) of Idera Pharmaceuticals, Inc.

(21) Registration Statement (Form S-8 No. 333-217665) pertaining to an Inducement Stock Option Award of Idera Pharmaceuticals, Inc.

(22) Registration Statement (Form S-8 No. 333-219740) pertaining to the 2017 Employee Stock Purchase Plan of Idera Pharmaceuticals, Inc.

(23) Registration Statement (Form S-8 No. 333-219741) pertaining to the 2013 Stock Incentive Plan, as amended, of Idera Pharmaceuticals, Inc.

(24) Registration Statement (Form S-8 No. 333-232609) pertaining to the 2017 Employee Stock Purchase Plan of Idera Pharmaceuticals, Inc.

(25) Registration Statement (Form S-8 No. 333-232610) pertaining to the 2013 Stock Incentive Plan, as amended, of Idera Pharmaceuticals, Inc.

(26) Registration Statement (Form S-3 No. 333-238868) of Idera Pharmaceuticals, Inc.

(27) Registration Statement (Form S-3 No. 333-240361) of Idera Pharmaceuticals, Inc.

(28) Registration Statement (Form S-3 No. 333-240366) of Idera Pharmaceuticals, Inc.

(29) Registration Statement (Form S-3 No. 333-248560) of Idera Pharmaceuticals, Inc.

(30) Registration Statement (Form S-3 and S-3/A No. 333-253804) of Idera Pharmaceuticals, Inc.

(31) Registration Statement (Form S-8 No. 333-266038) pertaining to the 2013 Stock Incentive Plan, as amended, of Idera Pharmaceuticals, Inc.

(32) Registration Statement (Form S-8 No. 333-266039) pertaining to the 2017 Employee Stock Purchase Plan, as amended, of Idera Pharmaceuticals, Inc.

(33) Registration Statement (Form S-8 No. 333-268965) pertaining to the 2021 Stock Incentive Plan of Aceragen, Inc.

(34) Registration Statement (Form S-8 No. 333-269511) pertaining to the 2022 Stock Incentive Plan of Idera Pharmaceuticals, Inc. and 2021 Stock Incentive Plan of Aceragen, Inc.

of our report dated April 13, 2023, with respect to the consolidated financial statements of Aceragen, Inc. included in this Annual Report (Form 10-K) of Aceragen, Inc. for the year ended December 31, 2022.

/s/ ERNST & YOUNG LLP

Philadelphia, Pennsylvania
April 13, 2023

**Exhibit 31.1**

**Certification of Chief Executive Officer pursuant to Exchange Act Rules 13a-14 and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002**

I, John Taylor, certify that:

1. I have reviewed this Annual Report on Form 10-K of Aceragen, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this annual report is being prepared;
    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
    d) Disclosed in this report any changes in the registrant's internal control over financial reporting that occurred during the registrant's fourth fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control and financial reporting.

/s/ John Taylor
John Taylor
Chief Executive Officer

Dated: April 13, 2023

**Exhibit 31.2**

**Certification of Chief Financial Officer pursuant to Exchange Act Rules 13a-14 and 15d-14, as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002**

I, John J. Kirby, certify that:

1. I have reviewed this Annual Report on Form 10-K of Aceragen, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this annual report is being prepared;
    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
    d) Disclosed in this report any changes in the registrant's internal control over financial reporting that occurred during the registrant's fourth fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control and financial reporting.

/s/ John J. Kirby
John J. Kirby
Chief Financial Officer

Dated: April 13, 2023

**Exhibit 32.1**

**Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**

In connection with the Annual Report on Form 10-K of Aceragen, Inc. (the "Company") for the period ended December 31, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, John Taylor, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

A signed original of this written statement has been provided to Aceragen, Inc. and will be retained by Aceragen, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

/s/ John Taylor
John Taylor
Chief Executive Officer

Dated: April 13, 2023

**Exhibit 32.2**

**Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**

In connection with the Annual Report on Form 10-K of Aceragen, Inc. (the "Company") for the period ended December 31, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, John J. Kirby, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

A signed original of this written statement has been provided to Aceragen, Inc. and will be retained by Aceragen, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

/s/ John J. Kirby
John J. Kirby
Chief Financial Officer

Dated: April 13, 2023