Therapeutics for Rare Pulmonary & Rheumatic Diseases

Nasdaq: ACGN

February 2023

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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.

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 Patient-driven breakthroughs 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 DEFENSE THREAT REDUCTION AGENCY

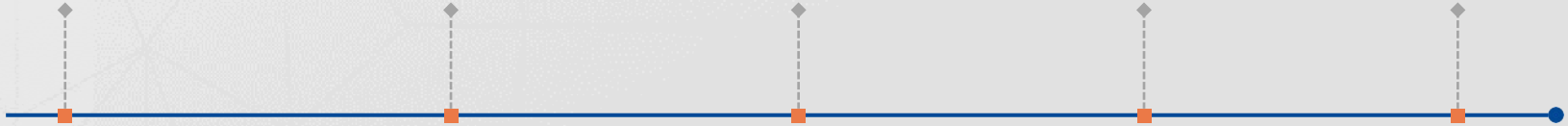
OCT 2021

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

PULMONARY & RHEUMATIC
Severe disease, efficient development

Current portfolio includes Farber and cystic fibrosis

MARKET POTENTIAL
Current programs estimated >$850 million

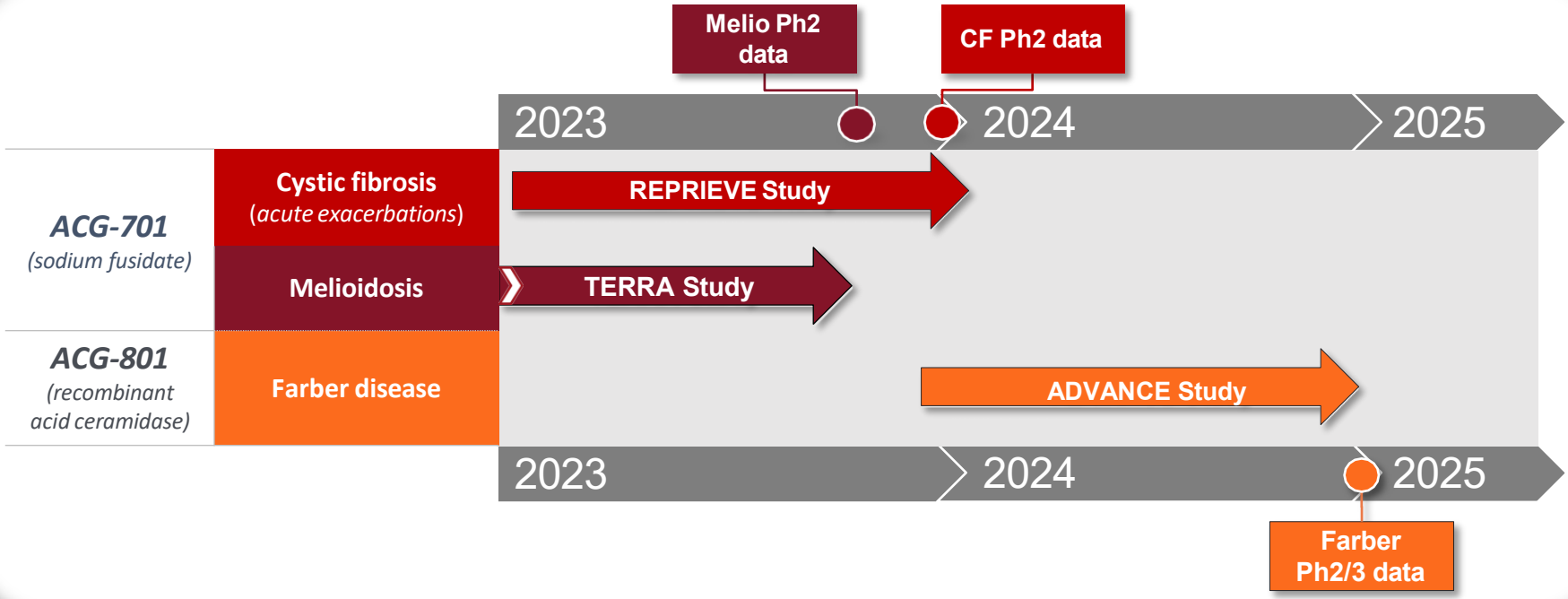
Concentrated commercial effort

INNOVATION GAP
Mission is to create value by meeting patient needs

No approved treatments for respective indications

Advanced Clinical Portfolio

Multiple Near-Term Inflection Points



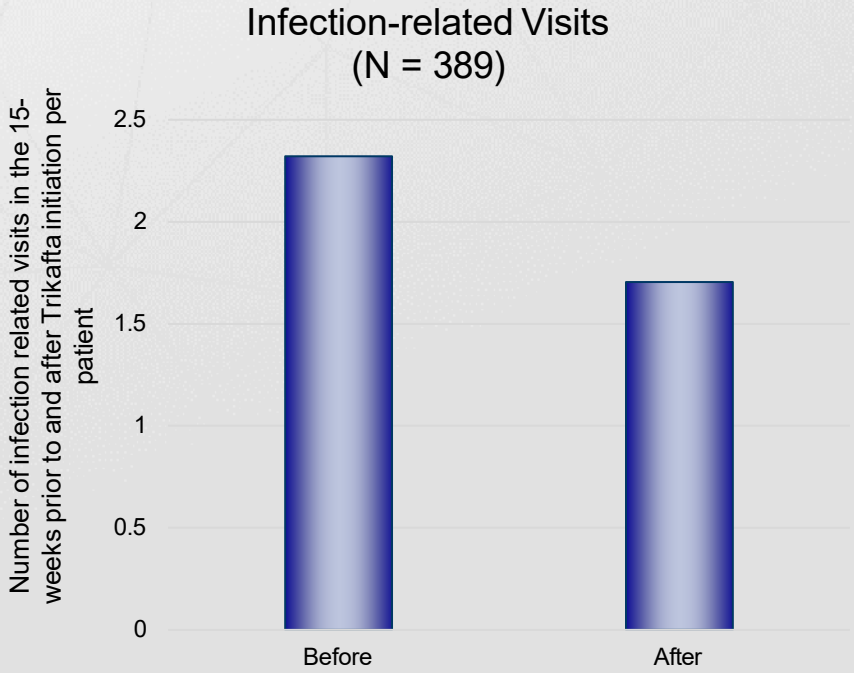
ACG-701

Investigational Treatment for Acute Exacerbations

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)

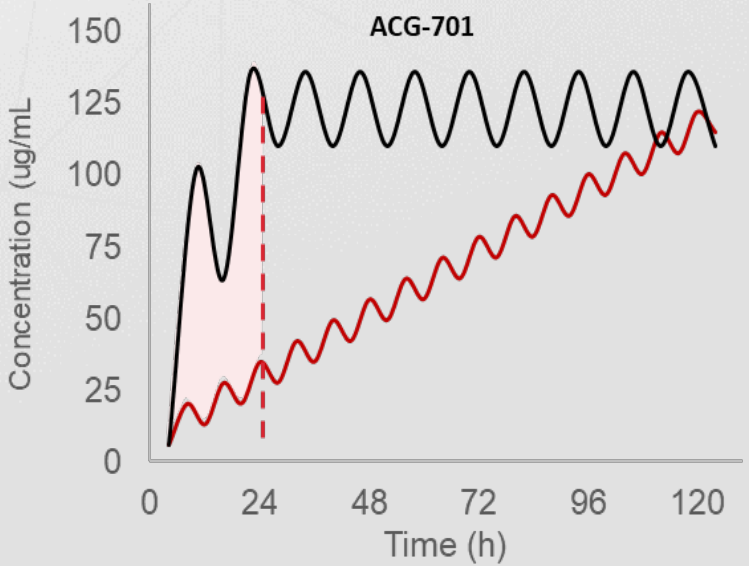
- CF PEx are characterized as respiratory events accompanied by an acute decrease in lung function
- The 35,000 CF patients in the US experience ~1-2 exacerbations/year
  - ~70% of the US CF population is at risk for MRSA infections
  - Exacerbations and related complications account for nearly two-thirds 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

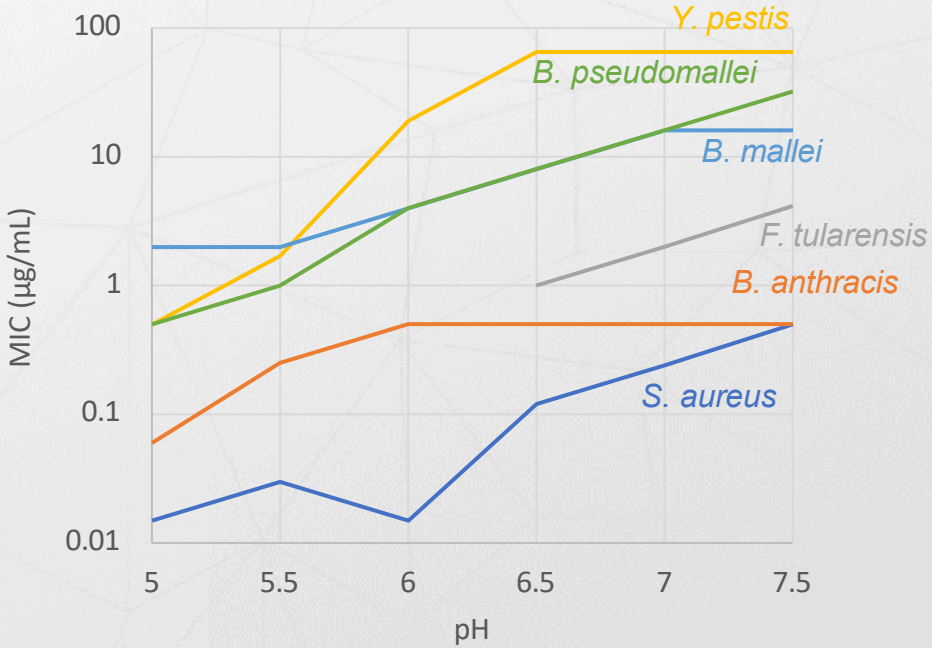
- Loading dose achieves IV-like blood levels within 24 hours
  - Dosing regimen enhances potency seen in prior clinical use
  - 3X increase in potency for Staph/MRSA
- Extensive safety database (positive Phase 3)
- **Potentially first product approved to treat acute PEx, intended to address major symptoms**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



Source: Fernandes. Cold Spring Harbor 2016

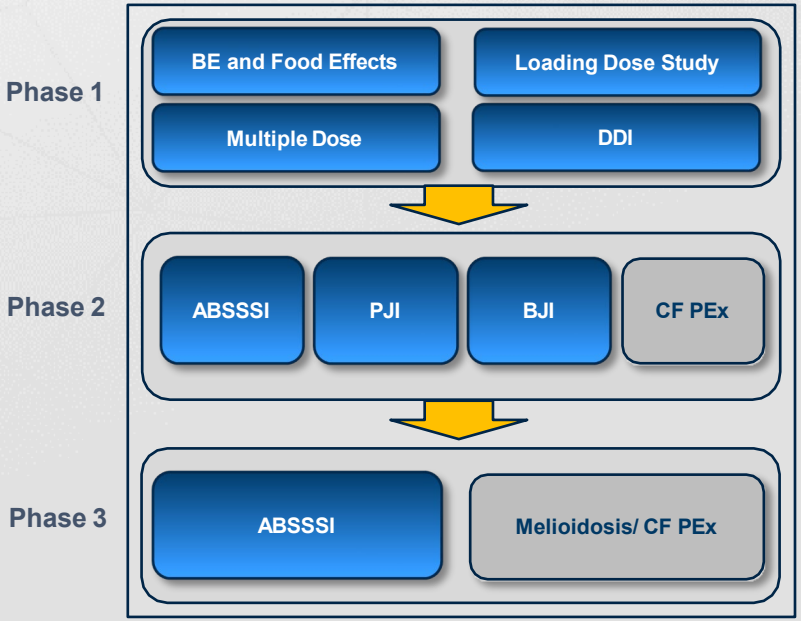
ACG-701: Unique Activity; Significant Prior Investment

Sodium fusidate has unique increase in potency at low pH



Source: On file

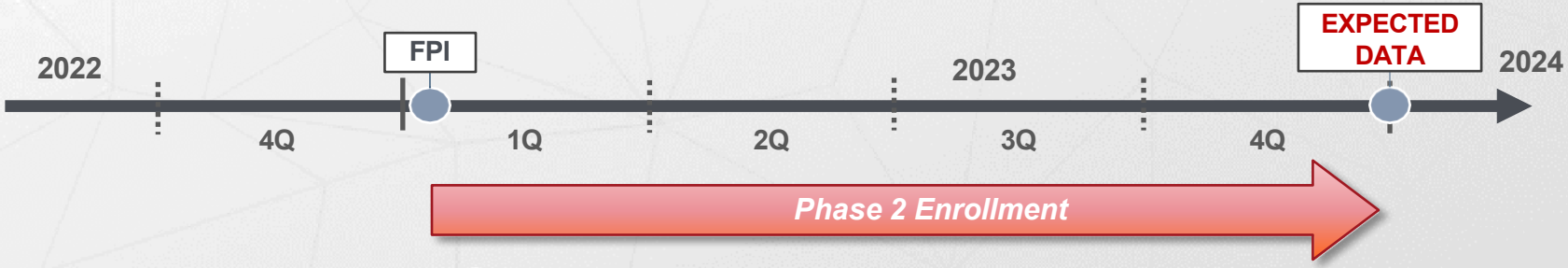
ACG-701 has a demonstrated safety and efficacy profile in eight studies across multiple indications



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

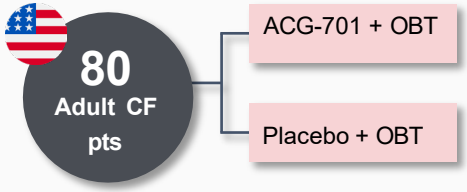
The REPRIEVE Study in CF PEx

Enrollment beginning 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)

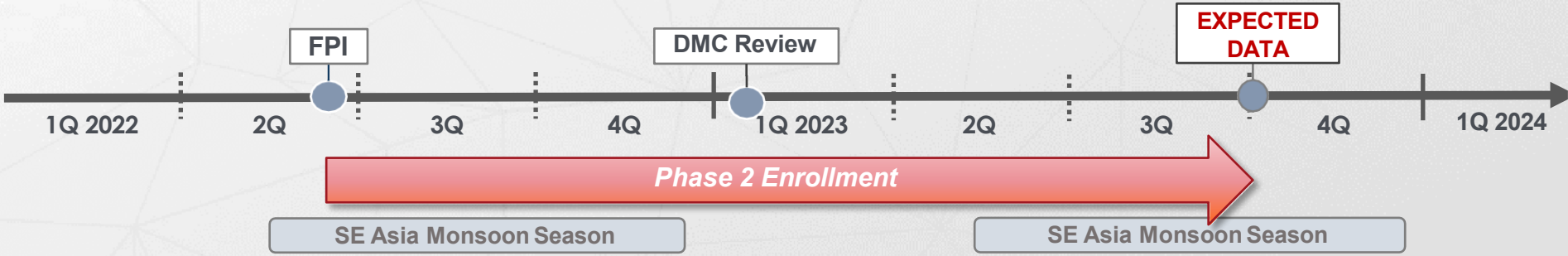


25 sites

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

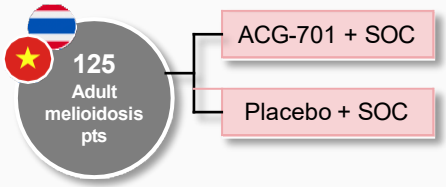
The TERRA Study in Melioidosis is Progressing

DoD funded program providing strategic support for commercial effort



The *TERRA* Study Design

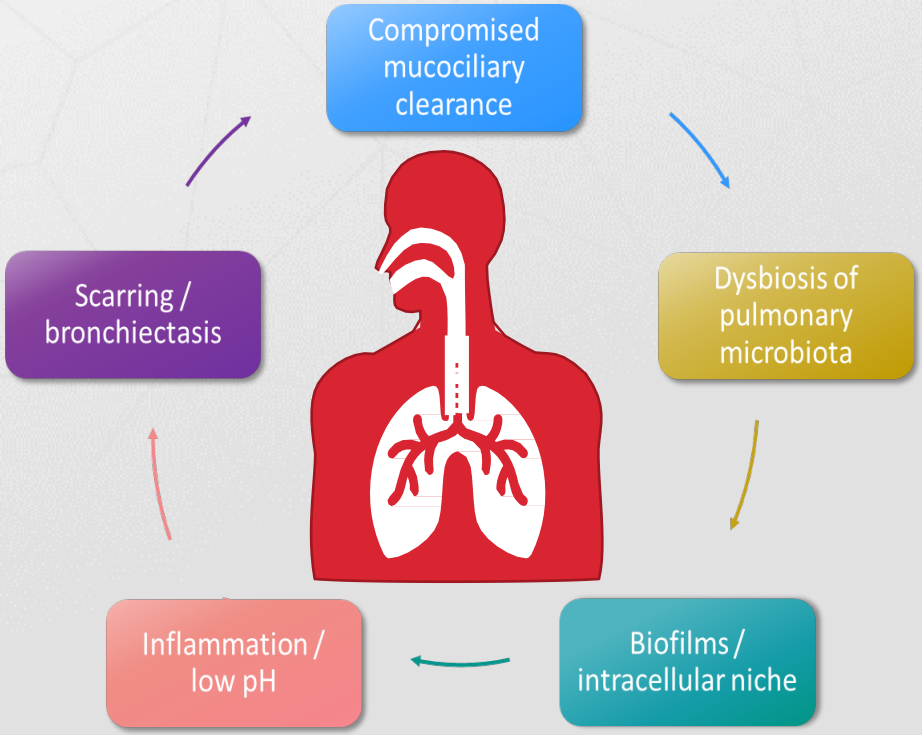
- Randomized, double-blind, placebo-controlled study in hospitalized melioidosis patients
- Two-week BID dosing with two-week follow-up
- Endpoints: mortality, organ failure, sepsis and treatment modifications through Day 14 in a single statistical measure (DOOR)
- DMC recommended study continuation with no changes



12 sites

ACG-701: Investigational Treatment for Acute Exacerbations

- 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
  - ~70,000 exacerbations/yr in established CF market
  - **Total addressable market for acute exacerbations = multi-billion dollar potential**
- Durable market exclusivity anticipated to capture market value



$500M sales potential in CF and melioidosis

ACG-801

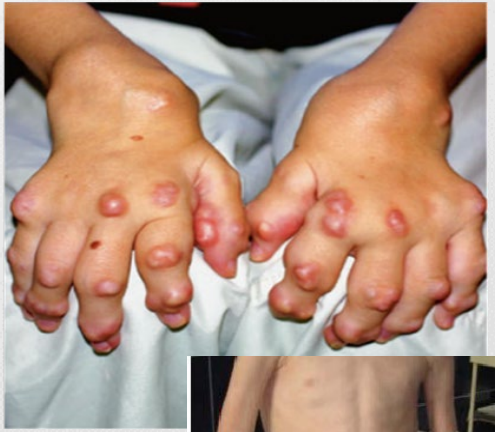
Recombinant Human Acid Ceramidase; Novel Therapy

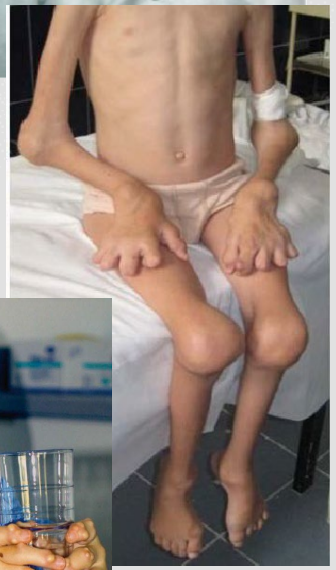
Target Product Profile	Disease-modifying enzyme replacement therapy (ERT) addressing enzyme deficiency and ceramide accumulation in Farber disease patients
Initial Indication	Farber disease (monogenic loss of function) – ***Study start expected 1Q'2024***
Regulatory Status / Designations	Orphan, Fast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

Farber Disease

A severe and progressive monogenic lysosomal storage disorder (LSD)

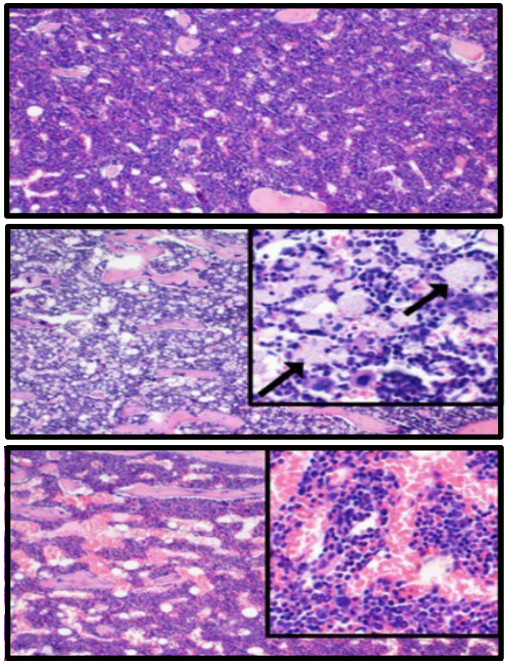
- Farber is an enzyme deficiency resulting in accumulation of ceramide, highly inflammatory lipid
- Disease severity is a spectrum; severe phenotype results in death before age 2
  - Respiratory failure common cause of death
- Current treatments relieve some pain but don't impact disease progression
- 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)

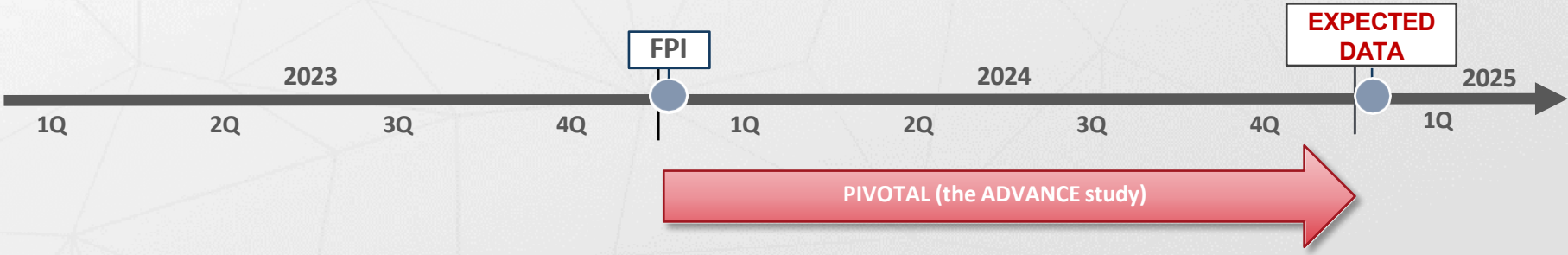
Source: ACG-801 Investigator's Brochure

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

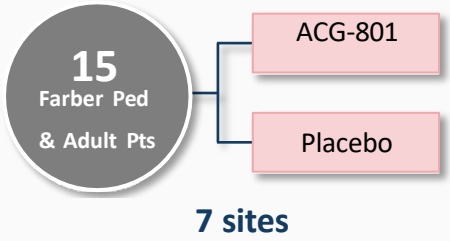
ACG-801 for Farber Disease

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



**Program Currently Under Clinical Hold*

ACG-801: Novel Treatment for Unserved Disease

- ACG-801 has potential to address disease causing enzyme deficiency
- ERT is established treatment modality and commercial model for LSDs
- Aceragen providing genetic tests globally to facilitate diagnosis
- Natural history study completed, publications pending
- 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

ACERAGEN

Key Takeaways

Near-Term Milestones, Building Toward Commercialization

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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