

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549

SCHEDULE 14A

**PROXY STATEMENT PURSUANT TO SECTION 14(a) OF THE
SECURITIES EXCHANGE ACT OF 1934**

Filed by the Registrant

Filed by a party other than the Registrant

Check the appropriate box:

- Preliminary Proxy Statement
- Confidential, for Use of the Commission Only (as permitted by Rule 14a-6(e)(2))**
- Definitive Proxy Statement
- Definitive Additional Materials
- Soliciting Material under § 240.14a-12

Aceragen, Inc.

(Name of Registrant as Specified in Its Charter)

(Name of Person(s) Filing Proxy Statement, if other than the Registrant)

Payment of Filing Fee (Check all boxes that apply):

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ACERAGEN, INC.
505 Eagleview Blvd., Suite 212
Exton, PA 19341
(484) 348-1600

Dear Stockholder:

You are invited to attend a special meeting of stockholders (the “Special Meeting”) of Aceragen, Inc., a Delaware corporation (“Aceragen”), which will be held on August 14, 2023 at 10:00 a.m. Eastern Time, as it may be adjourned or postponed from time to time. The Special Meeting will be conducted in-person at the offices of Morgan, Lewis & Bockius LLP, located at 1701 Market Street, Philadelphia, PA 19103.

The attached Notice of Special Meeting of Stockholders and proxy statement contain details of the business to be conducted at the Special Meeting.

Whether or not you attend the Special Meeting, it is important that your shares be represented and voted at the meeting. Therefore, I urge you to promptly vote and submit your proxy via the Internet, by phone, or by signing, dating and returning the enclosed proxy card in the enclosed envelope. If you decide to attend the Special Meeting, you will be able to change your vote or revoke your proxy, even if you have previously submitted your proxy.

On behalf of Aceragen, I would like to thank you for your continued support.

Sincerely,

/s/ John Taylor

John Taylor
*President, Chief Executive Officer
and Chief Financial Officer*

ACERAGEN, INC.
505 Eagleview Blvd., Suite 212
Exton, Pennsylvania 19341
(484) 348-1600

NOTICE OF SPECIAL MEETING OF STOCKHOLDERS

Time and Date	August 14, 2023 at 10:00 a.m. Eastern Time
Place	The special meeting of stockholders (the "Special Meeting") of Aceragen, Inc. ("Aceragen," the "Company," "we," "us" and "our") will held at the offices of Morgan, Lewis & Bockius LLP, 1701 Market Street, Philadelphia, PA 19103.
Items of Business	<ul style="list-style-type: none"> • To consider and vote upon a proposal to approve the resolutions attached hereto as <u>Exhibit A</u>, approving and authorizing the Board's determination to effect the transfer of all or substantially all of Aceragen's assets through an assignment for the benefit of creditors (the "Assignment," and such proposal, the "Assignment Proposal"). • To grant discretionary authority to Aceragen's board of directors (the "Board") to adjourn the Special Meeting, from time to time, to a later date or dates, even if a quorum is present, to solicit additional proxies in the event that there are insufficient shares present in person or by proxy voting in favor of the Assignment Proposal (the "Adjournment Proposal"). • To transact such other business as may properly come before the Special Meeting or any adjournments, postponements or continuations thereof.
Board Recommendations	<p>After careful consideration of a number of factors, as described in the attached proxy statement, the Board has unanimously determined that the Assignment is advisable and in the best interests of Aceragen and its stockholders.</p> <p>The Board unanimously recommends that you vote "FOR" both the Assignment Proposal and the Adjournment Proposal.</p>
Record Date	The close of business on July 19, 2023 (the "Record Date"). Only stockholders on the Record Date are entitled to receive notice of, and to vote at, the Special Meeting.
Proxy Voting	<p>IMPORTANT</p> <p>Please vote your shares at your earliest convenience. Promptly voting your shares via the Internet, by telephone, or by signing, dating and returning the enclosed proxy card will save the expenses and extra work of additional solicitation. If you wish to vote by mail, an addressed envelope is enclosed, postage prepaid if mailed in the United States. Submitting your proxy now will not prevent you from voting your shares at the Special Meeting, as your proxy is revocable at your option.</p>

Important Notice Regarding the Availability of Proxy Materials for the Special Meeting to Be Held on August 14, 2023 at 10:00 a.m. Eastern Time. This proxy statement is available on the "Investors" section of Aceragen's website at investors.aceragen.com and it is being mailed to stockholders on or about July 24, 2023.

By order of the Board of Directors,

/s/ John Taylor

John Taylor
President, Chief Executive Officer
and Chief Financial Officer
July 21, 2023

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This proxy statement, and the documents incorporated by reference into this proxy statement, contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding: Aceragen's ability to effect the Assignment, projected cash runways, and stockholder approval of the Assignment Proposal. The use of words such as, but not limited to, "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "will," or "would" and similar words and expressions are intended to identify forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our clinical results and other future conditions. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements. We may not actually achieve the forecasts disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Such forward-looking statements are subject to a number of material risks and uncertainties including but not limited to those set forth under the caption "Risk Factors" in Aceragen's most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (the "SEC"), as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the SEC. Any forward-looking statement speaks only as of the date on which it was made. Neither we, nor our affiliates, advisors or representatives, undertake any obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date hereof.

WEBSITES

Website addresses referenced in this proxy statement are provided for convenience only, and the content on the referenced websites does not constitute a part of this proxy statement.

SUMMARY

This summary highlights selected information contained in this proxy statement and may not contain all of the information that is important to you. To understand fully the legal requirements for the transfer of all or substantially all of the assets of Aceragen, Inc. (“Aceragen,” the “Company,” “we,” “us,” and “our”) through an assignment for the benefit of creditors under Delaware law and the Special Meeting (as defined below), you should carefully read this entire proxy statement and the documents delivered with this proxy statement.

About Aceragen

Aceragen is a clinical-stage biopharmaceutical company 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. Aceragen’s common stock, par value \$0.001 per share (the “Common Stock”), is traded on the Nasdaq Capital Market under the symbol “ACGN.”

General

Aceragen’s Board of Directors (the “Board”) has determined that due to, among other things, an inability to raise the additional capital needed to continue to fund Aceragen’s operations and service its obligations in the future, it is advisable and in the best interests of Aceragen and Aceragen’s stockholders that Aceragen assigns all or substantially all of Aceragen’s assets for the benefit of Aceragen’s creditors (the “Assignment”). The Assignment is a judicial insolvency procedure, which, assuming approval of the Assignment Proposal by Aceragen’s stockholders, is commenced by Aceragen entering a contractual assignment for the benefit of creditors (the “Assignment Agreement”) that effectuates the assignment, grant, conveyance, transfer, and setting over to a third-party assignee (the “Assignee”), in trust, of all of Aceragen’s currently existing right, title, and interest in all real or personal property and all other assets, whatsoever and where so ever situated. The Assignee will then file an application in the Delaware Court of Chancery, which commences a judicial proceeding for recognition of the assignment for the benefit of creditors. The Assignee will then liquidate the assets for the general benefit of all Aceragen’s creditors, without preference, according to their respective priorities at law to satisfy Aceragen’s obligations. If any proceeds remain after all of Aceragen’s obligations to creditors have been satisfied in full, those remaining proceeds will be distributed to you, the stockholders. Since Aceragen does not know the final amount that the Assignee will recover from a liquidation of Aceragen’s assets, Aceragen does not know whether any amounts will be available for distribution to the stockholders. The Board will determine the Assignee.

In furtherance of the Assignment, and consistent with requirements under Delaware law, the Board is presenting the Assignment Proposal (as defined below) for approval by Aceragen’s stockholders.

At the Special Meeting, Aceragen’s stockholders will be asked to consider and vote upon proposals to (i) approve the resolutions attached hereto as Exhibit A, approving and authorizing the Board’s determination to effect the Assignment (the “Assignment Proposal”) and (ii) grant discretionary authority to the Board to adjourn the Special Meeting, from time to time, to a later date or dates, even if a quorum is present, to solicit additional proxies in the event that there are insufficient shares present in person or by proxy voting in favor of the Assignment Proposal (the “Adjournment Proposal”).

Upon the execution of the Assignment Agreement followed by the filing of an application in the Delaware Court of Chancery to commence a judicial proceeding for recognition of the assignment for the benefit of creditors, the Assignee will have sole control over Aceragen’s assets and Aceragen will no longer control the operation of its business, the liquidation or distribution of its assets or the resolution of claims. If the Assignment Proposal is approved, Aceragen intends to seek delisting from the Nasdaq Capital Market. Whether or not the Assignment Proposal is approved, Aceragen continues to have an obligation to comply with the applicable reporting requirements of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Aceragen intends to seek relief from the Securities and Exchange Commission (the “SEC”) from certain reporting requirements under the Exchange Act, which may substantially reduce publicly available information about Aceragen. However, the SEC may not grant Aceragen the requested relief.

**QUESTIONS AND ANSWERS REGARDING THIS SOLICITATION
AND VOTING AT THE SPECIAL MEETING**

The following are some of the questions you may have as an Aceragen stockholder and answers to those questions. These questions and answers highlight only some of the information contained in this proxy statement. You should read carefully this entire document, including all exhibits and annexes hereto, to fully understand the Assignment Proposal, the Adjournment Proposal and the voting procedures for the Special Meeting.

Special Meeting and Voting

Why am I receiving these materials, and who is soliciting my vote?

We sent you this proxy statement because our Board is soliciting your proxy to vote at the Special Meeting that Aceragen is holding to seek stockholder approval of the Assignment Proposal and the Adjournment Proposal, as described in further detail herein. This proxy statement summarizes the information you need to vote at the Special Meeting. You do not need to attend the Special Meeting to vote your shares.

When are this proxy statement and the accompanying materials scheduled to be sent to stockholders?

On or about July 24, 2023, we will begin mailing our proxy materials, including this proxy statement, and the accompanying proxy card or, for shares held in street name (i.e., shares held for your account by a broker or other nominee), a voting instruction form.

When and where is the Special Meeting going to be held?

The special meeting will be held at 10:00 a.m. Eastern Time on August 14, 2023 at the offices of Morgan, Lewis & Bockius LLP, 1701 Market Street, Philadelphia, PA 19103 (together with any adjournment, postponement, continuation or other delay thereof, the "Special Meeting").

What is the purpose of the Special Meeting?

At the Special Meeting, stockholders will vote on the matters described in the accompanying Notice of Special Meeting and this proxy statement. The only matters expected to be voted upon at the Special Meeting are the Assignment Proposal and the Adjournment Proposal.

The Board is proposing the Assignment, and asking stockholders to approve the Assignment Proposal, as we are unable to continue our ongoing operations with our current cash and anticipated future cash flow and we have been unable to secure additional equity, debt or other financing. The Board has a duty to take the actions that it believes will result in the best recovery for Aceragen's creditors while preserving, without any assurance, the potential for a distribution of any residual value to stockholders. The Board has therefore deemed it advisable and in the best interests of Aceragen and its stockholders to effectuate the Assignment. The Board believes that when compared to a filing under federal bankruptcy laws (which the Board believes is the only alternative to the Assignment), the Assignment presents the best opportunity for the highest possible recovery under the circumstances for creditors, and while uncertain, preserving the opportunity for future payments to Aceragen's stockholders depending on the results of the Assignee's liquidation process.

When is the Record Date for the Special Meeting?

The Record Date for determination of stockholders entitled to vote at the Special Meeting is the close of business on July 19, 2023.

Which stockholders may vote?

The Board has fixed the close of business on July 19, 2023 as the Record Date for determining the stockholders of Aceragen who are entitled to receive notice of the Special Meeting and to vote their shares at the Special Meeting. Only stockholders as of the Record Date will be entitled to notice of, and to vote at,

the Special Meeting. Each share of Aceragen's Common Stock is entitled to one vote. As of the Record Date, Aceragen had issued and outstanding 8,420,276 shares of Common Stock.

What am I being asked to vote on?

The Board is asking Aceragen's stockholders of record at the close of business on July 19, 2023, the Record Date for the Special Meeting, to consider and vote upon the Assignment Proposal and the Adjournment Proposal. The Board currently knows of no other business that will be presented for consideration at the Special Meeting. In the event any matters other than those referred to in the accompanying Notice of Special Meeting and this proxy statement should properly come before and be considered at the Special Meeting, it is intended that proxies in the form Aceragen provides to its stockholders will be voted thereon in accordance with the discretion of the person or persons voting such proxies.

What are the recommendations of the Board for how I should vote my shares?

The Board unanimously recommends that you vote "FOR" the Assignment Proposal and "FOR" the Adjournment Proposal.

Why is Aceragen seeking a stockholder vote on the Assignment Proposal?

Under Section 271(a) of the Delaware General Corporation Law, a Delaware corporation must obtain stockholder approval, by the holders of a majority of the outstanding stock of the corporation entitled to vote thereon, before proceeding with a sale of all or substantially all of its property and assets, as contemplated by the Assignment. The Board therefore is seeking stockholder approval of the Assignment Proposal in order to comply with Delaware law.

Stockholders holding a majority of Aceragen's outstanding shares have already committed to vote their shares in favor of an Assignment pursuant to Stockholder Voting Agreements, as discussed below.

Why is Aceragen seeking a stockholder vote on the Adjournment Proposal?

Adjourning the Special Meeting to a later date will give the Board additional time to solicit proxies to vote in favor of approval of the Assignment Proposal if there are not sufficient votes in favor of the proposal. Consequently, Aceragen is seeking your approval of the Adjournment Proposal to ensure that, if necessary, Aceragen will have enough time to solicit the required votes for approval of the Assignment Proposal.

Who can attend the Special Meeting?

Only stockholders of record as of the close of business on the Record Date, or their duly appointed proxies, may attend the Special Meeting. To attend and participate in the Special Meeting, you will need the control number included on your proxy card. The Special Meeting will begin promptly on August 14, 2023 at 10:00 a.m. Eastern Time. You are encouraged to arrive at the meeting prior to the start time. Check-in will begin at 9:30 a.m. Eastern Time and you should allow ample time for the check-in procedures. A list of stockholders entitled to vote at the Special Meeting will be available (i) ten days prior to the Special Meeting and ending on the day prior to the Special Meeting upon request and (ii) consistent with the provisions of Aceragen's Second Amended and Restated By-Laws, during the Special Meeting.

Do I need an admission ticket to attend the Special Meeting?

Admission to the Special Meeting will be by proxy card control number only. If you are a stockholder of record and plan to attend the Special Meeting, you will need the control number included on your proxy card. We may ask you to present evidence of share ownership as of the Record Date, such as an account statement indicating ownership on that date, and valid photo identification, such as a driver's license or passport, to enter the Special Meeting.

What do I need to do to attend the Special Meeting?

If you were a stockholder of record at the close of business on July 19, 2023, you do not need to do anything in advance to attend and/or vote your shares at the Special Meeting.

If you were a beneficial owner at the close of business on the Record Date (meaning your shares are held in “street name” through a bank, broker or other nominee), you may not vote your shares at the Special Meeting unless you obtain a “legal proxy” from your broker, bank or other nominee who is the stockholder of record with respect to your shares.

How many votes must be present at the Special Meeting to constitute a quorum?

A quorum is the minimum number of shares required to be present at the Special Meeting for the meeting to be properly held under Aceragen’s bylaws and Delaware law. A quorum consists of the holders of at least one-third of the shares of our Common Stock issued and outstanding and entitled to vote at the Special Meeting. As of the Record Date, there were 8,420,276 shares of Aceragen’s Common Stock issued and outstanding. Shares of Common Stock present or represented by proxy (including broker non-votes and shares that are abstained or withheld or with respect to which no voting instructions are provided for one or more of the matters to be voted upon) will be counted for the purpose of determining whether a quorum exists.

If a quorum is not present, the Special Meeting will be adjourned until a quorum is obtained.

What vote is needed for each of the proposals to be adopted?

Under Delaware law, the affirmative vote of a majority of the outstanding shares of capital stock of Aceragen entitled to vote thereon is required to approve the Assignment Proposal. Abstentions and broker non-votes will have the same effect as votes “Against” the Assignment Proposal.

If a quorum is present at the Special Meeting, the affirmative vote of the holders of shares of Common Stock representing a majority of the votes present or represented and voting on the matter is required for the approval of the Adjournment Proposal. If a quorum is not present at the Special Meeting, the affirmative vote of the holders of a majority of the shares of Common Stock present at the Special Meeting or represented by proxy is required for the approval of the Adjournment Proposal. Broker non-votes (if any) and abstentions will not be counted as votes cast on the matter and will have no effect on the outcome of this proposal.

Stockholders holding a majority of our outstanding shares entitled to vote on the Assignment Proposal have already committed to vote their shares in favor of the Assignment Proposal pursuant to Stockholder Voting Agreements, as discussed below.

What is a broker non-vote?

Generally, a broker non-vote occurs when shares held by a bank, broker or other nominee for a beneficial owner are not voted with respect to a particular proposal because (i) the nominee has not received voting instructions from the beneficial owner and (ii) the nominee lacks discretionary voting power to vote such shares. Under applicable stock exchange rules, banks, brokers and other nominees who hold shares of Aceragen’s Common Stock for beneficial owners have the discretion to vote on routine matters when they have not received voting instructions from those beneficial owners. On a non-routine matter, banks, brokers and other nominees do not have the discretion to direct the voting of the beneficial owners’ shares (as they do on a routine matter), and, if the beneficial owner has not provided voting instructions with respect to that matter, there will be a “broker non-vote” on the matter. Aceragen urges you to provide instructions to your bank, broker or other nominee so that your votes may be counted for each proposal to be voted upon. You should provide voting instructions for your shares by following the instructions provided on the voting instruction form that you receive from your bank, broker or other nominee.

It is expected that both the Assignment Proposal and the Adjournment Proposal will be considered non-routine matters.

How can I vote?

You can vote in person or by valid proxy received by telephone, via the Internet or by mail. If you plan to attend the Special Meeting, you may vote during the meeting. Please have your proxy card in hand when you arrive. If you are unable to attend the Special Meeting, Aceragen urges you to submit a proxy by doing one of the following:

- *Submit a proxy by telephone:* To vote by telephone, dial toll-free 1-800-652-8683 and follow the recorded instructions. You will be asked to provide the control number from the proxy card. Your vote must be received by 11:59 p.m. on August 13, 2023 to be counted. If you vote by telephone, you do not need to return a proxy card by mail.
- *Submit a proxy via the Internet:* To vote via the Internet, go to <http://www.investorvote.com/ACGN> to complete an electronic proxy card. You will be asked to provide the control number from the proxy card you receive. Your vote must be received by 11:59 p.m. on August 13, 2023 to be counted. If you vote via the Internet, you do not need to return a proxy card by mail.
- *Submit a proxy by mail:* If you choose to submit a proxy by mail, sign, date and return your proxy card in the postage-paid envelope provided.

If your shares are held in “street name” through a bank, broker or other nominee, you may vote through your bank, broker or other nominee by completing and returning the voting instruction form provided by your bank, broker or other nominee, or, if such a service is provided by your bank, broker or other nominee, electronically over the Internet or by telephone. To vote over the Internet or by telephone through your bank, broker or other nominee, you should follow the instructions on the voting instruction form provided by your bank, broker or nominee.

If your shares are held in “street name,” you may not vote your shares at the Special Meeting unless you obtain a “legal proxy” from your broker, bank or other nominee who is the stockholder of record with respect to your shares.

Can I change my vote?

Yes. A proxy may be revoked at any time prior to the voting at the Special Meeting by submitting a later dated proxy (including a proxy authorization submitted by telephone or electronically via the Internet prior to the deadline for submitting a proxy by telephone or via the Internet), by sending a properly signed written notice of such revocation to Aceragen’s Corporate Secretary at info@aceragen.com in advance of the Special Meeting, or by attending the Special Meeting and voting by ballot in person. If your shares are held through a bank, broker or other nominee, you may change your voting instructions by submitting a later dated voting instruction form to your broker, bank or other nominee or fiduciary, or if you obtained a legal proxy from your broker, bank nominee or fiduciary giving you the right to vote your shares at the Special Meeting, by attending the Special Meeting and voting in person.

What if I vote for one, but not both, of the proposals?

Shares of Aceragen’s Common Stock represented by proxies received by Aceragen (whether received through the return of the enclosed proxy card or received by telephone or via the Internet) where the stockholder has provided voting instructions with respect to the proposals described in this proxy statement will be voted in accordance with the voting instructions so made. If your proxy card is properly executed and returned but does not contain voting instructions as to one or more of the proposals to be voted upon at the Special Meeting, or if you give your proxy by telephone or via the Internet without indicating how you want to vote on each of the proposals to be voted upon at the Special Meeting, your shares will be voted “FOR” both the Assignment Proposal and the Adjournment Proposal.

Who will pay for the cost of this proxy solicitation?

Aceragen is making the solicitation and will bear the costs of soliciting proxies. In addition to solicitations by mail, our directors, Chief Executive Officer, and employees, without additional remuneration, may solicit proxies by telephone, text message, facsimile, email, personal interviews, and other means. We have requested that brokerage houses, custodians, nominees, and fiduciaries forward copies of the proxy materials to the persons for whom they hold shares and request instructions for voting the proxies. We will reimburse the brokerage houses and other persons for their reasonable out-of-pocket expenses in connection with this distribution.

How can I access the proxy materials electronically?

Copies of the Notice of Special Meeting, this proxy statement and Aceragen's Annual Report on Form 10-K for the year ended December 31, 2022, as well as other materials filed by Aceragen with the SEC, are available without charge to stockholders on Aceragen's corporate website at investors.aceragen.com or upon request at info@aceragen.com.

Proposed Assignment for the Benefit of Creditors***Why is the Board recommending approval of the Assignment Proposal?***

After due consideration of the potential strategic alternatives available to Aceragen based on the costs and benefits of continuing its operations, the Board has determined that the Assignment is advisable and in the best interests of Aceragen and Aceragen's stockholders. Aceragen is unable to continue its ongoing operations with its current cash and anticipated future cash flow and has been unable to secure sufficient equity, debt or other financing. The Board believes that as compared to a filing under federal bankruptcy laws (which the Board believes is the only alternative to the Assignment), the Assignment presents the best opportunity to maximizing recoveries for creditors while preserving an opportunity for future payments of any residual value to stockholders.

What will happen if the Assignment is approved?

The Board proposes that Aceragen assign substantially all of its assets for the benefit of its creditors. The Assignment is a judicial insolvency procedure, which, if approved by Aceragen's stockholders, is commenced by Aceragen entering the Assignment Agreement that effectuates the assignment, grant, conveyance, transfer, and setting over to the Assignee, in trust, all of Aceragen's currently existing right, title, and interest in all real or personal property and all other assets, whatsoever and where so ever situated. The Assignee will then file an application in the Delaware Court of Chancery, which commences a judicial proceeding for recognition of the assignment for the benefit of creditors. The Assignee will then liquidate the assets for the general benefit of all Aceragen's creditors, without preference, according to their respective priorities at law to satisfy Aceragen's obligations. If any proceeds remain after all of Aceragen's obligations to creditors have been satisfied in full, those remaining proceeds, if any, will be distributed to you, the stockholders. Because Aceragen does not know the final amount that the Assignee will recover from a liquidation of Aceragen's assets, Aceragen does not know whether any amounts will be available for distribution to the stockholders.

When do you expect the assignment process to be completed?

Aceragen anticipates that the Assignment will be completed shortly after the date of the Special Meeting.

What will Aceragen's business be after completion of the Assignment?

Upon the completion of the Assignment, the Assignee will have sole control over Aceragen's assets and Aceragen will no longer control the operation of its business, the liquidation or distribution of its assets or the resolution of claims. Aceragen intends to seek delisting from the Nasdaq Capital Market and terminate Aceragen's status as a reporting company with the SEC.

Will I receive anything in this transaction?

Based upon information available at this time, Aceragen cannot forecast whether any amounts will be available to Aceragen's stockholders in connection with, or as a result of, the Assignment. Depending on the results of the Assignee's liquidation and claims resolution process, both of which will determine to the extent Aceragen's debts are ultimately satisfied, it is possible that residual value could become available for distribution to stockholders from the liquidation proceeds. However, there are too many variables and uncertainties for us to estimate whether any amounts will actually be paid or the amount of any such payments.

Can I still sell my shares of Common Stock?

Yes, for a limited period of time. We expect that our Common Stock will continue to be traded on the Nasdaq Capital Market prior to the Special Meeting. However, the Board may direct that our stock transfer books be closed and that recording of transfers of Common Stock discontinued upon the transfer of our assets to the Assignee.

Do I have appraisal rights?

No. Under Delaware law, you do not have appraisal rights in connection with any of the proposals.

Who can help answer my questions?

If you have any additional questions about the Special Meeting, the Assignment, the Assignment Proposal or the Adjournment Proposal, how to submit your proxy, or if you need additional copies of this proxy statement or the enclosed proxy card or voting instructions, please contact Aceragen at info@aceragen.com or (484) 348-1600.

THE SPECIAL MEETING

This proxy statement is being furnished in connection with the solicitation of proxies on behalf of the Board for use at the Special Meeting to be held on August 14, 2023 at 10:00 a.m. Eastern Time at Morgan, Lewis & Bockius LLP, 1701 Market Street, Philadelphia, PA 19103.

Purpose

The Special Meeting is being held to request that stockholders consider and vote upon the Assignment Proposal and the Adjournment Proposal, each as described in this proxy statement.

Record Date; Stockholders Entitled to Vote

The Board has specified the close of business on July 19, 2023 as the Record Date for purpose of determining the stockholders of Aceragen who are entitled to receive notice of and to vote at the Special Meeting. Only Aceragen's stockholders as of the Record Date are entitled to notice of and to vote at the Special Meeting. As of the Record Date, there were 8,420,276 shares of Aceragen's Common Stock issued and outstanding and entitled to notice of and to vote at the Special Meeting. Each share of Aceragen's Common Stock entitles its holder to one vote on each matter that properly comes before the Special Meeting.

Quorum; Required Votes

The Special Meeting will be held only if a quorum, consisting of at least one-third of the shares of Aceragen's Common Stock issued and outstanding and entitled to vote at the Special Meeting, is represented in person or by proxy. At the close of business on July 19, 2023, the Record Date for the Special Meeting, 8,420,276 shares of Common Stock were outstanding and eligible to vote at the Special Meeting, meaning that 2,806,759 shares of Common Stock must be represented at the Special Meeting in person or by proxy in order to have a quorum.

If a quorum is not present, the Special Meeting will be adjourned until a quorum is obtained.

The affirmative vote of a majority of the outstanding shares of capital stock of Aceragen entitled to vote thereon is required to approve the Assignment Proposal. Abstentions and broker non-votes will have the same effect as votes "Against" the Assignment Proposal.

If a quorum is present at the Special Meeting, the Adjournment Proposal must be approved by the affirmative vote of the holders of shares of Common Stock representing a majority of the votes present or represented and voting on the matter. If a quorum is not present at the Special Meeting, the affirmative vote of the holders of a majority of the shares of Common Stock present at the Special Meeting or represented by proxy is required for the approval of the Adjournment Proposal. Broker non-votes (if any) and abstentions will not be counted as votes cast on the matter and will have no effect on the outcome of this proposal.

Stockholders holding a majority of our outstanding shares have already committed to vote their shares in favor of the Assignment Proposal pursuant to Stockholder Voting Agreements, as discussed below.

Broker Non-Votes and Abstentions

For each proposal, you may vote "FOR," "AGAINST" or "ABSTAIN." Abstentions will count for the purpose of determining whether a quorum is present at the Special Meeting.

Generally, a broker non-vote occurs when shares held by a bank, broker or other nominee for a beneficial owner are not voted with respect to a particular proposal because (i) the nominee has not received voting instructions from the beneficial owner and (ii) the nominee lacks discretionary voting power to vote such shares. Under applicable stock exchange rules, banks, brokers and other nominees who hold shares of Aceragen's common stock for beneficial owners have the discretion to vote on routine matters when they have not received voting instructions from those beneficial owners. On a non-routine matter, banks, brokers and other nominees do not have the discretion to direct the voting of the beneficial owners' shares (as they do on a routine matter), and, if the beneficial owner has not provided voting instructions with respect

to that matter, there will be a “broker non-vote” on the matter. Aceragen urges you to provide instructions to your bank, broker or other nominee so that your votes may be counted for each proposal to be voted upon. You should provide voting instructions for your shares by following the instructions provided on the voting instruction form that you receive from your bank, broker or other nominee.

Broker non-votes, if any, will be counted for purposes of calculating whether a quorum is present at the Special Meeting.

Stockholder Voting Agreements and Irrevocable Proxy

On July 10, 2023, Aceragen entered into voting agreements (the “Stockholder Voting Agreements”) with Atul Chopra, John Taylor, Dan Salain and Andy Jordan (the “Majority Stockholders”), pursuant to which the Majority Stockholders have agreed to vote their shares of Aceragen at any annual or special meeting of stockholders of Aceragen, or in any action by written consent of stockholders, in favor of the Assignment. Pursuant to the Stockholder Voting Agreements, the Majority Stockholders granted Aceragen an irrevocable proxy to vote their shares with regard to the Assignment Proposal at any annual or special meeting of stockholders of Aceragen or in connection with any action by written consent by the stockholders of Aceragen.

Recommendation of the Board

The Board has unanimously approved effecting the Assignment and determined that proceeding with the Assignment and the other agreements and transactions contemplated by the Assignment are advisable to and in the best interests of Aceragen and Aceragen’s stockholders. The Board unanimously recommends that Aceragen’s stockholders approve the Board’s determination by voting “FOR” the approval of the Assignment Proposal. For a description of the factors considered by the Board in making its determinations with respect to the Assignment Proposal, see the section of this proxy statement captioned “*Reasons for the Proposed Assignment.*” The Board also unanimously recommends that Aceragen’s stockholders vote “FOR” the Adjournment Proposal.

THE BOARD UNANIMOUSLY RECOMMENDS THAT YOU VOTE “FOR” BOTH THE ASSIGNMENT PROPOSAL AND THE ADJOURNMENT PROPOSAL.

Solicitation of Proxies and Voting Procedures

Your shares may be voted at the Special Meeting only if you are present or represented by proxy. Whether or not you plan to attend the Special Meeting, you are encouraged to submit a proxy to ensure that your shares will be represented. See the section of this proxy statement captioned “*Questions and Answers Regarding This Solicitation and Voting at the Special Meeting — How do I vote?*” for additional information.

Voting by, and Revocability of, Proxies

If you submit a proxy via the Internet, by telephone or by returning a signed proxy card by mail, your shares will be voted at the Special Meeting as you indicate. If you sign your proxy card without indicating your vote, your shares will be voted “FOR” each of the Assignment Proposal and the Adjournment Proposal. If you hold your shares through an account with a bank or a broker, you will receive instructions from your broker, bank or other nominee that you must follow in order to have your shares voted. If you fail to correctly follow the instructions or your broker, bank or other nominee your shares may not be voted. See the section of this proxy statement captioned “*Broker Non-Votes and Abstentions*” above for additional information.

A proxy may be revoked at any time prior to the voting at the Special Meeting by submitting a later dated proxy (including a proxy authorization submitted by telephone or electronically via the Internet prior to the deadline for submitting a proxy by telephone or via the Internet), by sending a properly signed written notice of such revocation to Aceragen’s Corporate Secretary in advance of the Special Meeting or by attending the Special Meeting and voting by ballot. If your shares are held through a bank, broker or other nominee, you may change your voting instructions by submitting a later dated voting instruction form to your broker, bank or other nominee or fiduciary, or if you obtained a legal proxy from your broker, bank

nominee or fiduciary giving you the right to vote your shares at the Special Meeting, by attending the Special Meeting and voting by ballot. You should direct any written notices of revocation and related correspondence to Aceragen's Corporate Secretary at info@aceragen.com.

Questions and Additional Information

If you have questions about the Assignment, the Assignment Proposal, the Adjournment Proposal or how to submit your proxy, or if you need additional copies of this proxy statement or the enclosed proxy card or voting instructions, please contact Aceragen at info@aceragen.com or (484) 348-1600.

PROPOSAL 1: APPROVAL OF ASSIGNMENT

About Aceragen

Aceragen is a clinical-stage biopharmaceutical company 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. Aceragen's Common Stock is traded on the Nasdaq Capital Market under the symbol "ACGN." Prior to January 17, 2023, we were known as Idera Pharmaceuticals, Inc. On September 28, 2022, we acquired all of the outstanding equity interests in Aceragen, Inc., a private company ("Legacy Aceragen" and such transaction, the "Aceragen Acquisition"). In connection with the Aceragen Acquisition and related transactions, we changed our name to Aceragen, Inc.

General

On July 10, 2023, the Board determined that it is advisable and in the best interests of Aceragen and Aceragen's stockholders to effect the transfer of all or substantially all of Aceragen's assets through the Assignment. In furtherance of the Assignment, and pursuant to Delaware law, the Board is presenting the Assignment Proposal for approval by Aceragen's stockholders.

If the Assignment is approved by Aceragen's stockholders, Aceragen expects to transfer all of Aceragen's right, title, interest in, and custody and control of Aceragen's property to a third-party assignee in trust, as the Assignee. The Assignee will then liquidate the property and distribute the proceeds to Aceragen's creditors to satisfy Aceragen's obligations. If any proceeds remain after all of Aceragen's obligations have been satisfied, those remaining proceeds, if any, will be distributed to the stockholders. Because Aceragen does not know the final amount that the Assignee will recover from a liquidation of Aceragen's assets, Aceragen does not know whether any amounts will be available for distribution to the stockholders. The Board will determine the Assignee.

Summary of the Proposed Assignment

Upon the completion of the Assignment, the Assignee will have sole control over Aceragen's assets and Aceragen will no longer control the operation of its business, the liquidation or distribution of its assets or the resolution of claims. Aceragen intends to seek delisting from the Nasdaq Capital Market and terminate Aceragen's status as a reporting company with the SEC.

Based upon information available at this time, Aceragen cannot forecast whether any amounts will be available to Aceragen's stockholders in connection with, or as a result of, the Assignment. There is a possibility that if the Assignee is able to resolve all of Aceragen's obligations, that some amount of liquidation proceeds could be paid to stockholders. However, there are too many variables and uncertainties for us to estimate whether any amounts will actually be paid or the amount of any such payments.

Any distributions from Aceragen will be made to stockholders according to their holdings of Common Stock as of the Record Date.

During the Assignment process, Aceragen will pay certain of its officers, directors, service providers and agents, or any of them, compensation for services rendered in connection with continued operations of the Company's business and the implementation of the Assignment. Such compensation is not expected to be materially different from the compensation that would be paid to an outside party for similar services. Aceragen's directors and officers may be entitled to deferred compensation payments and indemnification and insurance coverage from Aceragen.

Reasons for the Proposed Assignment

After seeking potential funding sources and other ways to continue to operate Aceragen's business, Aceragen has been unable to find a viable alternative to the Assignment. The Board believes that, as compared to a filing under federal bankruptcy laws (which the Board believes is the only alternative), the Assignment presents the best opportunity for the best recovery for creditors and also may provide an

opportunity for future payments to stockholders. At this time, the Board has considered these options and has determined that it is in Aceragen’s best interests and in the best interests of Aceragen’s stockholders to effect the Assignment.

In arriving at this determination, the Board considered such terms and alternatives, as well as the following factors:

- Aceragen’s financial projections and continuing negative cash flow;
- Aceragen’s overall debts;
- Aceragen’s inability to continue to fund its existing operations with its current cash flow;
- Aceragen’s inability to raise additional capital; and
- general economic conditions.

The Board also considered the following risks in arriving at its conclusion that the Assignment is in Aceragen’s best interests and the best interests of Aceragen’s stockholders:

- the uncertainty of the timing, nature and amount of any distributions to stockholders;
- the possibility that the Assignment would not yield distributions to stockholders in excess of the amount that stockholders could have received upon a sale or other transaction involving Aceragen or a sale of shares on the open market;
- that terms and conditions of the Bridge Funding Agreement and Security Agreement with NovaQuest (as defined below), pursuant to which NovaQuest has agreed to provide up to \$3.0 million in financing to fund the Company until it is able to effect the Assignment to the Assignee and, thereafter, the Assignee’s efforts to liquidate the Company’s assets; and
- the possibility that certain of Aceragen’s executive officers and directors may have financial interests in the Assignment that may be different from, or in addition to, the interests of Aceragen’s stockholders generally.

In view of the variety of factors considered in connection with its evaluation of the Assignment, the Board did not find it practical, and did not quantify or otherwise attempt, to assign relative weight to the specific factors considered in reaching its conclusions. In addition, the Board did not undertake to make any specific determination as to whether any particular factor, or any aspect of any particular factor, was favorable or unfavorable to its ultimate determination, but rather conducted an overall analysis of the factors described above. In considering the factors described above, individual members of the Board may have given different weight to different factors.

Aceragen cannot offer any assurance that distributions, if any, to Aceragen’s stockholders following the Assignment will equal or exceed the price or prices at which shares of Common Stock recently have traded on Nasdaq Capital Market or could trade over-the-counter in the future. However, the Board believes that it is in Aceragen’s best interests and the best interests of Aceragen’s stockholders to approve the Assignment.

Background of the Proposed Assignment

Despite extensive efforts to raise additional financing, Aceragen has been unable to raise additional capital needed to continue to fund Aceragen’s operations and service its obligations in the future. Pursuant to a Bridge Funding Agreement, dated as of July 11, 2023 (the “Bridge Funding Agreement”), by and between the Company and NovaQuest Co-Investment Fund XV, L.P. (“NovaQuest”), NovaQuest has agreed to provide up to \$3.0 million in financing to fund certain liabilities of the Company and continuing operations of the Company until it is able to effect the Assignment to the Assignee and, thereafter, the Assignee’s efforts to liquidate the Company’s assets. Aceragen and NovaQuest also entered into a security agreement as of July 11, 2023 (the “Security Agreement”), pursuant to which Aceragen granted NovaQuest a security interest in Aceragen’s property in connection with the funds provided pursuant to the Bridge Funding Agreement. On July 10, 2023, the Board determined that the Assignment was advisable and in the best interests of Aceragen and Aceragen’s stockholders and approved the entry into the Bridge Funding Agreement, the Security Agreement, and the Stockholder Voting Agreements.

Consequences to Stockholders

Based upon information available at this time, we cannot forecast if Aceragen’s stockholders will receive any cash, stock or other property in connection with, or as a result of, the Assignment. Our Common Stock will, subject to regulatory considerations, be delisted following the Assignment and we plan to take steps to terminate our status as a reporting company with the SEC following the Special Meeting. As a result, we expect that our stockholders will have no readily available means to dispose of their shares of Common Stock following the Assignment.

Assignment Under Delaware Law

Delaware law provides that a corporation may make an assignment for the benefit of creditors upon the approval of a corporation’s board of directors followed by a majority vote of its stockholders. If such assignment is approved by the corporation’s stockholders, such corporation transfers all of its right, title, interest in, and custody and control of its property to a third-party assignee in trust. Such assignee then liquidates the property and distributes the proceeds to such corporation’s creditors to satisfy the corporation’s obligations. If any proceeds remain after all of the corporation’s obligations and costs associated with the liquidation process have been satisfied, remaining proceeds will be distributed to the corporation’s stockholders.

Absence of Appraisal Rights

Under Delaware law, Aceragen’s stockholders are not entitled to appraisal, dissenters’ or similar rights in connection with the Assignment.

Conduct of Aceragen Following the Approval of the Assignment

This section of the proxy statement describes material aspects of the proposed Assignment and the steps Aceragen and the Board expect to take in connection with the Assignment. While Aceragen believes that this description covers the material terms of the Assignment, this summary may not contain all of the information that is important to you. You should carefully read this entire proxy statement, including the section captioned “*Summary of the Assignment.*”

Approval of Assignment

To proceed with the Assignment, the Assignment Proposal must be approved by the affirmative vote of a majority of the outstanding shares of capital stock of Aceragen. The approval of the Assignment Proposal by the requisite vote of the holders of Aceragen’s Common Stock will constitute complete approval and authorization for the Board, without further stockholder action, to proceed with the Assignment in accordance with any applicable provision of Delaware law.

Assignment

If the Assignment Proposal is approved by the requisite vote of Aceragen’s stockholders, the steps set forth below may be completed at such times as the Board, in its discretion and in accordance with Delaware law, deems necessary, appropriate or advisable in Aceragen’s best interests and the best interests of Aceragen’s stockholders:

- the cessation of all of Aceragen’s business activities except those relating to the Assignment; and
- the taking of any and all other actions permitted or required by Delaware law and any other applicable laws and regulations.

In addition, if the Board determines that the Assignment is not in Aceragen’s best interest, the Board may direct that the Assignment be abandoned.

Distributions

Based upon information available at this time, Aceragen cannot forecast whether any amounts will be available to Aceragen’s stockholders in connection with, or as a result of, the Assignment or the liquidation

of the Company's assets. There is a possibility that if the Assignee is able to resolve all of Aceragen's obligations, that some amount of liquidation proceeds could be paid to stockholders. However, there are too many variables and uncertainties for us to estimate whether any amounts will actually be paid or the amount of any such payments.

Regulatory Approvals

We are not aware of any U.S. federal or state regulatory requirements or governmental approvals or actions that may be required to consummate the Assignment, except for compliance with applicable SEC regulations in connection with this Proxy Statement and compliance with the DGCL. If Aceragen's stockholders approve the Assignment Proposal and Aceragen enters into the Assignment Agreement, the Assignee will be required to file an application in the Delaware Court of Chancery, which commences a judicial proceeding for recognition of the assignment for the benefit of creditors. If Aceragen's stockholders approve the Assignment Proposal, we intend to enter into the Assignment Agreement and direct the Assignee to file such application as soon as reasonably practicable after the Special Meeting.

Cessation of Trading of Common Stock

We anticipate that we will notify FINRA of the Assignment and request that our Common Stock stop trading on the Nasdaq Capital Market as soon after the Assignment as is reasonably practicable. We also currently expect to close our stock transfer books subsequent to the Special Meeting and to discontinue recording transfers and issuing stock certificates at that time. Accordingly, it is expected that trading in our shares of Common Stock will cease on or very soon after the Assignment.

Reporting Requirements

Whether or not the Assignment Proposal is approved, we have an obligation to continue to comply with the applicable reporting requirements of the Exchange Act, even though compliance with such reporting requirements may be economically burdensome and of minimal value to stockholders. If our stockholders approve the Assignment Proposal, in order to curtail expenses, we intend, following the Special Meeting, to seek relief from the SEC to suspend our reporting obligations under the Exchange Act, and ultimately to terminate the registration of our Common Stock. If the Assignment Proposal is approved and, we transfer our assets to the Assignee, the Assignee (as successor to Aceragen) would likely, if granted relief from the SEC, be required to file periodic reports and current reports on Form 8-K along with any other reports that the SEC might require. It is possible that the SEC may not grant us the requested relief. If we are unable to suspend our obligation to file periodic reports with the SEC, we will be obligated to continue complying with the applicable reporting requirements of the Exchange Act and will be required to continue to incur the expenses associated with these reporting requirements, including legal and accounting expenses.

Accounting Treatment

If Aceragen's stockholders approve the Assignment Proposal, Aceragen will change its basis of accounting to the liquidation basis of accounting. Under the liquidation basis of accounting, assets are stated at their estimated net realizable values, and liabilities are stated at their estimated settlement amounts. Recorded liabilities will include the estimated expenses associated with carrying out the Assignment. For periodic reporting, a statement of net assets in liquidation will summarize the liquidation value per outstanding share of common stock. Valuations presented in the statement will represent management's estimates, based on present facts and circumstances, of the net realizable values of assets, satisfaction amounts of liabilities, and expenses associated with carrying out the Assignment based upon management assumptions. The valuation of assets and liabilities will necessarily require many estimates and assumptions, and there will be substantial uncertainties in carrying out the provisions of the Assignment. Ultimate values realized for Aceragen's assets and ultimate amounts paid to satisfy Aceragen's liabilities are expected to differ from estimates recorded in annual or interim financial statements.

Interests of Certain Persons in the Assignment

Certain of Aceragen's executive officers and directors may have financial interests in the Assignment that may be different from, or in addition to, the interests of Aceragen's stockholders generally. In particular:

- during the Assignment, Aceragen will pay certain of its officers, directors, service providers and agents, or any of them, compensation for services rendered in connection with continued operation

of the Company's business and the implementation of the Assignment. Such compensation is not expected to be materially different from the compensation that would be paid to an outside party for similar services;

- as of July 19, 2023, Aceragen's directors and executive officers beneficially held 2,487,867 shares of Aceragen's Common Stock, including 909,326 warrants to purchase Common Stock of the Company and 75,214 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023. The estimated value of such shares of Aceragen's Common Stock as of July 19, 2023 (based upon the \$1.44 per share closing price of Aceragen's Common Stock as of such date) is \$3,582,528. Aceragen's directors and executive officers who own shares of Aceragen's Common Stock will be entitled to receive, on the same terms and conditions as Aceragen's other stockholders, the same distributions and other benefits that Aceragen's stockholders would receive when Aceragen makes any distributions to Aceragen's stockholders of record; and
- Aceragen's directors and officers may be entitled to deferred compensation payments and indemnification and insurance coverage from Aceragen.

The Board was aware of those potentially differing interests and considered them, among other matters, in evaluating the Assignment and in reaching its decision to approve such plan and the actions contemplated thereby, as more fully discussed in the section of this proxy statement captioned "*Reasons for the Proposed Assignment.*"

Aceragen anticipates that, upon completion of the Assignment, the Board will appoint a sole director and that all other directors will resign.

Equity Compensation Plan Information

The following table provides information as of July 19, 2023 with respect to shares of Aceragen's Common Stock that may be issued under its existing equity compensation plans.

Plan Category	Number of Securities to be Issued upon Exercise of Outstanding Options, Restricted Stock Units and Rights	Weighted Average Exercise Price of Outstanding Options and Rights	Number of Securities Remaining Available for Future Issuance Under Equity Compensation Plans (Excluding Securities Reflected in the first Column)
Equity compensation plans approved by security holders			
Idera Pharmaceuticals, Inc. 2013 Stock Incentive Plan	242,440	\$ 79.39	—
Aceragen, Inc. 2021 Stock Incentive Plan	832,478	\$ 7.23	—
Idera Pharmaceuticals, Inc. 2022 Stock Incentive Plan	218,900	\$ 5.21	1,450,338
All plans approved by security holders	1,293,818	\$ 19.18	1,450,338
Equity compensation plans not approved by security holders			
	14,705	\$424.32	—
TOTAL	1,308,523	\$ 23.82	1,450,338

Executive Officer and Director Equity Holdings

The table below sets forth, with respect to each executive officer and director of Aceragen, as applicable, as of July 19, 2023 (i) the number of shares of Aceragen's Common Stock beneficially held without restriction by each individual, (ii) the value of each individual's Common Stock (based upon the \$1.44 per share closing price of Aceragen's Common Stock as of such date) and (iii) the percentage of the total number of issued and outstanding shares of Aceragen's Common Stock held by each individual (based upon 8,420,276 issued and outstanding shares of Aceragen's Common Stock as of that date).

Name of Beneficial Owner	Number of Shares Beneficially Owned	Value of Common Stock Beneficially Owned	Percentage of Shares Beneficially Owned
Directors and Executive Officers:			
John Taylor	1,469,482	\$2,116,054	17.45%
John J. Kirby	40,586	\$ 58,444	*
Bryant D. Lim	18,217	\$ 26,232	*
Vincent J. Milano	69,502	\$ 100,083	*
Daniel Soland	20,799	\$ 29,951	*
Cristina Csimma	5,762	\$ 8,297	*
Michael R. Dougherty	27,829	\$ 40,074	*
Maxine Gowen	5,966	\$ 8,591	*
Ronald Wooten	909,326	\$1,309,429	9.75%
All current directors and executive officers as a group (6 persons)	2,487,867**	\$3,582,528	26.45%

* Represents beneficial ownership of less than one percent (1%) of the outstanding shares of Aceragen's Common Stock.

** Includes 909,326 warrants to purchase Common Stock of the Company and 75,214 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023.

Employment Agreements and Other Arrangements with Executive Officers

John Taylor Employment Agreement

In connection with the execution of that certain Agreement and Plan of Merger, dated as of September 28, 2022 (the "Effective Date"), pursuant to which we acquired Legacy Aceragen, Mr. Taylor's "at will" employment agreement with Legacy Aceragen, dated February 25, 2021 (the "Taylor Employment Agreement"), was assumed by us 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 Aceragen 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 Aceragen property in his possession and executes a general release of claims in favor of Aceragen, 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 Aceragen portion of the monthly COBRA premiums for Aceragen'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 Aceragen or with Legacy Aceragen, as applicable) on any equity positions in Aceragen 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 Aceragen-sponsored employee benefit plans, including its medical, dental, vision, and 401(k) plans or similar arrangements. Additionally, Aceragen 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 Aceragen's policies. Mr. Taylor is also entitled to receive equity-based awards under Aceragen's equity incentive plans.

On July 19, 2023, the Board approved, as retention and performance incentives for John Taylor, the Company's Chief Executive Officer, relative to the Assignment and the Assignee's liquidation of the Company's assets (the "ABC Sales Process"), (a) that, effective as of June 1, 2023, Mr. Taylor is no longer subject to deferring any the portion of his base salary in excess of \$200,000, such deferral having been previously approved by the Board among other cost-cutting measures, and (b) the grant to Mr. Taylor of a \$100,000 success fee (the "Success Grant"). The Success Grant is contingent upon (i) Mr. Taylor's continued employment with the Company through both the date of the Special Meeting and the Assignment and (ii) Mr. Taylor's provision of consulting services reasonably requested by the Assignee in connection with the ABC Sales Process. Upon the successful completion of the ABC Sales Process, the Success Grant will be added to the other claims that Mr. Taylor holds as a creditor of the Company.

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 former Senior Vice President and Chief Financial Officer and an employment continuation and retention bonus letter agreement (the "Kirby Employee Retention Agreement"). Mr. Kirby had previously entered into an individual severance agreement (the "Kirby Severance Agreement") based on Aceragen's form Severance and Change of Control Agreement (the "Severance Agreement Form"), pursuant to which Mr. Kirby was eligible to receive certain severance payments and benefits upon certain terminations of employment with Aceragen, 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, while the remaining terms of the Kirby Severance Agreement remained in full force and effect. See "*Severance and Change in Control Benefits and Agreements*" below 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"). If Mr. Kirby's employment terminated for any reason (other than by Aceragen for Cause (as defined in the Kirby Severance Agreement)) prior to July 12, 2023, Mr. Kirby was eligible to 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").

As of April 28, 2023, Mr. Kirby no longer serves as Aceragen's Chief Financial Officer and is entitled to the Kirby Cash Retention Bonus.

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 former Senior Vice President, General Counsel, and Secretary. In connection with the Aceragen Acquisition, Aceragen and Mr. Lim entered into an employment continuation and retention bonus letter agreement (the "Lim Employee Retention Agreement") and a severance agreement (the "Lim Severance Agreement") based on the Severance Agreement Form, pursuant to which Mr. Lim was eligible to receive certain severance payments and benefits upon certain terminations of employment with Aceragen, 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 remained in full force and effect. See "*Severance and Change in Control Benefits and Agreements*" below for a description of the material terms of the Severance Agreement Form.

Pursuant to the Lim Employee Retention Agreement, Mr. Lim was eligible to receive an amount in stock and/or cash with an aggregate value equal to \$766,500 (the "Lim Retention Bonus"). If Mr. Lim's employment with Aceragen terminated for any reason (other than by Aceragen for Cause (as defined in the

Lim Severance Agreement)) prior to July 12, 2023, Mr. Lim was eligible 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”).

As Mr. Lim resigned from Aceragen in February 2023, he is entitled to receive the Lim Cash Retention Bonus.

Vincent J. Milano Employment Agreement and Separation Agreement

In connection with the completion of the Aceragen Acquisition, Mr. Milano transitioned from the role of chief executive officer to the role of non-employee Chair of the Board and, in connection with this transition, Aceragen 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 (together with the Original Milano Separation Agreement, the “Milano Separation Agreement”). The Milano Separation Agreement set forth certain amounts to be paid to Mr. Milano 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 and also was 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; (ii) \$606,357, payable in substantially equal installments in accordance with Aceragen’s payroll practices, over the 12 months following the closing of the Aceragen Acquisition; and (iii) fully vested shares of common stock with a value of \$800,000 (the “Milano Contingent Severance”), with the Milano Contingent Severance to be granted as soon as practicable following the date Aceragen 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.

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 completion of the Aceragen Acquisition, Aceragen entered into an executive transition and separation agreement (as amended, the “Soland Separation Agreement”) with Mr. Soland. Under the Soland Separation Agreement, set forth certain amounts to be paid to Mr. Soland 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, and Mr. Soland agreed to provide certain advisory and transition services to Aceragen 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 Aceragen in an independent contractor capacity. In addition, Mr. Soland was 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 closing of the Aceragen Acquisition; (ii) \$459,754, payable in substantially equal installments over the 12-month period starting on the first payroll date following the closing of the Aceragen Acquisition; and (iii) fully vested shares of Common Stock equal to a \$500,000 in value (the “Soland Contingent Severance”), with the Soland Contingent Severance to be granted as soon as practicable following the date Aceragen 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.

Severance and Change in Control Agreements

In 2017, the Board approved the Severance Agreement Form. The Severance Agreement Form provides that if we consummate a change of control (as defined therein), we will employ the executive 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 executive's position and duties for Aceragen will be commensurate with the most significant of the duties and positions held by the executive during the 90-day period preceding the date of the change of control;
- the executive's annual base salary will equal at least 12 times the highest monthly base salary paid to the executive during the 12 months prior to the date of the change of control;
- the executive will be entitled to an annual bonus equal to at least the greatest of (a) the average bonus paid to the executive 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 executive will be entitled to certain other benefits as are consistent with the benefits paid to the executive during the year prior to the change of control.

The Severance Agreement Form also provides that if an executive 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 executive's timely execution and non-revocation of a general release of claims in a form provided by us and the executive's continued compliance with the invention, non-disclosure, and non-competition agreement previously entered into in connection with the commencement of executive's employment, the executive would receive a lump sum cash payment payable within 30 days after the date of termination equal to:

- the executive's target bonus for the year of termination prorated for the portion of the year worked;
- 150% of the sum of (a) such executive'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 executive 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 Aceragen's share of the annual premium for group medical and/or dental insurance coverage that was in place for the executive immediately prior to the date of termination.

In addition, all unvested options, restricted stock, restricted stock units, or stock appreciation rights held by the executive 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 executive 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 executive is terminated without "cause" or resigns for "good reason," without regard to whether a change of control has occurred, such executive will be entitled to the following under the Severance Agreement Form (collectively, the "Without Cause/For Good Reason Benefits"), subject to the executive's timely execution and non-revocation of a general release of claims in a form provided by us and the executive's continued compliance with the invention, non-disclosure, and non-competition agreement previously entered into in connection with the commencement of executive'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 executive 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 executive's base salary payable in accordance with our standard payroll practices over the one-year period following termination; and

- if the executive elects to continue receiving group medical and/or dental insurance under COBRA (to the extent the executive 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 remained in full force and effect.

As discussed above, Mr. Milano's and Mr. 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.

Indemnification and Insurance

In connection with the Assignment, Aceragen will continue to indemnify its directors and officers to the maximum extent permitted in accordance with applicable law, Aceragen's charter and bylaws, and any contractual arrangements, for actions taken in connection with the Assignment. The Board is authorized to obtain and maintain insurance as may be necessary, appropriate or advisable to satisfy such indemnification obligations, including seeking an extension in time and coverage of Aceragen's insurance policies currently in effect.

Other than as set forth above, it is not currently anticipated that the Assignment will result in any material benefit to any of Aceragen's executive officers or to directors who participated in the vote to adopt the Assignment.

Certain Material U.S. Federal Income Tax Consequences

The following is a summary of certain material United States federal income tax consequences of the Assignment that are applicable to stockholders. This discussion is included for general information purposes only and is not intended to be, and is not, legal or tax advice to any particular stockholder. This summary is based on the current provisions of the Internal Revenue Code of 1986, as amended (the "Code"), and other legal authorities, all of which are subject to change, possibly with retroactive effect. No rulings from the Internal Revenue Service (the "IRS") or opinions of counsel have been or will be requested concerning the matters discussed below. The tax consequences set forth in the following discussion are not binding on the IRS or the courts, and no assurance can be given that contrary positions will not be successfully asserted by the IRS or adopted by a court.

This discussion does not describe all of the U.S. federal tax consequences that may be relevant to you in light of your particular circumstances, such as consequences arising under the federal estate and gift tax, the alternative minimum tax or the Medicare tax on net investment income or the consequences to you if you are an accrual-method taxpayer that is required to conform the timing of recognition of items of income to an "applicable financial statement" under Section 451(b) of the Code, or differing tax consequences applicable to you if you are a beneficial owner of Common Stock ("stockholder") subject to special rules. Such special rules and circumstances include stockholders who are dealers in securities, financial institutions, insurance companies, entities classified as partnerships for income tax purposes, tax-exempt organizations, foreign persons, stockholders who acquired their Common Stock through stock option or stock purchase programs or in other compensatory transactions, stockholders who are subject to alternative minimum tax, stockholders who hold their Common Stock as part of an integrated investment, including a straddle, comprising shares of Common Stock and one or more other positions, or stockholders who have entered into a constructive sale of Common Stock under the Code.

For the avoidance of doubt, this summary applies to you only if you are a U.S. Holder. You are a U.S. Holder if, for U.S. federal income tax purposes, you are a stockholder that is:

- an individual who is a citizen or resident of the United States;
- a corporation created or organized in or under the laws of the United States, any state thereof or the District of Columbia; or
- an estate or trust the income of which is subject to U.S. federal income taxation regardless of its source.

EACH STOCKHOLDER SHOULD CONSULT THE STOCKHOLDER'S OWN TAX ADVISOR TO DETERMINE THE STOCKHOLDER'S PARTICULAR U.S. FEDERAL INCOME TAX CONSEQUENCES AND OTHER TAX CONSEQUENCES TO THE STOCKHOLDER OF THE ASSIGNMENT, INCLUDING ANY STATE, LOCAL AND FOREIGN TAX LAWS AND THE EFFECT OF ANY CHANGES IN SUCH LAWS.

Worthless Stock. Under U.S. federal income tax law, if stock becomes worthless, the resulting loss is treated as a loss from the sale or exchange of a capital asset on the last day of the tax year during which the stock first becomes worthless. Stock is generally treated as worthless if there is no current liquidating value, no reasonable hope and expectation that the stock will become valuable at some future time and there is an identifiable event establishing the worthlessness of the stock.

Upon completion of the Assignment, Aceragen expects that its shares will be delisted from trading on the Nasdaq Capital Market. If the stockholders do not receive any liquidating distributions, it is likely that the Assignment will be the identifiable event that establishes the worthlessness of the stock of Aceragen, and that each stockholder should recognize a capital loss equal to the stockholder's adjusted tax basis in the stockholder's shares. For purposes of determining a stockholder's holding period in Aceragen's stock, and whether the capital loss is a long-term or a short-term capital loss, a stockholder's holding period should be determined as if the stock were sold on the last day of the stockholder's taxable year (December 31 for individual stockholders).

If certain requirements of Section 1244 of the Code are met, an individual or partnership stockholder may be able to claim an ordinary loss as a result of the Assignment and stock worthlessness rather than a capital loss with respect to such stockholder's Aceragen Common Stock. Among these requirements are that such Aceragen stock was purchased by the stockholder for cash or other property before Aceragen received aggregate contributions of money or other property for its stock, as a contribution to capital and as paid-in surplus, of \$1,000,000. Other requirements include, for example, the requirement that Aceragen, during Aceragen's most recent five taxable years, has derived more than fifty percent of its aggregate gross receipts from sources other than royalties, rents, dividends, interest, annuities and sales or exchanges of stock or securities. Amounts treated as an ordinary loss under these rules, if any, are limited to \$100,000 in the case of a husband and wife filing a joint income tax return and \$50,000 in all other cases. Stockholders who believe that these rules may apply to them are strongly urged to consult with their tax advisors regarding the potential applicability of Section 1244 of the Code to a loss resulting from the Assignment and stock worthlessness.

Potential Subsequent Liquidation. If there is a subsequent liquidating distribution in the same tax year as the Assignment, amounts received by stockholders in complete liquidation of Aceragen should be treated as payments made in exchange for their Common Stock. In general, each Aceragen stockholder should recognize capital gain or loss equal to the difference between the amount of consideration received by such Aceragen stockholder and the Aceragen stockholder's adjusted tax basis in the shares of Aceragen stock surrendered.

If a liquidating distribution is received in a year subsequent to the Assignment and if a stockholder has treated the Assignment and other events as establishing worthlessness, then the stockholder should consider filing an amended return to undo the effect of the worthless stock loss and report a gain or loss solely with respect to the subsequent year's liquidating distribution.

Gain or loss should be determined separately for each block of shares, with a "block" consisting of shares acquired at the same cost in a single transaction. That gain or loss will be long-term capital gain or

loss, provided the shares are held for investment and the stockholder's holding period for such shares is more than one year. For non-corporate Aceragen stockholders, long-term capital gains are generally subject to a maximum federal income tax rate of 20% and short-term capital gains are subject to tax at ordinary income tax rates. Capital losses not offset by capital gains may be deducted against a non-corporate stockholder's ordinary income only up to a maximum annual amount of \$3,000. A non-corporate stockholder may not carry back capital losses, but such losses may be carried forward to subsequent tax years. All net capital gains for a corporate stockholder are subject to tax at regular corporate tax rates. Although a corporate stockholder can generally deduct capital losses only to the extent of capital gains, any unused capital losses of a corporate stockholder may generally be carried back three years and forward five years.

Backup Withholding if there are Liquidating Distributions. Federal income tax laws require that, to avoid backup withholding with respect to "reportable payments" (currently at a rate of 24%), each Company stockholder must (a) provide Aceragen with such stockholder's correct taxpayer identification number ("TIN") on Form W-9 and certify as to no loss of exemption from backup withholding, or (b) establish a basis for exemption from backup withholding on an appropriate Form W-8 (including a Form W-8BEN, W-8ECI, W-8EXP and W-8IMY) or Form W-9, as applicable. Exempt stockholders (including, among others, all corporations and certain foreign individuals) are not subject to backup withholding and reporting requirements. Backup withholding is not an additional tax. The amount of any backup withholding from a payment to you will be allowed as a credit against your U.S. federal income tax liability, if any, and may entitle you to a refund, provided that the required information is timely furnished to the IRS. Reportable payments, if any, made to stockholders pursuant to a liquidation of Aceragen will be reported by Aceragen to the IRS to the extent required by law.

Recommendation of the Board

On July 10, 2023, the Board determined that the Assignment and the other transactions contemplated thereby are advisable and in the best interests of Aceragen and its stockholders, approved submitting the Assignment Proposal to the Company's stockholders and recommended the approval thereof.

The Board unanimously recommends that you vote "FOR" the Assignment Proposal.

**PROPOSAL 2: APPROVAL OF ADJOURNMENT OF SPECIAL MEETING
TO SOLICIT ADDITIONAL PROXIES**

General

If necessary, including if at the Special Meeting the number of shares voting in favor of the approval of the Assignment Proposal is insufficient to approve such proposal under Delaware law, Aceragen intends to move to adjourn the Special Meeting in order to enable the Board to solicit additional proxies in respect of the approval of the Assignment Proposal. In that event, Aceragen will ask Aceragen's stockholders to vote only upon the Adjournment Proposal during such portion of the Special Meeting.

In the Adjournment Proposal, Aceragen is asking you to authorize the holder of any proxy solicited by the Board to vote in favor of granting discretionary authority to the proxy holders, and each of them individually, to adjourn the Special Meeting, from time to time, to a later date or dates, for the purpose of soliciting additional proxies. If the stockholders approve the Adjournment Proposal, Aceragen could adjourn the Special Meeting and use the additional time to solicit additional proxies, including the solicitation of proxies from stockholders that have previously voted.

Recommendation of the Board

The Board believes that if the number of shares voting in favor of the Assignment Proposal is insufficient to approve such proposal, it is in the best interests of Aceragen to enable Aceragen to continue to seek to obtain a sufficient number of additional votes to bring about the approval of the Assignment Proposal.

The Board unanimously recommends that you vote "FOR" the Adjournment Proposal.

IMPORTANT INFORMATION CONCERNING ACERAGEN, INC.**Description of Business***Overview*

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, 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., a private company, and its subsidiaries prior to the consummation of the Aceragen Acquisition.

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.

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.

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 was to develop and optimize commercial value of ACG-701 and ACG-801 for appropriate patients. Accordingly, we sought to develop 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.

Cost-Reduction Plan Implementation and Reduction in Workforce

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 furloughed 12 employees, representing approximately 46% of our workforce at that time. Additionally, certain of our employees and executive officers agreed to 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.

On April 28, 2023, our Board approved a reduction in workforce in which approximately 80% of the Company’s employees, including our Chief Financial Officer, were terminated effective immediately in an effort to reduce operating costs. This reduction in workforce required the Company to cease development of ACG-701 (patented formulation of sodium fusidate) for Cystic Fibrosis Pulmonary Exacerbations and ACG-801 (recombinant human acid ceramidase (rhAC)) for Farber Disease.

Clinical Development

In light of the cost reduction plan implementation and reduction in workforce discussed above, post April 28, 2023, we continued to develop only ACG-701 to treat melioidosis.

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.

Description of Property

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 that expired on March 31, 2023.

Financial Statements

Our consolidated financial statements as of and for the years ended December 31, 2022 and 2021 and the notes thereto are included as *Annex A* to this proxy statement. Our condensed consolidated financial statements as of and for the three months ended March 31, 2023 and 2022, and the notes thereto, are included as *Annex B* to this proxy statement.

Management's Discussion and Analysis of Financial Condition and Results of Operations

Management's Discussion and Analysis of Financial Condition and Results of Operations of Aceragen ("MD&A") for the years ended December 31, 2022 and 2021 and the periods ended March 31, 2023 and March 31, 2022 is attached to this proxy statement as *Annex C*. The MD&A should be read in conjunction with the audited consolidated financial statements and notes for December 31, 2022 and 2021, attached as *Annex A*, and the unaudited condensed consolidated financial statements and accompanying notes for the three months ended March 31, 2023 and 2022, attached as *Annex B*. In addition to historical information, the MD&A contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Exchange Act, which are intended to be covered by the safe harbors created thereby. See "*Cautionary Information Regarding Forward-Looking Statements.*"

Quantitative and Qualitative Disclosures about Market Risk

As of December 31, 2022, all material assets and liabilities were 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 consolidated 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

Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

There were no changes in or disagreements with accountants on matters of accounting principles or practices or financial disclosures for the periods covered by the Form 10-K for the fiscal year ended December 31, 2022 and the Form 10-Q for the fiscal quarter ended March 31, 2023.

SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT

The following table sets forth certain information with respect to the beneficial ownership of Aceragen's Common Stock as of July 19, 2023 for:

- each of Aceragen's directors;
- each of Aceragen's named executive officers;
- all of Aceragen's current directors and executive officers as a group; and
- each person or group who beneficially owned more than 5% of Aceragen's Common Stock.

Aceragen has determined beneficial ownership in accordance with the rules of the SEC and the information is not necessarily indicative of beneficial ownership for any other purpose. Unless otherwise indicated below, to Aceragen's knowledge, the persons and entities named in the table have sole voting and sole investment power with respect to all shares that they beneficially owned, subject to community property laws where applicable.

Aceragen has based the calculation of the percentage of beneficial ownership on 8,420,276 shares of Aceragen's Common Stock outstanding as of July 19, 2023. Aceragen has deemed shares of Aceragen's Common Stock subject to stock options that are currently exercisable or exercisable within 60 days of July 19, 2023, to be outstanding and to be beneficially owned by the person holding the stock option for the purpose of computing the percentage ownership of that person. Aceragen did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person.

Name of Beneficial Owner ⁽¹⁾	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned
5% Stockholders:		
NovaQuest Co-Investment Fund XV, L.P. ⁽²⁾ 4208 Six Forks Road, Suite 920 Raleigh, North Carolina 27609	909,326	9.75%
Pillar Investment Entities ⁽³⁾ c/o Stuarts Corporate Services Ltd. Kensington House, 69 Dr. Roy's Drive Georgetown, Grand Cayman KY1-1104 Cayman Islands	571,588	6.72%
Atul Chopra ⁽⁴⁾ 4002 White Oak Trail Beachwood, Ohio 44122	1,205,158	14.31%
Daniel Salain ⁽⁵⁾	1,469,482	17.45%
Andrew Jordan ⁽⁶⁾	451,890	5.37%
Executive Officers and Directors:		
John Taylor	1,469,482	17.45%
John J. Kirby ⁽⁷⁾	40,586	*
Bryant D. Lim ⁽⁸⁾	18,217	*
Vincent J. Milano ⁽⁹⁾	69,502	*
Daniel Soland ⁽¹⁰⁾	20,799	*
Cristina Csimma ⁽¹¹⁾	5,762	*
Michael R. Dougherty ⁽¹²⁾	27,829	*
Maxine Gowen ⁽¹³⁾	5,966	*
Ronald Wooten ⁽¹⁴⁾	909,326	9.75%
All current directors and executive officers as a group (6 persons) ⁽¹⁵⁾	2,487,867	26.45%

* Represents beneficial ownership of less than one percent (1%) of the outstanding shares of Aceragen's Common Stock.

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

- (2) Consists of 909,326 warrants to purchase Common Stock of the Company, with an exercise price of \$7.82, beneficially owned by NovaQuest Co-Investment Fund XV, L.P. (“NovaQuest”), and based on the Schedule 13D filed with the SEC on January 18, 2023 by NovaQuest, which reported sole voting power with respect to zero shares, shared voting power with respect to 909,326 shares underlying warrants, sole dispositive power with respect to zero shares, and shared dispositive power with respect to 909,326 shares underlying warrants as of January 17, 2023.
- (3) Based on Amendment No. 16 to the Schedule 13D filed with the SEC on July 17, 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 571,588 shares, sole dispositive power with respect to zero shares, and shared dispositive power with respect to 571,588 shares as of July 13, 2023.
- The Reporting Persons expressly disclaim status as a “group” for purposes of Amendment No. 16 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, and as supplemented by the Form 4 filed with the SEC on May 18, 2023 by Atul Chopra, MD, PhD, as trustee of the Chopra Revocable Trust, which reported 1,205,158 shares beneficially owned by Dr. Chopra as of May 17, 2023.
- (5) Based on the Form 4 filed with the SEC on January 19, 2023 by Daniel Salain, which reported 1,469,482 shares beneficially owned by Mr. Salain as of January 17, 2023.
- (6) Based on the Form 4 filed on January 19, 2023 by Andrew Jordan, which reported 451,890 shares beneficially owned by Mr. Jordan as of January 17, 2023.
- (7) Includes 21,536 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023.
- (8) Based on the Form 4 filed with the SEC on January 10, 2023 by Bryant D. Lim, which reported 309,695 shares beneficially owned by Mr. Lim as of January 6, 2023, adjusted to reflect a 1-for-17 reverse stock split, which became effective on January 17, 2023 at 4:59 p.m. Eastern Time.
- (9) Includes 57,775 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023.
- (10) Includes 16,162 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023.
- (11) Consists of 5,762 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023.
- (12) Includes 5,762 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023.
- (13) Includes 5,915 shares of Common Stock subject to outstanding stock options that are exercisable within 60 days after July 19, 2023.
- (14) Consists of 909,326 warrants to purchase Common Stock of the Company, with an exercise price of \$7.82, 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.

- (15) Includes 909,326 warrants to purchase Common Stock of the Company and 75,214 shares of Common Stock subject to outstanding stock options beneficially owned by our current directors and executive officers as a group that are exercisable within 60 days after July 19, 2023.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We file annual and quarterly reports and other reports and information with the SEC. The SEC maintains an Internet website that contains reports, proxy and information statements, and other information regarding issuers, including us, that file electronically with the SEC. The public can obtain any documents that we file electronically with the SEC at <http://www.sec.gov>. We will provide without charge to you, upon written or oral request, a copy of the reports and other information filed with the SEC.

Any requests for copies of information, reports or other filings with the SEC should be directed to Aceragen, Inc., 505 Eagleview Blvd., Suite 212, Exton, Pennsylvania 19341, Attention: Corporate Secretary. In order to receive timely delivery of the documents in advance of the Special Meeting, you must make your request for information no later than August 7, 2023.

HOUSEHOLDING

Some banks, brokers, and other nominee record holders may be participating in the practice of “house holding” proxy statements and annual reports. This means that only one copy of our proxy statement, may have been sent to multiple stockholders sharing the same household. We will promptly deliver a separate copy of the proxy statement to you upon written request to Aceragen, Inc., 505 Eagleview Blvd., Suite 212, Exton, Pennsylvania 19341, Attention: Corporate Secretary. If you want to receive separate copies of the proxy statement or annual reports to stockholders in the future, or if you are receiving multiple copies and would like to receive only one copy per household, you should contact your bank, broker, or other nominee record holder, or you may contact us at the above address.

OTHER MATTERS

Our Board does not know of any other matters to be brought before the Special Meeting. If any other matters not mentioned in this proxy statement are properly brought before the Special Meeting, the individuals named in the enclosed proxy intend to use their discretionary voting authority under the proxy to vote the proxy in accordance with their best judgment on those matters.

EXHIBIT A

ACERAGEN, INC.

STOCKHOLDER RESOLUTIONS

WHEREAS, the Board of Directors (the “Board”) of Aceragen, Inc., a Delaware corporation (“Aceragen”), has determined that effecting the transfer of all or substantially all of Aceragen’s assets through an assignment for the benefit of creditors (the “Assignment”) is advisable and in the best interests of Aceragen and Aceragen’s stockholders;

WHEREAS, the Board has authorized and approved the Assignment and has recommended that Aceragen’s stockholders authorize and approve the Assignment;

WHEREAS, Section 271 of the Delaware General Corporation Law provides that the affirmative vote of a majority of the voting power of the outstanding shares of capital stock of Aceragen is required to approve the Assignment; and

WHEREAS, such holders of a majority of the voting power of the outstanding shares of capital stock of Aceragen desire to authorize and approve the Assignment pursuant to the resolutions set forth below.

NOW THEREFORE, BE IT RESOLVED, that the Assignment is hereby approved and authorized in all respects; and be it further

RESOLVED, that the appropriate officers of Aceragen are hereby authorized to take or cause to be taken all such further actions as the Board deems necessary or advisable to consummate and perform on behalf of Aceragen the transactions contemplated by the Assignment, including the execution and delivery of such agreements, certificates, documents and instruments as shall be necessary or desirable to effect the Assignment and to carry out the purposes and intent of the foregoing resolutions, and the paying of all necessary fees and expenses in connection with the Assignment.

ACERAGEN, INC. AND SUBSIDIARIES
INDEX TO CONSOLIDATED FINANCIAL STATEMENTS
December 31, 2022 and 2021

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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.

<i>Description of the Matter</i>	<p>Valuation of intangible assets acquired in a business combination</p> <p>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.</p> <p>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.</p>
<i>How We Addressed the Matter in Our Audit</i>	<p>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.</p>
<i>Description of the Matter</i>	<p>Valuation of Series X preferred stock liability</p> <p>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.</p> <p>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</p>

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	<u>\$ 101,107</u>	<u>\$ 34,864</u>
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	<u>11,826</u>	<u>29,453</u>
Total liabilities, convertible redeemable preferred stock, and stockholders' equity	<u>\$ 101,107</u>	<u>\$ 34,864</u>

* 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		Series Z Preferred		Series B Preferred		Common Stock		Additional Paid-In Capital*	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' Equity (Deficit)
	Number of Shares	\$0.01 Par Value	Number of Shares	\$0.01 Par Value	Number of Shares	\$0.01 Par Value	Number of Shares*	\$0.001 Par Value*				
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, Arrebus, Inc., a Delaware Corporation ("Arrebus"), 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	<u>\$65,616</u>

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
	<u>\$90,671</u>
Liabilities assumed:	
Accounts Payable and accrued expenses	\$ 7,886
Acquisition Obligation (Note 7)	7,546
Operating lease liabilities	22
Deferred tax liabilities	9,601
	<u>\$25,055</u>
Net assets acquired	<u>\$65,616</u>

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)	<u>\$71,600</u>	<u>\$ —</u>	<u>\$71,600</u>

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.

(In thousands)	December 31,	
	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.

(In thousands)	December 31, 2022			
	Total	Level 1	Level 2	Level 3
Assets				
Cash	\$ 3,342	\$ 3,342	\$ —	\$ —
Cash equivalents – money market funds	8,702	8,702	—	—
Total assets	<u>\$12,044</u>	<u>\$12,044</u>	<u>\$ —</u>	<u>\$ —</u>
Liabilities				
Warrant liability	\$ 2,819	\$ —	\$ —	\$ 2,819
Series X Preferred Stock liability	34,300	—	—	34,300
Total liabilities	<u>\$37,119</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$37,119</u>

(In thousands)	December 31, 2021			
	Total	Level 1	Level 2	Level 3
Assets				
Cash	\$ 250	\$ 250	\$ —	\$ —
Cash equivalents – money market funds	32,295	32,295	—	—
Total assets	<u>\$32,545</u>	<u>\$32,545</u>	<u>\$ —</u>	<u>\$ —</u>

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	<u>\$2,819</u>	<u>\$34,300</u>

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	<u>\$ 7</u>	<u>\$ 22</u>

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	<u>\$9,911</u>	<u>\$4,088</u>

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 a 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:

Description	Number of Warrants		Weighted-Average Exercise Price	Expiration Date
	December 31, 2022	December 31, 2021		
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
	<u>503,069</u>	<u>513,658</u>		
Liability-classified warrants:				
Assumed Legacy Aceragen Series Z Warrants ⁽¹⁾	14,215	—	\$460.00	Mar 2031
	<u>14,215</u>	<u>—</u>		
Total outstanding	<u>517,284</u>	<u>513,658</u>		

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	<u>503,069</u>	<u>14,215</u>	<u>517,284</u>	<u>\$17.58</u>

(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 Licensee 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.

(\$ in thousands, except per share data)	Common Stock Options			
	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⁾	<u>406,174</u>	<u>\$ 83.00</u>	<u>6.1</u>	<u>\$102</u>
Exercisable at December 31, 2022	<u>239,866</u>	<u>\$132.26</u>	<u>4.1</u>	<u>\$ 54</u>

(\$ in thousands, except per share data)	Preferred Stock Options			
	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⁾	<u>17,522</u>	<u>\$397.02</u>	<u>9.1</u>	<u>\$1,073</u>
Exercisable at December 31, 2022	<u>5,229</u>	<u>\$317.43</u>	<u>8.9</u>	<u>\$ 568</u>

(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	<u>19,096</u>	<u>\$10.73</u>	<u>29,814</u>	<u>\$26.14</u>

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	<u>15,432</u>	<u>2,756</u>

(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	<u>\$626</u>
Less: imputed interest	<u>(66)</u>
Total present value of lease liabilities	<u>\$560</u>

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)	<u>\$ (3,283)</u>	<u>\$ —</u>

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	<u>(21.3)%</u>	<u>0.0%</u>

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	<u>\$ (6,318)</u>	<u>\$ —</u>

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:

(\$ in thousands except share and per share data)	Year Ended December 31,	
	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 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 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.

Consent of Independent Registered Public Accounting Firm

We consent to the use of our report dated April 13, 2023, included in the Proxy Statement of Aceragen, Inc. for the year ended December 31, 2022, with respect to the consolidated financial statements of Aceragen, Inc., included in this Proxy Statement.

/s/ Ernst & Young LLP

Philadelphia, PA
July 21, 2023

ACERAGEN, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(UNAUDITED)

(In thousands, except share and per share amounts)	March 31, 2023	December 31, 2022*
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 2,118	\$ 12,044
Accounts receivable	2,203	4,208
Prepaid expenses and other current assets	1,315	1,611
Total current assets	5,636	17,863
Property and equipment, net	4	7
Intangible assets	67,000	71,600
Goodwill	4,629	11,100
Operating lease right-of-use assets	471	537
Other assets	752	—
Total assets	<u>\$ 78,492</u>	<u>\$ 101,107</u>
LIABILITIES, CONVERTIBLE REDEEMABLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 3,409	\$ 5,200
Accrued expenses	7,198	9,911
Acquisition obligation, net	6,300	6,078
Operating lease liability	223	234
Other current liability	733	—
Total current liabilities	17,863	21,423
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 6)	34,800	34,300
Operating lease liability, net of current portion	271	326
Deferred tax liability	3,010	3,283
Other liabilities	—	22
Total liabilities	55,944	62,173
Commitments and contingencies		
Preferred stock, \$0.01 par value, Authorized – 5,000,000 shares:		
Series Z convertible redeemable preferred stock (Note 8); Designated – 150,000 shares, Issued and outstanding – 0 and 77,900 shares at March 31, 2023 and December 31, 2022, respectively	—	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 – 0 and 62,355 shares at March 31, 2023 and December 31, 2022, respectively	—	1
Common stock, \$0.001 par value, Authorized – 140,000,000 shares; Issued and outstanding – 8,346,949 and 3,653,685 shares at March 31, 2023 and December 31, 2022, respectively	8	4
Additional paid-in capital	803,251	770,663
Accumulated deficit	(780,688)	(758,821)
Accumulated other comprehensive income (loss)	(23)	(21)
Total stockholders' equity	22,548	11,826
Total liabilities, convertible redeemable preferred stock, and stockholders' equity	<u>\$ 78,492</u>	<u>\$ 101,107</u>

* The condensed consolidated balance sheet at December 31, 2022 has been derived from the audited consolidated financial statements at that date.

The accompanying notes are an integral part of these condensed consolidated financial statements.

ACERAGEN, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND
COMPREHENSIVE LOSS
(UNAUDITED)

(In thousands, except share and per share amounts)	Three Months Ended March 31,	
	2023	2022
Government contracts revenue	\$ 2,470	\$ —
Operating expenses:		
Research and development	4,993	1,784
General and administrative	4,920	2,398
Goodwill and intangible assets impairment	11,071	—
Restructuring and other costs	1,300	—
Acquisition-related costs	794	—
Total operating expenses	23,078	4,182
Loss from operations	(20,608)	(4,182)
Other income (expense):		
Interest income (expense), net	(185)	3
Warrant revaluation loss	(874)	—
Series X preferred stock liability loss	(500)	—
Foreign currency exchange and other gain (loss), net	27	1
Loss before income tax benefit	\$ (22,140)	\$ (4,178)
Income tax benefit	273	—
Net loss	\$ (21,867)	\$ (4,178)
Net loss per share applicable to common stockholders – Basic and Diluted	\$ (2.94)	\$ (1.34)
Weighted-average number of common shares used in computing net loss per share applicable to common stockholders – Basic and Diluted	7,448,799	3,111,354
Comprehensive loss:		
Net loss	\$ (21,867)	\$ (4,178)
Other comprehensive income (loss), net of tax:		
Foreign currency translation	(2)	—
Total other comprehensive (loss) income, net of tax	(2)	—
Total comprehensive loss	\$ (21,869)	\$ (4,178)

The accompanying notes are an integral part of these condensed consolidated financial statements.

ACERAGEN, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(UNAUDITED)

(In thousands)	Three Months Ended March 31,	
	2023	2022
Cash Flows from Operating Activities:		
Net loss	\$(21,867)	\$ (4,178)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	1,231	545
Foreign currency translation	(9)	—
Goodwill and intangible assets impairment	11,071	—
Warrant revaluation loss	874	—
Series X preferred stock liability loss	500	—
Issuance of common stock for services rendered	15	22
Accretion of discounts on acquisition obligation	222	—
Depreciation and amortization expense	3	4
Deferred tax benefit	(273)	—
Changes in operating assets and liabilities		
Accounts receivable	2,005	—
Prepaid expenses and other assets	449	124
Accounts payable, accrued expenses, and other liabilities	(3,826)	(1,086)
Other	(247)	1
Net cash used in operating activities	<u>(9,852)</u>	<u>(4,568)</u>
Cash Flows from Financing Activities:		
Proceeds from employee stock purchases	—	16
Proceeds from exercise of stock options	88	—
Payments on seller-financed purchases	(170)	—
Other	8	—
Net cash (used in) provided by financing activities	<u>(74)</u>	<u>16</u>
Net decrease in cash and cash equivalents	(9,926)	(4,552)
Cash and cash equivalent, beginning of period	12,044	32,545
Cash and cash equivalents, end of period	<u>\$ 2,118</u>	<u>\$27,993</u>
Supplemental disclosure of cash flow information:		
Cash paid for interest	<u>\$ —</u>	<u>\$ 5</u>
Supplemental disclosure of non-cash financing and investing activities:		
Offering costs in accrued expenses	<u>\$ —</u>	<u>\$ 15</u>
Non-cash seller-financed purchases	<u>\$ 903</u>	<u>\$ —</u>

The accompanying notes are an integral part of these condensed consolidated financial statements.

ACERAGEN, INC.

CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY
(UNAUDITED)

(In thousands, except share and per share amounts)	Three Months Ended March 31, 2023									
	Series Z Preferred		Series B Preferred		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Number of Shares	\$0.01 Par Value	Number of Shares	\$0.01 Par Value	Number of Shares	\$0.001 Par Value				
Balance, December 31, 2022	77,900	\$ 27,108	62,355	\$ 1	3,653,685	\$ 4	\$770,663	\$(758,821)	\$ (21)	\$ 11,826
Vesting of restricted stock awards	47	—	—	—	6,386	—	—	—	—	—
Issuance of common stock under equity incentive plan upon vesting of restricted stock units and exercise of options	—	—	—	—	55,891	—	88	—	—	88
Issuance of common stock under equity incentive plan for retention payments	—	—	—	—	43,900	—	456	—	—	456
Issuance of common stock for services rendered	—	—	—	—	1,972	—	15	—	—	15
Conversion of Series Z Preferred Stock to common stock, net of unvested portion, and redemption of Series B Preferred Stock	(77,947)	(27,108)	(62,355)	(1)	4,585,115	4	27,105	—	—	27,108
Reclassification of warrant liability upon conversion of Series Z Preferred Stock warrants to common stock warrants	—	—	—	—	—	—	3,693	—	—	3,693
Stock-based compensation	—	—	—	—	—	—	1,231	—	—	1,231
Foreign currency translation	—	—	—	—	—	—	—	—	(2)	(2)
Net loss	—	—	—	—	—	—	—	(21,867)	—	(21,867)
Balance, March 31, 2023	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>8,346,949</u>	<u>\$ 8</u>	<u>\$803,251</u>	<u>\$(780,688)</u>	<u>\$ (23)</u>	<u>\$ 22,548</u>

(In thousands, except share and per share amounts)	Three Months Ended March 31, 2022									
	Series Z Preferred		Series B Preferred		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Number of Shares	\$0.01 Par Value	Number of Shares	\$0.01 Par Value	Number of Shares	\$0.001 Par Value				
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 under employee stock purchase plan	—	—	—	—	2,482	—	16	—	—	16
Issuance of common stock under equity incentive plan upon vesting of restricted stock units	—	—	—	—	1,600	—	—	—	—	—
Issuance of common stock for services rendered	—	—	—	—	2,180	—	22	—	—	22
Stock-based compensation	—	—	—	—	—	—	545	—	—	545
Net loss	—	—	—	—	—	—	—	(4,178)	—	(4,178)
Balance, March 31, 2022	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>3,113,209</u>	<u>\$ 3</u>	<u>\$765,479</u>	<u>\$(739,639)</u>	<u>\$ —</u>	<u>\$25,843</u>

The accompanying notes are an integral part of these condensed consolidated financial statements.

ACERAGEN, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)
March 31, 2023**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 originally 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.

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.

Presently, the Board of Directors of the Company (the “Board”) is evaluating several options, including an assignment for the benefit of creditors, wind-down, or liquidation and dissolution.

Liquidity, Financial Condition and Consideration as a Going Concern

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 condensed 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 \$780.7 million as of March 31, 2023. The Company’s cash and cash equivalents balance of \$2.1 million as of March 31, 2023 is not sufficient to fund its operations for the one-year period after the date that the condensed consolidated financial statements were issued. These factors raise substantial doubt about the Company’s ability to continue as a going concern. The accompanying condensed 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 condensed 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.

Funding for Future Operations

Management has evaluated different strategies to obtain the required funding for future operations. Despite its efforts, the Company has been unsuccessful in securing additional capital to fund operations, restructure its outstanding debt and otherwise satisfy creditor obligations. As further discussed in Note 15, on April 28, 2023, the Company’s Board of Directors (the “Board”) approved a reduction in workforce in which approximately 80% of the Company’s employees were terminated, effective immediately. This reduction in workforce required the Company to cease development of ACG-701 (patented formulation of sodium fusidate) for Cystic Fibrosis Pulmonary Exacerbations and ACG-801 (recombinant human acid ceramidase (rhAC)) for Farber Disease. Currently, the Company continues to develop only ACG-701 for Melioidosis subsequent to the reduction in workforce.

As a result, management and the Board are evaluating an assignment for benefit of creditors and other strategic alternatives that may be available, including bankruptcy and liquidation of the Company.

NovaQuest

Pursuant to the Stock and Warrant Purchase Agreement, dated as of March 24, 2021, by and between Legacy Aceragen and NovaQuest Co-Investment Fund XV, L.P. (“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.

Subsequent to the Company’s cessation of development efforts around ACG-801, NovaQuest has alleged that the Company is in breach of the Purchase Agreement and is demanding the return of \$35.0 million, plus 12% interest compounded annually and accruing from March 24, 2021 until paid. The Company is currently negotiating with NovaQuest with respect to the Purchase Agreement, however, there is no assurance that such negotiations will be successful.

Reverse Stock Split

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 condensed 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 unaudited condensed consolidated financial statements included herein have been prepared by the Company in accordance with U.S. generally accepted accounting principles (“GAAP”) for interim financial information and pursuant to the rules and regulations of the SEC. Accordingly, certain information and footnote disclosures normally included in consolidated financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. In the opinion of management, all adjustments, consisting of normal recurring adjustments, and disclosures considered necessary for a fair presentation of interim period results have been included. Interim results for the three months ended March 31, 2023 are not necessarily indicative of results that may be expected for the year ending December 31, 2023. For further information, refer to the consolidated financial statements and footnotes thereto included in the Company’s 2022 Form 10-K filed with the SEC.

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 March 31, 2023 and 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. 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 March 31, 2023, 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.

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 March 31, 2023 and December 31, 2022 consisted of cash and money market funds.

Accounts Receivable

The U.S. Government accounted for all of the Company's accounts receivable as of March 31, 2023. 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 March 31, 2023 is \$0.3 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.

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 the Company's 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. During the three months ended March 31, 2023, management identified an indicator of impairment related to the decrease in the Company's market capitalization. As a result, the Company performed an interim impairment test that resulted in the recognition of an impairment loss of \$4.6 million related to IPR&D (see Note 3 and 4 for further discussion).

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 condensed 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 the carrying amount of the reporting unit is greater than its fair value. During the three months ended March 31, 2023, management identified an indicator of impairment related to the decrease in the Company's market capitalization. As a result, the Company performed an interim impairment test that resulted in the recognition of an impairment loss of \$6.5 million related to goodwill (see Note 3 and 4 for further discussion).

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 condensed 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 condensed 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 March 31, 2023 and December 31, 2022, the Company's operating lease ROU assets and corresponding short-term and long-term lease liabilities primarily relate to its existing Exton, PA 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.

Other Current Liability

In January 2023, the Company entered into a short-term financing arrangement with a third-party vendor to finance insurance premiums. The aggregate amount financed under this agreement was \$0.9 million. As of March 31, 2023, the balance of \$0.7 million, which is included in "Other current liability" in the Company's condensed consolidated balance sheets, is scheduled to be paid in monthly installments through August 2023. Total interest to be incurred under the financing arrangement is not material.

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 condensed 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. During the three months ended March 31, 2023, all of the Company's liability-classified Series Z Warrants were converted into warrants to purchase common stock and, accordingly, the Series Z Warrant liability was reclassified to stockholders' equity.

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.

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 condensed consolidated statements of operations until it is settled. See Note 6 to these condensed consolidated financial statements for further discussion of the Series X Preferred Stock Liability.

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.

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 10, “Government Contracts Revenue,” of the notes to these condensed consolidated financial statements for discussion of the Company’s cost reimbursement contracts.

Customer Concentration Risk

The U.S. Government accounted for all of the Company’s revenues for the three months ended March 31, 2023.

Goodwill and Intangible Assets Impairment

The Company incurred total impairment losses of \$11.1 million during the three months ended March 31, 2023, consisting of impairment losses of its IPR&D and goodwill totaling \$4.6 million and \$6.5 million, respectively, as more fully described above.

Restructuring and Other Costs

In connection with the Aceragen Acquisition, the Company determined to restructure its operations and reduce its workforce which resulted in seven positions being eliminated by December 31, 2022, representing approximately 54% of the Company’s pre-Aceragen Acquisition employees. As a result of the above restructuring initiatives, the Company incurred total restructuring-related charges of \$5.0 million to date, including \$1.3 million during the three months ended March 31, 2023 related to severance payments to two former executives which were contingent on obtaining shareholder approval at the Special Meeting, which occurred in January 2023. Such amounts are payable in stock and are included in accrued expenses as of March 31, 2023.

As of March 31, 2023, of the \$5.0 million total restructuring-related charges incurred, \$3.4 million remain unpaid and are included in accrued expenses in the accompanying condensed consolidated balance sheet. See Note 5.

Acquisition-Related Costs

Acquisition-related costs include direct expenses incurred in connection with the Aceragen Acquisition, as well as integration-related professional fees, employee retention-related benefits, and other incremental costs directly associated to the Aceragen Acquisition. For the three months ended March 31, 2023 acquisition-related costs totaled \$0.8 million.

Income Taxes

In accordance with ASC 270, *Interim Reporting*, and ASC 740, *Income Taxes*, the Company is required at the end of each interim period to determine the best estimate of its annual effective tax rate and then apply that rate in providing for income taxes on a current year-to-date (interim period) basis. For the three months ended March 31, 2023, the Company recorded a \$0.3 million non-cash income tax benefit due to a reduction in deferred tax liabilities associated with the Company's IPR&D assets acquired in connection with the Aceragen Acquisition following the recognition of an impairment loss on such IPR&D assets (see Note 4) and reevaluation of the realizability of the Company's deferred tax assets. No such income tax benefit (or expense) was recorded during the three months ended March 31, 2022. The Company had no uncertain tax positions as of March 31, 2023 and December 31, 2022.

Net 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, and warrants 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 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 consolidated 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 12), (ii) 80,656 shares of Series Z Preferred Stock (inclusive of unvested restricted preferred stock — see Note 12) 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 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 the Company's 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 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. 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 8)	25,085
Series X liability in connection with Aceragen Acquisition (Note 6)	31,900
Stock options, restricted stock and warrants allocated to consideration paid	5,822
Total Consideration paid	<u>\$65,616</u>

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
	<u>\$90,671</u>
Liabilities assumed:	
Accounts Payable and accrued expenses	\$ 7,886
Acquisition Obligation (Note 7)	7,546
Operating lease liabilities	22
Deferred tax liabilities	9,601
	<u>\$25,055</u>
Net assets acquired	<u>\$65,616</u>

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 indefinite-lived tangible assets acquired in connection with the Aceragen Acquisition and their carrying value as of March 31, 2023 (see Note 4 for further discussion):

(In thousands)	Acquisition Date Fair Value	Impairment	Carrying Value as of March 31, 2023
ACG-701 for Cystic Fibrosis	\$50,700	\$(2,600)	\$48,100
ACG-701 for Melioidosis	14,900	—	14,900
ACG-801 for Farber Disease	6,000	(2,000)	4,000
Total in-process research and development costs (IPR&D)	<u>\$71,600</u>	<u>\$(4,600)</u>	<u>\$67,000</u>

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. See Note 4.

Pro Forma Financial Information

The following pro forma financial information reflects the consolidated results of operations of the Company for the three months ended March 31, 2022 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.

(In thousands)	March 31, 2022
Net revenues	\$ 5,688
Net loss	\$(7,354)

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
- 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 three months ended March 31, 2023.

The table below presents the assets and liabilities measured and recorded in the condensed consolidated financial statements at fair value on a recurring basis at March 31, 2023 and December 31, 2022 categorized by the level of inputs used in the valuation of each asset and liability.

(In thousands)	March 31, 2023			
	Total	Level 1	Level 2	Level 3
Assets				
Cash	\$ 500	\$ 500	\$ —	\$ —
Cash equivalents – money market funds	1,618	1,618	—	—
Total assets	<u>\$ 2,118</u>	<u>\$2,118</u>	<u>\$ —</u>	<u>\$ —</u>
Liabilities				
Series X Preferred Stock liability	\$34,800	\$ —	\$ —	\$34,800
Total liabilities	<u>\$34,800</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$34,800</u>

(In thousands)	December 31, 2022			
	Total	Level 1	Level 2	Level 3
Assets				
Cash	\$ 3,342	\$ 3,342	\$ —	\$ —
Cash equivalents – money market funds	8,702	8,702	—	—
Total assets	<u>\$12,044</u>	<u>\$12,044</u>	<u>\$ —</u>	<u>\$ —</u>
Liabilities				
Warrant liability	\$ 2,819	\$ —	\$ —	\$ 2,819
Series X Preferred Stock liability	34,300	—	—	34,300
Total liabilities	<u>\$37,119</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$37,119</u>

The Level 1 assets consist of money market funds, which are actively traded daily.

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, 2022	\$ 2,819	\$34,300
Change in fair value	874	500
Reclassification to stockholders' equity ⁽¹⁾	(3,693)	—
Balance, March 31, 2023	<u>\$ —</u>	<u>\$34,800</u>

- (1) During the three months ended March 31, 2023, the Company's liability-classified warrants, representing warrants exercisable for Series Z Preferred Stock, were reclassified to stockholders' equity upon conversion of such warrants to warrants exercisable for common stock in January 2023.

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.

Assets measured at Fair Value on a Nonrecurring Basis

The Company's non-financial assets, which primarily consist of goodwill and IPR&D assets are not required to be measured at fair value on a recurring basis, and instead are reported at their carrying amount. However, on a periodic basis whenever events or changes in circumstances indicate that their carrying values may not be fully recoverable (and at least annually for goodwill and indefinite-lived intangible assets), non-financial assets are assessed for impairment. If the fair value is determined to be lower than the carrying amount, an impairment charge is recorded to write down the asset to its fair value. As a result of the decrease in the Company's market capitalization during the quarter ended March 31, 2023, the Company determined that a sufficient indicator existed to trigger the performance of an interim impairment analysis for goodwill and IPR&D assets. The IPR&D asset related to ACG-701 for Cystic Fibrosis with a carrying amount of \$50.7 million was written down to a fair value of \$48.1 million (Level 3 fair value measurement) and the IPR&D asset related to ACG-801 for Farber Disease with a carrying amount of \$6.0 million was written down to a fair value of \$4.0 million (Level 3 fair value measurement) during the three months ended March 31, 2023. The fair value associated with the IPR&D asset related to ACG-701 for Melioidosis exceeded its carrying value at March 31, 2023. Goodwill with a carrying value of \$11.1 million was written down to a fair value of \$4.6 million during the three months ended March 31, 2023. The fair value of the Company's one reporting unit for interim goodwill impairment testing was determined using a market approach. See Note 3.

Note 5. Accrued Expenses

At March 31, 2023 and December 31, 2022, accrued expenses consisted of the following:

(\$ in thousands)	March 31, 2023	December 31, 2022
Payroll and related costs	\$ 339	\$1,886
Clinical and nonclinical trial expenses	1,576	2,106
Professional and consulting fees	1,041	1,637
Restructuring and other costs ⁽¹⁾	2,882	2,327
Acquisition-related costs	1,144	1,666
Other	216	289
Total accrued expenses	\$7,198	\$9,911

(1) Includes \$1.3 million of severance due to two former executives payable in stock.

Note 6. 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 condensed consolidated financial statements. Any changes in the fair value of the liability are recognized in the condensed consolidated statement of operations until it is settled.

Note 7. Acquisition Obligation

In connection with the Aceragen Acquisition, the Company assumed an obligation pursuant to the Arrebus Merger Agreement (as defined below), whereby Legacy Aceragen was obligated to make an aggregate future payment of \$7.8 million to the Former Stockholders (as defined below), \$6.3 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 \$7.8 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 Arrebus (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, Arrebus, and their respective affiliates (the "Arrebus Merger Agreement"), in an aggregate amount of \$6.0 million (the "Deferred Payments") until October 24, 2023. The Deferred Payments bear annual interest at 12% 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.

Pursuant to the terms of the Term Sheet, on January 31, 2023, the Company issued 12% convertible unsecured promissory notes (the “Convertible Notes”) in an aggregate amount of approximately \$5.9 million to certain Former Stockholders the Company determined to be accredited investors and 12% unsecured promissory notes (the “Promissory Notes”) in an aggregate amount of approximately \$0.4 million to certain Former Stockholders the determined to be unaccredited investors (collectively, the Convertible Notes and the Promissory Notes, the “January 2023 Notes”).

The January 2023 Notes bear annual interest at 12% beginning April 1, 2023. 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.

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. Upon issuance of the January Notes, the Company recognized the remaining unamortized discount on the Acquisition Obligation, resulting in total non-cash charges for the accretion of discounts on the Acquisition Obligation of \$0.2 million, which is included in “Interest income (expense), net” within the accompanying condensed consolidated statements of operations and comprehensive loss.

Note 8. Redeemable Convertible Preferred Stock

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.

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, all 80,656 outstanding shares of Series Z Preferred Stock were automatically converted into 4,744,467 shares (including unvested restricted stock awards which are excluded from stockholders’ equity) of the Company’s common stock

pursuant to the terms of the Series Z Preferred Stock. Upon conversion, the carrying value of the Series Z Preferred Stock was reclassified to stockholders' equity.

Note 9. Stockholders' Equity

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 and preferred stock warrants as equity instruments or liabilities, depending on the specific terms of the warrant agreement.

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 until the warrants are exercised, reclassified, expire, or otherwise settled.

The following table summarizes outstanding warrants to purchase shares of the Company's common stock and/or preferred stock as of March 31, 2023 and December 31, 2022:

Description	Number of Warrants		Weighted-Average Exercise Price	Expiration Date
	March 31, 2023	December 31, 2022		
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
July 2020 Private Placement first closing warrants	162,601	162,601	\$ 43.86	Jul 2023
Assumed Legacy Aceragen warrants	915,772	79,596	\$ 7.82	Mar 2031
	<u>1,339,245</u>	<u>503,069</u>		
Liability-classified warrants:				
Assumed Legacy Aceragen Series Z Warrants ⁽¹⁾	—	14,215	\$460.00	Mar 2031
	—	<u>14,215</u>		
Total outstanding	<u><u>1,339,245</u></u>	<u><u>517,284</u></u>		

The table below is a summary of the Company's warrant activity for the three months ended March 31, 2023.

	Number of Warrants			Weighted-Average Exercise Price ⁽¹⁾
	Common Warrants	Series Z Warrants	Total	
Outstanding at December 31, 2022	503,069	14,215	517,284	\$17.58
Issued	—	—	—	—
Exercised	—	—	—	—
Expired	—	—	—	—
Conversion	836,176	(14,215)	821,961	7.82
Outstanding at March 31, 2023	<u><u>1,339,245</u></u>	<u><u>—</u></u>	<u><u>1,339,245</u></u>	<u><u>\$17.58</u></u>

(1) During the three months ended March 31, 2023, the Company's Series Z Preferred Stock Warrants were converted into warrants to purchase common stock.

“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 through JMP as its agent. During each of the three months ended March 31, 2023 and 2022, the Company sold no shares of common stock pursuant to the ATM Agreement.

Note 10. Government Contracts Revenue

Government contracts revenue for the three months ended March 31, 2023 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 March 31, 2023, 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 \$18.6 million has been used as of March 31, 2023. Of the \$27.7 million total contractual value remaining as of March 31, 2023, \$27.6 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 three months ended March 31, 2023, the Company recognized government contract revenues of \$2.5 million, of which approximately \$2.4 million related to the DTRA Award. No such revenues were recognized during the three months ended March 31, 2022. As of March 31, 2023, there were no material amounts of remaining performance obligations that are required to be disclosed.

Note 11. Clinical Funding, Collaboration and License Agreements***Scriptr Collaboration and Option Agreement***

In February 2021, the Company entered into a collaboration and option agreement (the “Scriptr Agreement”) with Scriptr Global, Inc. (“Scriptr”), pursuant to which (i) the Company and Scriptr conducted a research collaboration utilizing Scriptr Platform Technology 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 and (b) Friedreich’s Ataxia 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 License Agreement (as defined in the Scriptr Agreement) (the “Scriptr License”) for a given Research Program (as defined in the Scriptr Agreement), to make use of Collaboration Candidates and related intellectual property.

The Company incurred approximately \$0.3 million in research and development expenses under the Scriptr Agreement during the three months ended March 31, 2022. No such costs were incurred during the three months ended March 31, 2023.

In June 2023, the Company and Scriptr mutually agreed to terminate the Scriptr Agreement and all rights and licenses granted pursuant to the Scriptr Agreement, including the option to make effective the License Agreement (as defined in the Scriptr Agreement), were terminated.

Note 12. Stock-Based Compensation***Equity Incentive and Employee Stock Purchase Plans***

As of March 31, 2023, the only equity compensation plans from which the Company may currently issue new awards are the 2022 Equity Plan (defined below) and the 2017 Employee Stock Purchase Plan (as amended to date, the “2017 ESPP”).

2022 Stock Incentive 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 Idera Pharmaceuticals, Inc. 2013 Stock Incentive Plan (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.

As of March 31, 2023, options to purchase a total of 295,000 shares of common stock were outstanding under the 2022 Equity Plan and 1,319,206 remained available for issuance under the 2022 Equity Plan.

2021 Legacy Aceragen 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.

As of March 31, 2023, options to purchase a total of 1,068,004 shares of common stock were outstanding under the Legacy Aceragen Plan.

Other Awards and Inducement Grants

As of March 31, 2023, options to purchase a total of 271,025 and 4,908 shares of common stock were outstanding under the 2013 Plan and the Idera Pharmaceuticals, Inc. 2008 Stock Incentive Plan (the "2008 Plan"), respectively, and 26,447 unvested restricted stock units were outstanding under the 2013 Plan. In addition, as of March 31, 2023, non-statutory stock options to purchase an aggregate of 19,116 shares of common stock were outstanding that were issued outside of approved equity plans 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 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. As of March 31, 2023, 39,048 shares remained available for issuance under the 2017 ESPP, however, future offering periods have been suspended until further notice.

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 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 the 15% discount on shares purchased.

Total stock-based compensation expense attributable to stock-based awards made to employees and directors and employee stock purchases included in operating expenses in the Company's condensed statements of operations for the three months ended March 31, 2023 and 2022 were as follows:

(in thousands)	Three Months Ended March 31,	
	2023	2022
Stock-based compensation:		
Research and development		
Employee Stock Purchase Plan	\$ —	\$ 6
Equity Incentive Plans	500	89
	<u>\$ 500</u>	<u>\$ 95</u>
General and administrative		
Employee Stock Purchase Plan	\$ —	\$ 2
Equity Incentive Plans	731	448
	<u>\$ 731</u>	<u>\$450</u>
Total stock-based compensation expense	<u>\$1,231</u>	<u>\$545</u>

During the three months ended March 31, 2023 and 2022, the weighted average fair market value of stock options granted was \$3.49 and \$7.14, respectively.

The following weighted average assumptions apply to the options to purchase 321,900 and 11,658 shares of common stock granted to employees during the three months ended March 31, 2023 and 2022, respectively:

	2023	2022
Average risk-free interest rate	3.9%	1.3%
Expected dividend yield	—	—
Expected lives (years)	3.1	3.8
Expected volatility	106%	105%
Weighted average exercise price (per share)	\$5.21	\$10.20

All options granted during the three months ended March 31, 2023 and 2022 were granted at exercise prices equal to the fair market value of the Company's common stock on the date of grant.

Stock Option Activity

The following table summarizes stock option activity for the three months ended March 31, 2023:

(\$ in thousands, except per share data)	Common Stock Options			
	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in years)	Aggregate Intrinsic Value
Outstanding at December 31, 2022	406,174	\$83.00	6.1	\$102
Granted	321,900	5.21		
Conversion of preferred stock options to common stock options	994,399	6.91		
Exercised	(3,458)	2.21		
Forfeited	(43,565)	7.94		
Expired	(17,397)	2.21		
Outstanding at March 31, 2023⁽¹⁾	<u>1,658,053</u>	<u>\$25.26</u>	<u>8.2</u>	<u>\$ 20</u>
Exercisable at March 31, 2023	<u>593,192</u>	<u>\$57.65</u>	<u>6.5</u>	<u>\$ 10</u>

(\$ in thousands, except per share data)	Preferred Stock Options			
	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in years)	Aggregate Intrinsic Value
Outstanding at December 31, 2022	17,522	\$397.02	9.1	\$1,073
Exercised	(617)	130.00		
Conversion of preferred stock options to common stock options	(16,905)	406.76		
Outstanding at March 31, 2023⁽¹⁾	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>
Exercisable at March 31, 2023	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>

(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.

As of March 31, 2023, there was \$4.2 million of unrecognized compensation cost related to unvested options, which the Company expects to recognize over a weighted average period of 2.9 years.

Restricted Stock Unit Activity

The following table summarizes restricted stock unit activity for the three months ended March 31, 2023:

	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, 2022	19,096	\$10.73	29,814	\$26.14
Granted	—	—	—	—
Cancelled	(294)	—	(6,030)	—
Vested	(1,601)	—	(14,538)	—
Nonvested shares at March 31, 2023	<u>17,201</u>	<u>\$ 7.54</u>	<u>9,246</u>	<u>\$26.14</u>

As of March 31, 2023, remaining unrecognized compensation expense related to the Company's outstanding RSUs was immaterial.

Restricted Stock Activity

The following table summarizes restricted stock activity for the three months ended March 31, 2023:

	Number of Shares	
	Common Stock	Series Z
Nonvested shares at December 31, 2022	15,432	2,756
Granted	—	—
Conversion of preferred stock RSAs to common stock RSAs	75,471	(1,283)
Cancelled	(7,962)	(1,426)
Vested	(6,386)	(47)
Nonvested shares at March 31, 2023	<u>76,555</u>	<u>—</u>

Note 13. Related Party Transactions**Pillar Investment Entities**

Youssef El Zein, a member of the Company's Board 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 March 31, 2023, the Pillar Investment Entities beneficially owned 985,204 shares of the Company's common stock.

As of March 31, 2023, 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, is a member of the investment committee of NQ POF V GP, Ltd. ("NovaQuest GP"), which is the general partner of NovaQuest.

In connection with the Aceragen Acquisition, NovaQuest was issued five shares of Series X Preferred Stock. 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. Following the Conversion Approval, warrants held by NovaQuest to purchase 14,115 shares of Series Z Preferred Stock were automatically converted into warrants to purchase 830,294 shares of the Company's common stock.

As of March 31, 2023, NovaQuest held five shares of Series X Preferred Stock and warrants to purchase 909,326 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 less than \$0.1 million incurred during the three months ended March 31, 2022, the Company issued 2,420 shares of common stock to a director. No shares were issued in lieu of director board and committee fees incurred during the three months ended March 31, 2023. 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 14. Net Loss per Common Share

During periods the Company realizes net income, it uses the two-class method to compute net income per common share and has securities outstanding (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”). During each of the three months ended March 31, 2023 and 2022, the two-class method was not applicable as the Company incurred a net loss and holders of the redeemable convertible preferred stock have no obligation to fund losses.

The Company also analyzes the potential dilutive effect of stock options, unvested restricted stock and restricted stock units, and warrants 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.

During periods the Company realizes net loss, basic and diluted net loss per common share applicable to common stockholders is calculated by dividing net loss applicable to common stockholders by the weighted-average number of shares of common stock outstanding during the period, without consideration of common stock equivalents. The Company’s potentially dilutive shares, which include outstanding stock option awards, unvested restricted stock and restricted stock units, warrants and convertible preferred stock, are considered to be common stock equivalents and are only included in the calculation of diluted net loss per share when their effect is dilutive.

For the three months ended March 31, 2023 and 2022, diluted net loss per common share applicable to common stockholders was the same as basic net loss per common share applicable to common stockholders as the effects of the Company’s potential common stock equivalents were antidilutive.

Total antidilutive securities excluded from the calculation of diluted net loss per share for the three months ended March 31, 2023 and 2022 were as follows:

	Three Months Ended March 31,	
	2023	2022
Common stock options	1,658,053	283,526
Restricted stock units and restricted stock awards	103,002	32,253
Common stock warrants	1,339,245	513,658
Convertible preferred stock	14	14
Total	3,100,314	829,451

Note 15. Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the condensed consolidated 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.

Cost-reduction Plan Implementation and Reduction in Workforce

On April 28, 2023, the Board approved a reduction in workforce (the “Reduction”) in which approximately 80% of the Company’s employees were terminated, effective immediately, in an effort to reduce operating costs. This follows the previously disclosed approval by the Board, on April 13, 2023, of certain cost-cutting measures, including the furlough of approximately 46% of the Company’s workforce and the deferral of base salaries in amounts that exceed \$200,000, effective as of April 5, 2023.

In connection with the Reduction, the Company incurred aggregate restructuring charges in the second quarter of 2023 of approximately \$4.6 million related to severance payments and other employee-related

costs. The Company may also incur additional costs not currently contemplated due to events that may occur as a result of, or that are associated with, the Reduction.

Nasdaq Delisting Notice and Public Shell Inquiry

On May 24, 2023, the Company received a notice (the “Notice”) from the Nasdaq Listing Qualifications Department (the “Staff”), stating that because the Company had not yet filed its Quarterly Report on Form 10-Q for the three months ended March 31, 2023, the Company was not in compliance with Nasdaq Listing Rule 5250(c)(1). Nasdaq Listing Rule 5250(c)(1) requires listed companies to timely file all required periodic financial reports with the SEC. The Notice had no immediate effect on the listing of the Company’s common stock. Subsequent to receiving the Notice, the Company received an inquiry from Staff noting concerns regarding the Company’s potential to be a public shell company and requesting information to assist the Staff in its analysis in determining whether Nasdaq deems the Company to be a public shell. The Company has responded to the Staff’s inquiry, which remains ongoing.

Collaboration with Scriptr

In June 2023, the Company and Scriptr mutually agreed to terminate the Scriptr Agreement and all rights and licenses granted pursuant to the Scriptr Agreement, including the option to make effective the License Agreement (as defined in the Scriptr Agreement), were terminated. The termination of the Scriptr Agreement is not expected to have a material effect on the Company’s condensed consolidated financial statements.

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

Prior to January 17, 2023, we were known as Idera Pharmaceuticals, Inc. On September 28, 2022, we completed the acquisition (the "Aceragen Acquisition") of Aceragen, Inc. ("Legacy Aceragen"), 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 drug candidates for rare disease indications characterized by small, well-defined patient populations with serious unmet medical needs.

Recent Developments***Cost-reduction Plan Implementation and Reduction in Workforce***

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 commenced the furlough of 12 employees, representing approximately 46% of our workforce. Additionally, certain of our employees and executive officers agreed to 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.

On April 28, 2023, the Board approved a reduction in workforce in which approximately 80% of the Company's employees, including our Chief Financial Officer, were terminated effective immediately in an effort to further reduce operating costs. This reduction in workforce required the Company to cease development of ACG-701 (patented formulation of sodium fusidate) for Cystic Fibrosis Pulmonary Exacerbations and ACG-801 (recombinant human acid ceramidase (rhAC)) for Farber disease. The Company continued to develop only ACG-701 for Melioidosis subsequent to the reduction in workforce.

Pursuant to the Stock and Warrant Purchase Agreement, dated as of March 24, 2021, by and between Legacy Aceragen and NovaQuest Co-Investment Fund XV, L.P. ("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.

Subsequent to the Company's cessation of development efforts around ACG-801, NovaQuest has alleged that the Company is in breach of the Purchase Agreement and is demanding the return of \$35.0 million, plus 12% interest compounded annually and accruing from March 24, 2021 until paid. The Company is currently negotiating with NovaQuest with respect to the Purchase Agreement, however, there is no assurance that such negotiations will be successful.

Results of Operations

Results of Operations — Years ended December 31, 2022 and 2021

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.

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:

(\$ in thousands)	Year Ended December 31,		\$ Change	% Change
	2022	2021		
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	<u>\$(27,818)</u>	<u>\$(27,673)</u>	<u>\$ (145)</u>	<u>1%</u>

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 Script 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.

(\$ in thousands)	Year Ended December 31,		\$ Change	% Change
	2022	2021		
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 in *Annex A*.

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 in *Annex A*, 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 the 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.

Results of Operations — Three Months Ended March 31, 2023 and 2022**Overview**

During the three months ended March 31, 2023, our loss from operations totaled \$20.6 million, a 393% increase compared to a loss from operations of \$4.2 million for the three months ended March 31, 2022.

Prior to the Aceragen Acquisition, all our revenues had been from collaboration and license agreements, although we did not generate any such revenue in 2022 and have received no revenues from the sale of commercial products. Following the Aceragen Acquisition, all of our revenues have been derived from U.S. government contracts. Additionally, during the three months ended March 31, 2022, general and administrative expenses comprised the majority of our total operating expenses as we had terminated our ILLUMINATE development program in December 2021 and were focused on strategic alternatives (i.e., Aceragen Acquisition). During the three months ended March 31, 2023, while research and development expenses increased resulting from the clinical programs acquired in connection with the Aceragen Acquisition, general administrative expenses, goodwill and intangible assets impairment and restructuring costs comprised the majority of our total operating expenses.

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

(\$ in thousands)	Three months ended March 31,		\$ Change	% Change
	2023	2022		
Government contracts revenue	\$ 2,470	\$ —	\$ 2,470	100%
Operating expenses:				
Research and development	4,993	1,784	3,209	180%
General and administrative	4,920	2,398	2,522	105%
Goodwill and intangible assets impairment	11,071	—	11,071	100%
Restructuring and other costs	1,300	—	1,300	100%
Acquisition-related costs	794	—	794	100%
Total operating expenses	\$ 23,078	\$ 4,182	\$ 18,896	452%
Loss from operations	<u>\$(20,608)</u>	<u>\$(4,182)</u>	<u>\$(16,426)</u>	<u>393%</u>

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 three months ended March 31, 2023 totaled \$2.5 million of which approximately \$2.4 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 \$27.7 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 three months ended March 31, 2022.

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 three months ended March 31, 2023, our overall research and development expenses increased by 180%, as compared to 2022, primarily due to external development costs associated with the development of ACG-701 and ACG-801, programs that were acquired in connection with the Aceragen Acquisition in the third quarter of 2022. 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.

(\$ in thousands)	Three months ended March 31,		\$ Change	% Change
	2023	2022		
ACG-701 development expense				
REPRIEVE Study (CF PEx)	\$1,318	\$ —	\$1,318	100%
TERRA Study (Melioidosis)	1,572	—	1,572	100%
Subtotal	\$2,890	\$ —	\$2,890	100%
ACG-801 development expense (Farber disease)	557	—	557	100%
Tilsotolimod (IMO-2125) development expense	—	724	(724)	(100)%
Other drug development expense	1,547	1,060	487	46%
Total research and development expenses	<u>\$4,994</u>	<u>\$1,784</u>	<u>\$3,210</u>	<u>180%</u>

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 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.

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 previously anticipated ADVANCE Study, which has been halted as a result of our financial position. 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. Accordingly, no such program-related costs were incurred during the three months ended 2022. As a result of our financial condition, we have halted further development of ACG-801 and do not expect to continue to incur significant additional development expenses related to ACG-801.

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 discontinued in December 2021. All significant study-related activities concluded during 2022 and we did not incur any expenses during the three months ended March 31, 2023 related to tilsotolimod nor anticipate incurring additional expenses in future periods.

Other Drug Development Expenses

These expenses primarily include internal costs, such as salary and other personnel-related costs and overhead expenses not allocated to a specific development program. The increase in other drug development expenses during the three months ended March 31, 2023, as compared to 2022, was primarily due to additional personnel-related costs resulting from employees acquired in connection with 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 three months ended March 31, 2023 and 2022, general and administrative expenses totaled \$4.9 million and \$2.4 million, respectively. The increase in general and administrative expenses during the three months ended March 31, 2023, as compared to the 2022 period, 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.

Goodwill and Intangible Assets Impairment

The Company incurred total impairment losses of \$11.1 million during the three months ended March 31, 2023, consisting of impairment losses of its IPR&D intangible assets and Goodwill totaling \$4.6 million and \$6.5 million, respectively, as more fully described above in Note 2 to the condensed consolidated financial statements appearing elsewhere in this Form 10-Q.

Restructuring and Other Costs

In connection with the Aceragen Acquisition, the Company determined to restructure its operations and reduce its workforce which resulted in seven positions being eliminated by December 31, 2022, representing approximately 54% of the Company's pre-Aceragen Acquisition employees. As a result of the above restructuring initiatives, the Company incurred restructuring-related charges of \$1.3 million during the three months ended March 31, 2023 related to severance due to two former executives which were contingent on obtaining shareholder approval at the Special Meeting. Such amounts are payable in stock and are included in accrued expenses as of March 31, 2023. No such expenses were incurred during the 2022 period.

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 three months ended March 31, 2023 totaled \$0.8 million. All acquisition-related costs related to the Aceragen Acquisition and primarily consisted of legal and professional fees and employee retention-related benefits directly associated with the Aceragen Acquisition. No such costs were incurred during the 2022 period.

Interest Income (Expense), net

Interest expense, net of interest income (which included non-cash charges for accretion of discounts on the Acquisition Obligation of approximately \$0.2 million), for the three months ended March 31, 2023 totaled \$0.2 million. Interest income, net of interest expense, for the three months ended March 31, 2022 was not material. The increase in interest expense, net during the 2023 period was primarily due to accretion of the discount recorded on the Aceragen Obligation in connection with accounting for the Aceragen Acquisition. Amounts may fluctuate from period to period due to changes in average investment balances (including composition of investments), money market funds classified as cash equivalents, and changes in outstanding debt balances.

Warrant Revaluation Loss

During the three months ended March 31, 2023, we recorded a non-cash warrant revaluation loss of approximately \$0.9 million. We did not record any non-cash warrant revaluation gain or loss during the 2022 period. The non-cash loss for the three months ended March 31, 2023 related to an increase in the fair value of our liability-classified warrants assumed in connection with the Aceragen Acquisition as of immediately prior to the derecognition of the liability upon reclassification of the warrants to stockholders' equity in January 2023. Such increase in fair value from January 1, 2023 through the date of reclassification on January 17, 2023 was primarily driven by an increase in our stock price during the period.

Series X Preferred Stock Liability Loss

During the three months ended March 31, 2023, we recorded a non-cash Series X Preferred Stock liability loss of approximately \$0.5 million 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. No such gain or loss was recorded during the three months ended March 31, 2022. The increase in fair value of our liability-classified Series X Preferred Stock during the three months ended March 31, 2023 was primarily driven by the passage of time and resulting decrease in the expected term.

Foreign Currency Exchange and Other Gain (Loss), net

During each of the three months ended March 31, 2023 and 2022, 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 three months ended March 31, 2023, we recorded a \$0.3 million non-cash income tax benefit related to the reduction in deferred tax liabilities associated with our IPR&D assets acquired in connection with the Aceragen Acquisition following the recognition of an impairment loss on such IPR&D assets and revaluation of the realizability of our deferred tax assets.

There was no income tax benefit or expense recorded during the three months ended March 31, 2022.

Net Loss Applicable to Common Stockholders

As a result of the factors discussed above, our basic and diluted net loss applicable to common stockholders for the three months ended March 31, 2023 was \$21.9 million, or \$2.94 per basic and diluted share, as compared to net loss of \$4.2 million for the three months ended March 31, 2022, or \$1.34 per basic and diluted share.

Financial Condition, Liquidity and Capital Resources**Financial Condition**

As of March 31, 2023, we had an accumulated deficit of \$780.7 million. To date, substantially all of our revenues have been from collaboration and license agreements and a contract with the U.S. government that we assumed in connection with the Aceragen Acquisition, and we have received no revenues from the sale of commercial products.

We have devoted substantially all of 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 have not experienced any losses with respect to our funds that had been deposited with SVB.

Funding Requirements

We had cash and cash equivalents of approximately \$2.1 million at March 31, 2023. We believe based on our current operating plan, our existing cash and cash equivalents on hand as of March 31, 2023 and cash received related to U.S. Government reimbursements through the filing date of this Form 10-Q will enable us to fund our operations into August 2023 while maintaining a level of general and administrative expenses to support the business and continue development of ACG-701 for Melioidosis with the reduced workforce effective on April 28, 2023.

Management has evaluated different strategies to obtain the required funding for future operations. Despite its efforts, the Company has been unsuccessful in securing additional capital to fund operations, restructure its outstanding debt and otherwise satisfy creditor obligations. As a result, management and the Board are evaluating an assignment for benefit of creditors and other strategic alternatives that may be available, including bankruptcy and liquidation of the Company.

Cash Flows

The following table presents a summary of the primary sources and uses of cash for the three months ended March 31, 2023 and 2022:

(in thousands)	Three Months Ended March 31,	
	2023	2022
Net cash provided by (used in):		
Operating activities	\$(9,852)	\$(4,568)
Financing activities	(74)	16
Decrease in cash and cash equivalents	<u>\$(9,926)</u>	<u>\$(4,552)</u>

Operating Activities. The net cash used in operating activities for all periods presented consists primarily of our net income adjusted for non-cash charges and changes in components of working capital.

The increase in cash used in operating activities for the three months ended March 31, 2023, as compared to 2022, was primarily due to the impact of the Aceragen Acquisition, including cash outflows associated with acquired development programs (ACG-701 and ACG-801).

Financing Activities. Net cash (used in) provided by financing activities primarily consisted of the following amounts received in connection with the following transactions:

- for the three months ended March 31, 2023, \$0.2 million in payments related to our short-term insurance premium financing arrangement, partially offset by \$0.1 million in proceeds received from stock option exercises; and
- for the three months ended March 31, 2022, less than \$0.1 million in proceeds received from employee stock purchases.

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 *Annex A* 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 in *Annex A* 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 in *Annex A*, 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.

Special Meeting of Stockholders of Aceragen, Inc. will be held at
 Morgan, Lewis & Bockius LLP, 1701 Market Street, Philadelphia, PA 19103 on August 14, 2023 at 10:00 A.M. Eastern Time.

	<p>Small steps make an impact.</p> <p>Help the environment by consenting to receive electronic delivery, sign up at http://www.investorvote.com/ACGN</p>	
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▼ IF VOTING BY MAIL, SIGN, DETACH AND RETURN THE BOTTOM PORTION IN THE ENCLOSED ENVELOPE. ▼

Proxy – Aceragen, Inc.



Special Meeting of Stockholders of Aceragen, Inc.
 August 14, 2023 at 10:00 A.M. Eastern Time

This proxy is solicited by the Board of Directors for use at the Special Meeting of Stockholders.

The undersigned hereby appoints Vincent Milano and John Taylor, and each of them, with full power of substitution, to vote, as designated below, all the shares of Aceragen, Inc. Common Stock held of record by the undersigned at the close of business on July 19, 2023, at the Special Meeting of Stockholders (the "Special Meeting"), to be held on August 14, 2023 at 10:00 A.M. Eastern Time, and at any and all adjournments, continuations, or postponements thereof. The undersigned hereby revokes any and all earlier dated proxies with respect to the Special Meeting. This proxy, when properly executed, will be voted in the manner directed herein by the undersigned. If no direction is made, this proxy will be voted **FOR Proposal 1 to approve the transfer of all or substantially all of Aceragen, Inc.'s assets through an assignment for the benefit of creditors, and FOR Proposal 2 to approve the grant of discretionary authority to Aceragen Inc.'s board of directors to adjourn the special meeting, from time to time, to a later date or dates, even if a quorum is present, to solicit additional proxies in the event that there are insufficient shares present in person or by proxy voting in favor of Proposal 1.**

If any other business is presented at the Special Meeting, including matters incidental to the conduct of the meeting or otherwise, this proxy will be voted by those named in this proxy in their best judgment. At the present time, the Board of Directors knows of no other business to be presented at the Special Meeting.

THIS PROXY WHEN PROPERLY EXECUTED WILL BE VOTED AS DIRECTED OR, IF NO DIRECTION IS GIVEN, WILL BE VOTED AS THE BOARD OF DIRECTORS RECOMMENDS.

See reverse for voting instructions.

C Non-Voting Items

Change of Address – Please print new address below.

Meeting Attendance
 Mark box to the right if you plan to attend the Special Meeting.

