UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 8-K

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

Date of Report (Date of earliest event reported): **January 18, 2023**

Aceragen, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**001-31918**	**04-3072298**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

505 Eagleview Blvd., Suite 212 **Exton, Pennsylvania**	**19341**
(Address of Principal Executive Offices)	(Zip Code)

Registrant's telephone number, including area code: **(484) 348-1600**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425).
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12).
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240-14d-2(b)).
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240-13e-4(c)).

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	IDRA	Nasdaq Capital Market

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

Indicate by check mark whether the registrant is an emerging growth company as defined in as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

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

Item 7.01 Regulation FD Disclosure.

On January 18, 2023, Aceragen, Inc. (formerly known as Idera Pharmaceuticals, Inc.) (the "Company," "we," "us," and "our") uploaded a presentation to its website, www.aceragen.com, discussing the state of the Company. We may rely on all or part of this presentation any time we are discussing the current state of the Company in communications with investors or at conferences. A copy of the presentation is attached to this Current Report on Form 8-K as Exhibit 99.1 (the "Presentation").

The information contained in the Presentation is summary information that is intended to be considered in the context of the Company's Securities and Exchange Commission ("SEC") filings and other public announcements that the Company may make, by press release or otherwise, from time to time. The Company undertakes no duty or obligation to publicly update or revise the information contained in this report, although it may do so from time to time as its management believes is warranted. Any such updating may be made through the filing of other reports or documents with the SEC, through press releases or through other public disclosure.

The information in this Current Report on Form 8-K, including Exhibit 99.1 attached hereto, shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, and shall not be deemed to be incorporated by reference into any of the Company's filings under the Securities Act of 1933, as amended, whether made before or after the date hereof and regardless of any general incorporation language in such filings, except to the extent expressly set forth by specific reference in such a filing.

The website address set forth above is included as an inactive textual reference only. The information contained on the website referenced herein is not incorporated into this Current Report on Form 8-K.

Item 9.01. Financial Statements and Exhibits.

(d)

Exhibit No.	Exhibit Name
99.1	Investor Presentation dated January 18, 2023.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

ACERAGEN, INC.

By: /s/ Bryant D. Lim
Bryant D. Lim
Chief Business Officer and General Counsel

Dated: January 18, 2023

Exhibit 99.1

ACERAGEN

Therapeutics for Rare Pulmonary & Rheumatic Diseases

Nasdaq: ACGN

January 2023

Important Information

The information provided in this presentation pertaining to Aceragen, Inc. (“Aceragen” or “Company”) is for informational purposes only to assist interested parties in making their own independent evaluation. Information contained in this presentation should not be relied upon as advice to buy or sell any securities of Aceragen. You should not construe the contents of this presentation as legal, tax, accounting or investment advice or a recommendation. You should consult your own counsel and tax and financial advisors as to legal and related matters concerning the matters described herein. No legally binding obligations will be created, implied, or inferred from this presentation or the information contained herein. The products outlined in this presentation are still under development. The features of any final product may be different from the features of any product under development described herein, and nothing should be construed as a commitment by Aceragen.

While the information in this presentation is believed to be accurate, Aceragen and their respective agents, advisors, directors, officers, employees and stockholders make no representation or warranties, expressed or implied, as to the accuracy, completeness or reliability of such information. Neither Aceragen, nor any of its affiliates, agents, advisors, directors, officers, employees and stockholders shall have any liability whatsoever, under contract, tort, trust or otherwise, to you or any person resulting from the use of the information in this presentation by you or any of your representatives or for omissions from the information in this presentation. We reserve the right to amend or replace the information contained herein, in part or entirely, at any time, and undertakes no obligation to provide you with access to the amended information or to notify you thereof.

The distribution of this presentation may also be restricted by law and persons into whose possession this presentation comes should inform themselves about and observe any such restrictions. The presentation is not directed to any person in any jurisdiction where (by reason of that person’s nationality, residence or otherwise) the publication or availability of the presentation is prohibited. Persons in respect of whom such prohibitions apply must not access the presentation.

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. All statements, other than statements of historical fact, included or incorporated in this presentation, including, without limitation, statements regarding the Company's new development opportunities, clinical trials and studies, product designation and/or status, financial position, funding for continued operations, cash reserves, projected costs, prospects, clinical trials, plans, expectations, strategies, projections and objectives of management, are forward-looking statements. The words "believes," "anticipates," "estimates," "plans," "expects," "intends," "may," "could," "should," "potential," "likely," "projects," "continue," "will," “schedule,” and "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements are predictions based on our current expectations and projections about future events and various assumptions.

We cannot guarantee that we will achieve the plans, intentions, or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. These forward-looking statements involve known and unknown risks, uncertainties, and other factors, which may be beyond our control, and which may cause our actual results, performance, or achievements to differ materially from future results, performance, or achievements expressed or implied by such forward-looking statements. There are a number of important factors that could cause our actual results to differ materially from those indicated or implied by its forward-looking statements including, without limitation: whether we will be able to successfully integrate the acquired operations; whether our cash resources will be sufficient to fund continuing operations; and newly acquired operations,; whether our products will advance into or through the clinical trial process when anticipated or at all or warrant submission for regulatory approval; whether such products will receive approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies; whether our products receive approval, they will be successfully distributed and marketed; and whether our collaborations will be successful. All forward-looking statements included in this press release are made as of the date hereof and are expressly qualified in their entirety by this cautionary notice, including, without limitation, those risks and uncertainties described in the Company’s Annual Report on Form 10-K for the year ended December 31, 2021, and otherwise in the Company’s subsequent filings and reports filed with Securities and Exchange Commission.

The forward-looking statements contained in this presentation are expressly qualified by this cautionary statement. Except as required by law, we undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise, after the date on which the statements are made or to reflect the occurrence of unanticipated events. Readers are cautioned not to place undue reliance on forward-looking statements.

This presentation does not constitute an offer to sell, or the solicitation of an offer to buy, any securities, nor shall there be any sale of securities in any states or jurisdictions in which such offer, solicitation or sale would be unlawful.

Executive Leadership with Significant Rare Disease Experience

John Taylor
Chief Executive Officer

Biopharma executive and with significant experience financing and building therapeutic companies primarily focused on products for orphan and rare diseases.

Responsible for >$900M in executed transactions and investments over 25-year career.

Dan Salain
Chief Operating Officer

30 years pharma leadership experience including product development, manufacturing, QA/QC, distribution, business development, and corporate operations.

Developed and launched over 30 products globally.

John Kirby
Chief Financial Officer

More than 25 years of public-company finance and business experience from his roles with small to large-sized public pharma companies, including ViroPharma and AstraZeneca, and in public accounting with KPMG.

Responsible for raising over $175M.

Carl Kraus
Chief Medical Officer

Infectious disease physician with 20+ years of clinical experience treating patients and 15 years CMO experience in related drug development.

Former medical officer in the Office of Antimicrobial Products at FDA.

Bryant Lim
Chief Business Officer & General Counsel

25+ years legal and business experience across multiple small & large commercial- and development-stage public pharma companies.

Former Chief Compliance Officer of Incyte Corp. and CBO/GC Idera.

Andy Jordan
Chief Strategy Officer

35+ years executive experience in finance, accounting, and corporate governance including 20-years at KPMG and as CFO of public and private biopharma companies. Led multiple IPOs and raised over $600M.

Finance and Equity

- Cash balance of ~$26.8M as of September 30, 2022
- Cash available expected to provide capital runway for the Company into Q3 2023
- Common shares outstanding as of 1/18/2023: **~8.5M**
  - Share count is post conversion of Series Z preferred and post 1-for-17 reverse split of the Company's issued and outstanding shares

Creating Long-term Value Via Deal-Making

Aceragen launches with **$35M** in product financing from and acquires worldwide rights to ACG-801 from ENZYVANT

MARCH 2021

Aceragen acquires ARREVUS and ACG-701 as a treatment for cystic fibrosis pulmonary exacerbations

OCT 2021

Awarded **$45M** in funding for the development of ACG-701 for the treatment of melioidosis from DTRA

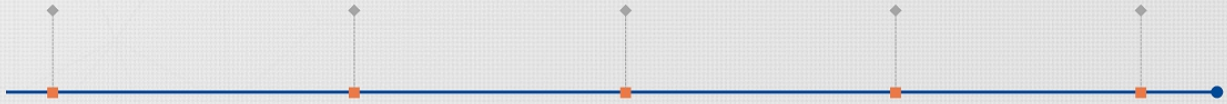
OCT 2021

Awarded **$3.5M** development award for ACG-701 for the treatment of CF from CYSTIC FIBROSIS FOUNDATION ADDING TOMORROWS

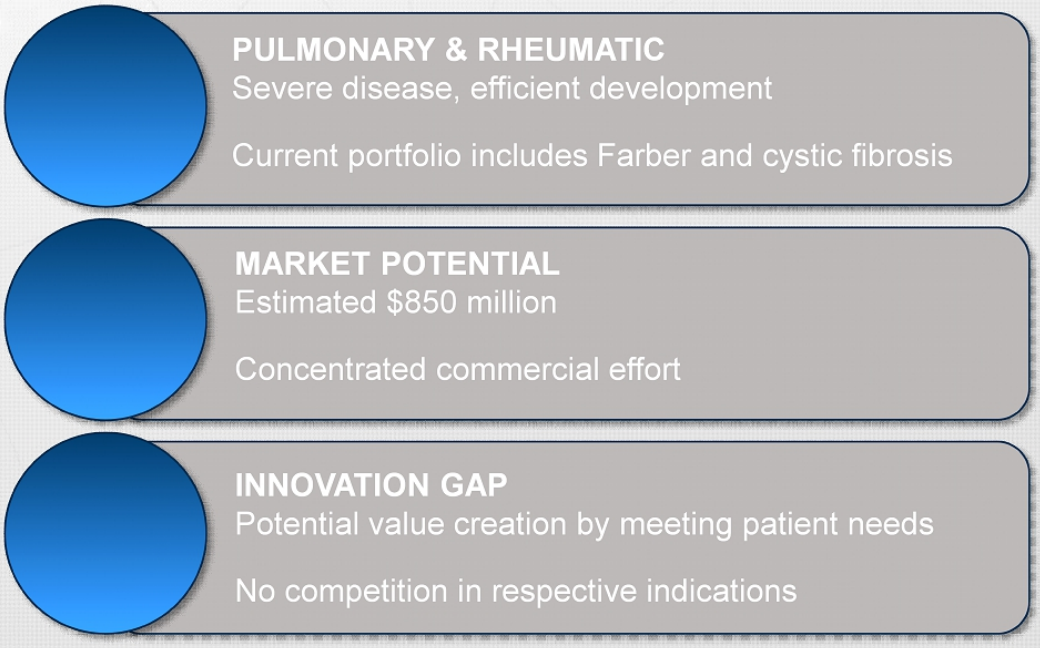
JAN 2022

Aceragen merges with idera to form a Nasdaq-listed rare disease company

SEPT 2022

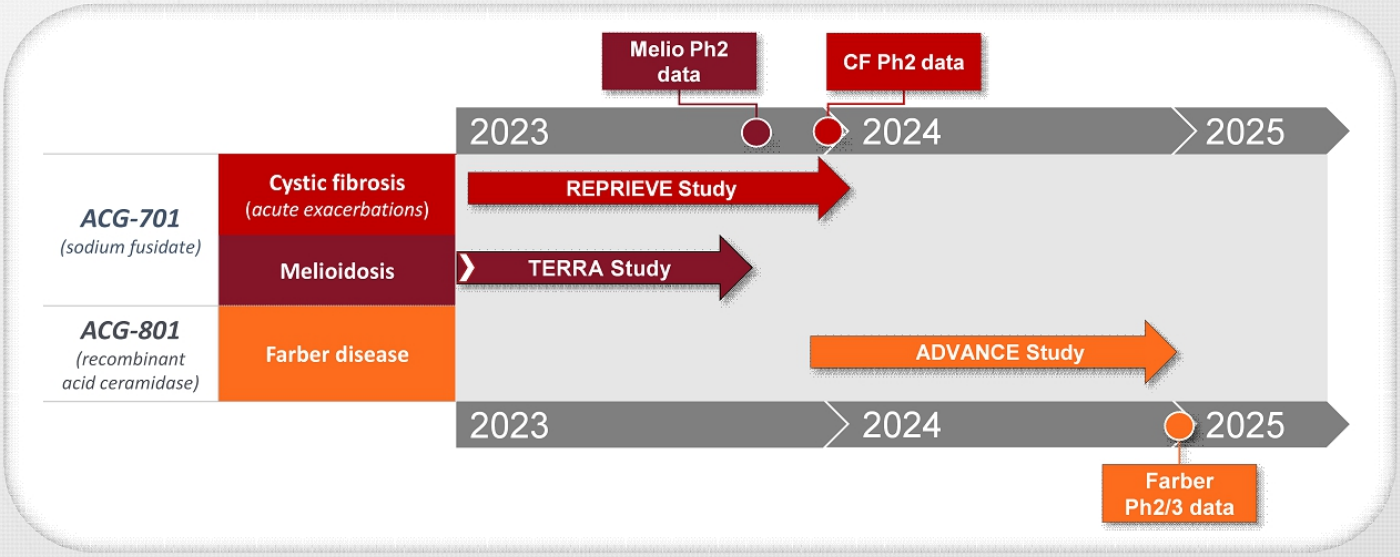


Rare Disease Company



Advanced Clinical Portfolio

Multiple Near-Term Inflection Points



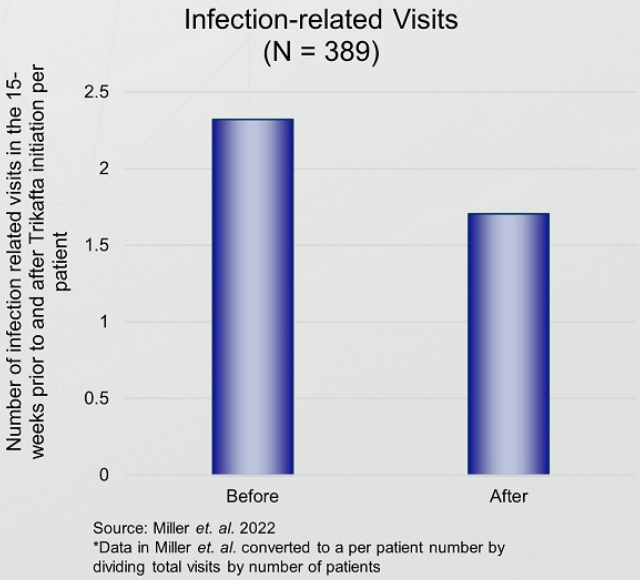
ACG-701

Patented Oral Formulation of Sodium Fusidate

Target Product Profile	Differentiated oral product for acute pulmonary exacerbations with anti-inflammatory, anti-infective, and mucin inhibitory activity
Initial Indications	Acute CF pulmonary exacerbations – ***Data expected 2H'2023*** Melioidosis – ***Data expected 2H'2023***
Regulatory Status / Designations	NCE status, Orphan, Fast Track, and QIDP
Estimated Market Potential	**>$500M**

CF Pulmonary Exacerbations (CF PEx)

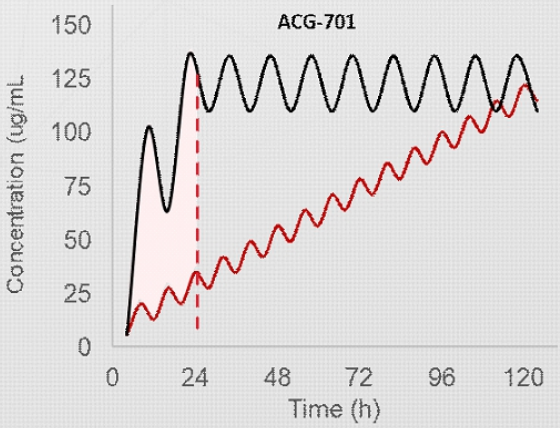
- CF PEx are characterized as respiratory events accompanied by an acute decrease in lung function
- The 35,000 CF patients in the US experience ~1-2 exacerbations/year
  - ~70% of the US CF population is at risk for MRSA infections
  - Exacerbations and related complications account for nearly two-thirds of morbidity and mortality for CF patients
- No therapies FDA approved to treat acute CF PEx



Source: Miller *et. al.* 2022
*Data in Miller *et. al.* converted to a per patient number by dividing total visits by number of patients

ACG-701: Loading Dose Formulation Uniquely Suited for Severe Pulmonary Disease

- Loading dose achieves IV-like blood levels within 24 hours
  - Dosing regimen enhances potency seen in prior clinical use
  - 3X increase in potency for Staph/MRSA
- Extensive safety database (positive Phase 3)
- **Potentially first product approved to treat acute PEx, addresses major symptoms**
  - Anti-inflammatory
  - Anti-infective
  - Mucin suppression
- Potential for **12 years of exclusivity** from first approval in the US (NCE/Orphan Status/QIDP) and 10 years of exclusivity in Japan and EU (Orphan Status)

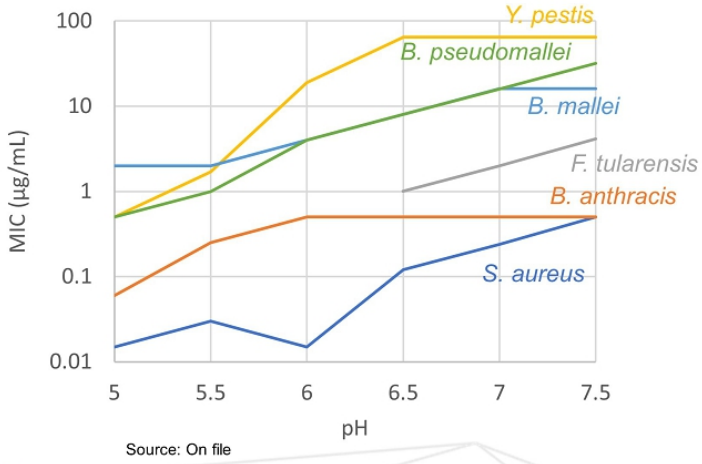


Source: Fernandes. Cold Spring Harbor 2016

ACG-701: Patented Sodium Fusidate Formulation

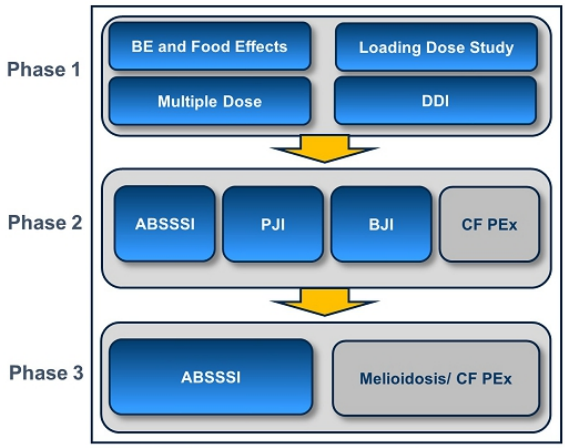
Multiple Clinical Studies Demonstrating Efficacy

Sodium fusidate has unique activity at low pH



Source: On file

ACG-701 has demonstrated safety and efficacy in 650+ patients across multiple indications



ABSSSI: Acute bacterial skin and skin structure infections
PJI: Prosthetic Joint Infections
BJI: Bone & joint infections

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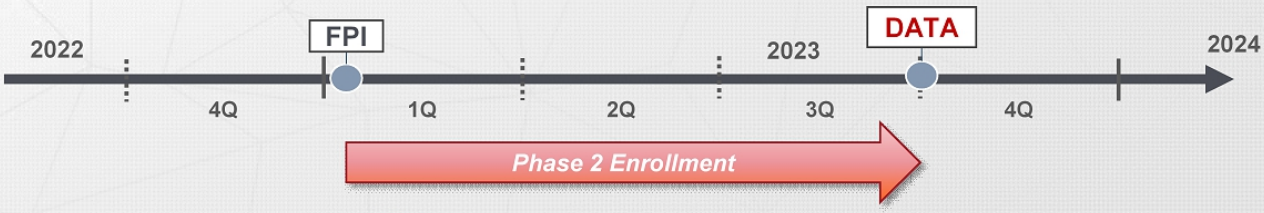
The REPRIEVE Study in CF PEx

Expected to begin in Q1'2023



THE *REPRIEVE* STUDY DESIGN

- Randomized, double-blind, placebo-controlled study for newly diagnosed pulmonary exacerbations in CF patients
- Two-week oral BID treatment plus two-week follow-up
- Endpoints: CRISS, FEV1, and antimicrobial regimen changes through Day 14 in a single statistical measure (DOOR)

CRISS: difficulty breathing, cough, cough up mucus, chest tightness, wheeze, feeling feverish, tired, and chills/sweats

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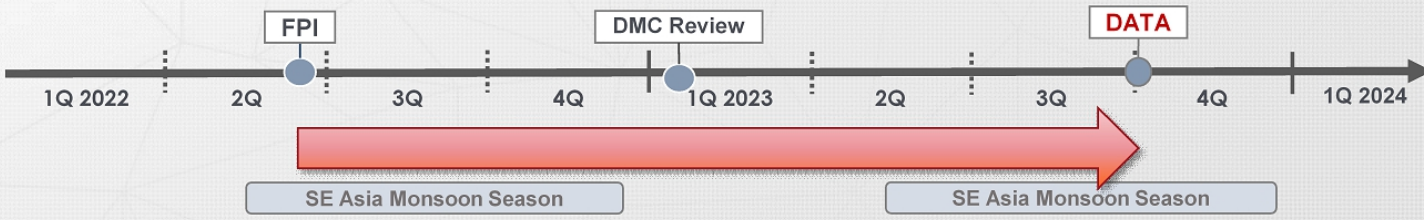
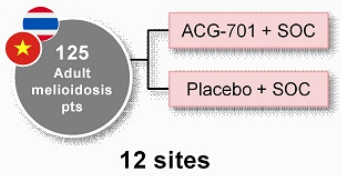
The TERRA Study in Melioidosis is Underway

DoD funded program providing strategic support for commercial effort



The *TERRA* Study Design

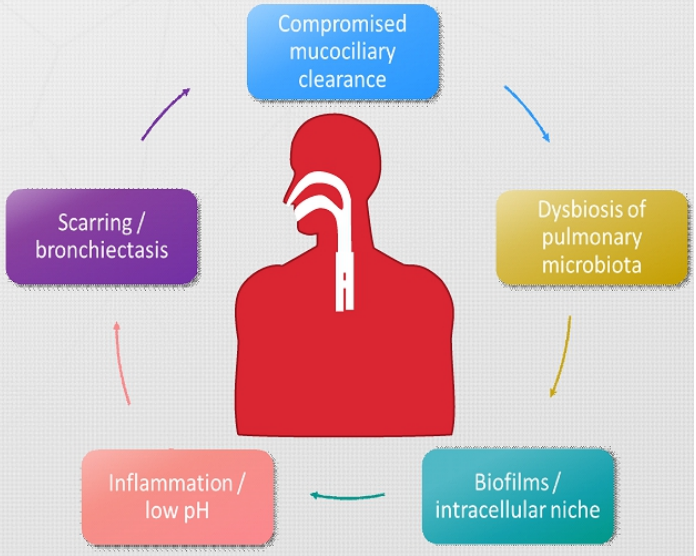
- Randomized, double-blind, placebo-controlled study in hospitalized melioidosis patients
- Two-week BID dosing with two-week follow-up
- Endpoints: mortality, organ failure, sepsis and treatment modifications through Day 14 in a single statistical measure (DOOR)



12 sites

ACG-701: Key Points

- Compound has established efficacy and safety profile
- Loading dose designed to enhance performance in severe disease
  - Extensive safety database for product
- Rare disease business model
  - ~60,000 exacerbations/yr in established CF market
  - **Total addressable market for acute exacerbations = multi-billion dollar potential**
- 12 years of market exclusivity anticipated from approval



$500M sales potential in CF and melioidosis

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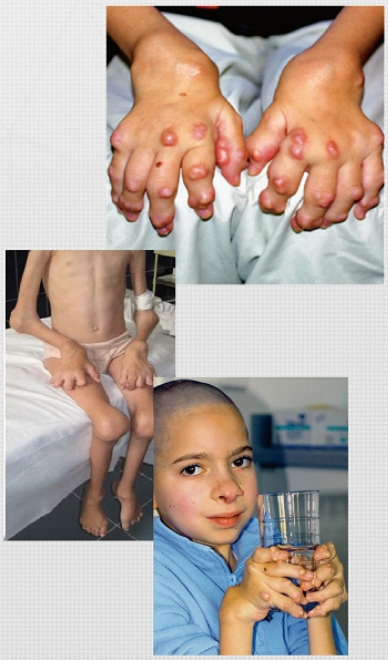
ACG-801

Recombinant Human Acid Ceramidase

Target Product Profile	Disease-modifying enzyme replacement therapy (ERT) addressing enzyme deficiency and ceramide accumulation in Farber disease patients
Initial Indication	Farber disease (monogenic loss of function) – ***Study start expected 1Q'2024***
Regulatory Status / Designations	Orphan, Fast Track, Biologic and Rare Pediatric Disease
Annual Peak Sales Estimate	**>$350M**

Farber Disease

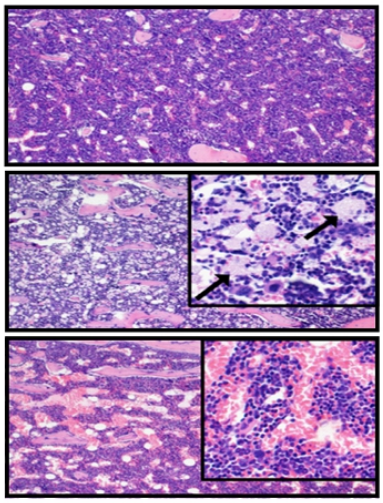
A severe and progressive monogenic lysosomal storage disorder (LSD)



- Farber is an enzyme deficiency resulting in accumulation of ceramide, highly inflammatory lipid
- Disease severity is a spectrum; severe phenotype results in death before age 2
  - Respiratory failure common cause of death
- Current treatments relieve some pain but don't impact disease progression
- Worldwide prevalence estimated to be 1,000-1,500 patients (similar to MPS VI)

ACG-801 for Farber Disease

ACG-801 EFFECTIVE IN FARBER MOUSE MODEL



WT mouse
(bone marrow)

Farber mouse
untreated
(black arrows:
macrophage infiltrates)

Farber mouse
+ ACG-801
(resolution of
macrophage infiltrates)

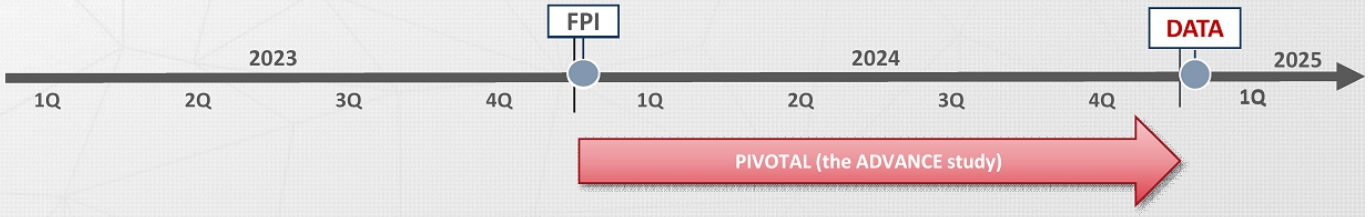
Source: ACG-801 Investigator's Brochure

ACG-801 treatment of Farber mice results in:

- ✓ Reduction of ceramide deposits in target tissues
- ✓ Reduction of inflammatory biomarkers/cell types to wild type levels
- ✓ **Amelioration of bone, joint, and soft tissue lesions (pathophysiology same as clinical endpoint)**
- ✓ Additional 3-week lifespan in newborn mice

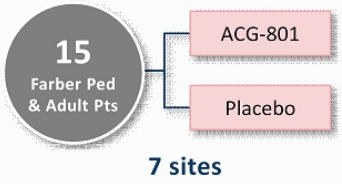
ACG-801 for Farber Disease

Aceragen is planning a single registrational trial for US/EU submission



THE *ADVANCE* STUDY DESIGN

- Randomized, double-blind, placebo-controlled, first-in-human Phase 2/3 study in Farber disease patients
- Systemic IV infusion, every other week
- Endpoints are nodule changes and patient-specific disease burden improvement (e.g., pain, mobility, impact score) through week 28



7 sites

ACG-801: Enzyme Replacement Therapy For Enzyme Deficiencies Have Good History Of Success

- ACG-801 has potential to address disease causing enzyme deficiency
- Historically, animal data have been highly predictive for ERT development in monogenic LSDs
- Global IP portfolio
- No known competitors

PRECLINICAL EFFICACY HAS BEEN PREDICTIVE OF CLINICAL EFFICACY FOR ERTs

Therapy (Disease)	Pre-Clinical Effective Dose
Aldurazyme (MPS I)	0.5-2mg/kg weekly (canine)
Fabrazyme (Fabry)	0.3-3mg/kg bi-weekly (rodent)
Naglazyme (MPS VI)	1-2mg/kg weekly (feline)
Myozyme (Pompe)	10-100mg/kg bi-weekly (rodent)
Kanuma (LALD)	0.35-3mg/kg bi-weekly (rodent)
ACG-801 (Farber)	**1-10mg/kg bi-weekly (rodent)**

>$350M in worldwide annual peak sales estimated for ACG-801 for Farber disease

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Key Takeaways

Near-Term Milestones, Long-Term Value

Late-Stage Clinical Portfolio

- 3 clinical programs with near-term inflection points, high unmet need
- Annual sales potential exceeding $850M
- Full commercial ownership of programs; centers of excellence model

Management Team with Proven Rare Disease Expertise

- Rare product leadership and commercialization (Cinryze, Kanuma, Zenpep)
- Collective experience that spans all aspects of product development

Capital Efficient Development

- Strategic, non-dilutive funding
- NovaQuest, DoD and CF Foundation partnerships

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