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A Clinical-Stage Orphan Lung Disease Company Based in Austin and Copenhagen

Focused Therapeutic Area

Developing new medicines for rare lung diseases.

Clear Vision

To become THE orphan lung disease company.

Attractive Pipeline

Includes 3 late-stage programs.

~ $100M*
Cash, cash equivalents and short-term investments

Additional ~ $46M†
Second tranche from Dec. 2019 financing

*As of June 30, 2020.
†Gross proceeds if milestone warrants are exercised in full.
Executive Leadership

We are a team with deep expertise in orphan lung diseases and pulmonary medicine and a proven track record that spans from early clinical development through commercialization.

Rob Neville  
*CEO*

Taneli Jouhikainen  
*CBO*

Dave Lowrance  
*CFO*

Badrul Chowdhury  
*CMO*
### Current Late-Stage Pipeline and Anticipated Milestones

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**SAVARA**

5
Molgradex

Inhaled GM-CSF for Autoimmune Pulmonary Alveolar Proteinosis (aPAP)
APAP: Excess of Surfactant in the Lungs

Mechanism of disease well understood

US prevalence of ~2,500 patients*
Typical onset 30-50 yrs

Anti-GM-CSF antibodies cause accumulation of surfactant in the alveoli

Decreased oxygen delivery
Hypoxia and shortness of breath

Currently treated by whole lung lavage (WLL)

IMPALA Study Did Not Meet Primary Endpoint

IMPALA was conducted at 34 sites across 18 countries.

[Graph showing change in A-aDO2 from baseline over 24 weeks (ITT)]

- **Full Analysis Set (FAS)**
  - Estimated treatment difference of -4.6 mmHg (p=0.17)

- **Revised FAS**
  - Estimated treatment difference of -6.5 mmHG (p=0.025)

*Protocol specified analysis (ITT).

†Revised analysis excludes 4 patients using supplemental oxygen during testing. (Placebo: n=2, Intermittent: n=1, Continuous: n=1).
IMPALA: DLCO and SGRQ Showed Robust Improvement with Continuous Dose (OD)

Change in Diffusion Capacity for Carbon Monoxide (DLCO) From Baseline Over 24-weeks (FAS)

Change in St. George’s Respiratory Questionnaire (SGRQ) From Baseline Over 24-weeks (FAS)

OD estimated treatment difference of 7.9% predicted (p=0.007)

OD estimated treatment difference of 7.6 points (p=0.01)

Results not adjusted for multiplicity.
IMPALA Open-Label Data Show Sustained Effect, or Continued Improvement, after Longer-Term Drug Exposure

Continuous Molgradex  Placebo  Intermittent Molgradex

Dosing schedules for blinded and open-label period were different.

All patients received intermittent Molgradex during open-label period.
IMPALA 2 Study Design

**Primary Endpoints**
- Change from baseline in DLCO

**Secondary Endpoints**
- SGRQ Total Score
- SGRQ Activity Score
- Exercise capacity using treadmill test

**Screening**
- n=80
- Molgradex 300 µg daily dosing
- Placebo
- BL
- W 24
- W 48

**Period 1: Double-blind**
- n=80
- Molgradex 300 µg daily dosing
- W 24
- W 48

**Period 2: Open-label**
- Molgradex 300 µg daily dosing
- W 96

Study expected to start in Q1 2021.
IMPALA 2 will be conducted at ~50 sites across ~15 countries.
APULMIQ

Inhaled Ciprofloxacin
Lead Indication: Non-CF Bronchiectasis (NCFB)

March 2020: Savara obtained the rights to develop and commercialize Apulmiq
High Unmet Need for Inhaled NCFB Treatment

- 150,000+ prevalence in U.S.
  More prominent in older individuals
- Vicious cycle of infection driving inflammatory damage
- Sputum volume/purulence, breathlessness, fatigue, hemoptysis
  Exacerbations lead to hospitalization and are associated with increased mortality
- No approved medicines
Time-to-First Pulmonary Exacerbation Improved in ORBIT-4 but not in ORBIT-3

Data shown is based on re-adjudication of pulmonary exacerbation events. The original p-values were: ORBIT-4, p=0.058; ORBIT-3, p=0.826.
Frequency of Pulmonary Exacerbations Improved in Both ORBIT-4 and ORBIT-3

Next steps: Discuss design of a confirmatory program with the FDA. There is general consensus that frequency of pulmonary exacerbation is the most clinically relevant endpoint.
AEROVANC

Inhaled Vancomycin for MRSA in Cystic Fibrosis
High Unmet Need for Inhaled MRSA Treatment

**US prevalence of CF***
30,000 patients, 26% MRSA infected

**Persistent lung infections managed with chronic inhaled antibiotics**

**MRSA infection associated with worse clinical outcomes**

**No approved inhaled MRSA antibiotic, emerging use of nebulized IV form of vancomycin**


*Source: 2015 CFF Patient Registry

Dasenbrook, et al. reprinted with permission, Copyright © (2010) JAMA
**FEV$_1$ Improvement Consistent with TOBI Data**

- **Best FEV$_1$ improvement in young patients**
- **Similar FEV$_1$ response profile in prior TOBI studies**
- **Absolute change in FEV$_1$ selected as Phase 3 primary endpoint**
- **Phase 3 powered and focused on patients 6-21 years of age**

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**Graph:**
- **Absolute Change in FEV$_1$, Percent Predicted (%):**
  - PP population, 32 mg dose cohort, < 21 years of age, n = 16, post hoc analysis

**Bar Chart:**
- **Relative Change in FEV$_1$, Percent Predicted (%):**
  - Age 6-12, 13-19, 20+
AVAIL Phase 3 Study Design

Primary Endpoint

- FEV₁ improvement at weeks 4, 12, and 20 (absolute change analyzed sequentially)
- Primary analysis based on patients 6-21 years of age

Secondary Endpoints

- Time to use of another antibiotic for pulmonary infection
- FEV₁ improvement (relative change, number of response cycles)
- Respiratory Symptoms Diary

Enrollment closed in March 2020 due to COVID-19. Total enrollment was 133 patients ≤ 21 years (target=150), 55 patients > 21 years (target=50)

Top line results expected in early 2021
Molgradex

Inhaled GM-CSF for Nontuberculous Mycobacterial (NTM) Lung Infection
The Conundrum of NTM Lung Infection

- US prevalence of up to 80,000 patients*
  - Typical onset 50-75 yrs
- Inability of macrophages to kill bacteria in the lungs
- Cough, fatigue, weight loss, progressive lung destruction
- Current antibiotic treatments toxic, burdensome, and often inadequate

Molgradex Phase 2A Optima Open-Label Study

Primary Endpoint
- NTM sputum culture conversion to negative
  (conversion = negative culture at 3 consecutive timepoints)

Secondary Endpoints
- NTM sputum smear conversion to negative
- Durability of NTM sputum conversion
- Reduction of NTM in sputum
- Change in 6-minute walk distance
- Change in body weight
- Change in QoL and symptom scores

Q1 2019:
Initiated ENCORE, a new NTM study in patients with CF

Q1 2020:
Announced OPTIMA top line microbiology results
Phase 2a Optima Top Line Results*

**MICROBIOLOGICAL DATA (n=32)**

<table>
<thead>
<tr>
<th>Infection Type</th>
<th># Pts: Group 1 (anti-mycobacterial)</th>
<th># Pts: Group 2 (no anti-mycobacterial)</th>
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<tbody>
<tr>
<td>MAC (n=24)</td>
<td>11</td>
<td>13</td>
</tr>
<tr>
<td>• 5 culture neg</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>• 2 culture neg through follow-up period</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>MABSC (n=8)</td>
<td>3</td>
<td>5</td>
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<tr>
<td>• Neg smear/culture not observed</td>
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*Results as of March 2020

- Assessment of data continues to better understand clinical outcomes in specific patients.
- Next steps TBD once results from ENCORE study (NTM in CF) are available.
Financials and Milestones
Savara Financial Overview

As of June 30, 2020

- **Cash, cash equivalents, and short-term investment securities**
  - ~$100 million*

- **Common stock**
  - ~53 million (outstanding)
  - ~101 million (fully diluted)

- **December 2019 private placement**
  - Additional potential gross proceeds of ~ $46M
  - (Second tranche includes milestone warrants exercisable following a defined clinical milestone or 2 years after closing date of financing.)

- **Loan and security agreement**
  - $25 million outstanding

*Includes first tranche ($26.8M) from 12/19 financing.
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