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Call agenda

- Introduction
  - Brett Kaplan, M.D., Chief Financial Officer

- Overview
  - Asa Abeliovich, M.D., Ph.D., CEO & Founder

- Clinical Update
  - Jeffrey Sevigny, M.D., Chief Medical Officer

- Financial Update
  - Brett Kaplan, M.D., Chief Financial Officer

- Q&A
Overview

Asa Abeliovich, M.D., Ph.D., CEO & Founder
Focused on our mission

- Potential disease-modifying targets identified based on human genetics
- Targeting genetically defined patient populations
- Gene delivery with AAV9 vector has a track record of efficacy and safety

- Phase 1/2 PROPEL trial for Parkinson’s with GBA1 mutations (PD-GBA) underway; PD-GBA affects >90K Americans
- Potential for rapid proof-of-concept for PR001 in neuronopathic Gaucher disease; PROVIDE trial planned to initiate enrollment in 2H 2020

- PR006 IND active for frontotemporal dementia with GRN mutations (FTD-GRN)
- Phase 1/2 PROCLAIM trial on track to initiate enrollment in 2H 2020

- Expertise in developing therapies for neurodegenerative diseases
- Additional genetically-validated targets in Parkinson’s, Alzheimer’s, ALS, FTD
- Leaders in gene therapy manufacturing and process development
Rapid advancement to clinical stage

**2017**
- **AUGUST 2017:** Seed financing and REGENXBIO license
- **OCTOBER 2017:** Lab space secured in NYC

**2018**
- **MARCH 2018:** $75M Series A
- **MAY 2018:** REGENXBIO second agreement

**2019**
- **MARCH 2019:** $50M Series B
- **MAY 2019:** PR001 PD-GBA IND active
- **DECEMBER 2019:** PR001 nGD IND active

**2020**
- **MARCH 2020:** PR006 FTD-GRN IND active
Unique pipeline of potentially disease-modifying AAV9 gene therapies for neurodegenerative diseases

<table>
<thead>
<tr>
<th>Programs</th>
<th>Indication</th>
<th>Approach</th>
<th>Stage of Development</th>
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<td>GBA1 Gene Transfer</td>
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<td>Neuronopathic Gaucher disease</td>
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<td>FTD-GRN</td>
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<td>IND Active</td>
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<td>PR004</td>
<td>Synucleinopathies</td>
<td>GBA1 Gene Transfer + α-Synuclein Knockdown</td>
<td>Profile</td>
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**FTD granted by FDA for PR001 for the treatment of PD-GBA**
**ODD & RPDD granted by FDA for PR001 for the treatment of GD & nGD, respectively**
**ODD & FTD granted by FDA for PR006 for the treatment of FTD & FTD-GRN, respectively**

Prevail owns worldwide commercial rights to all product candidates in the pipeline
Second quarter business highlights

- Clinical development of PR001 advances
  - Two nGD patients dosed via compassionate use requests
  - Two PD-GBA patients enrolled in Phase 1/2 PROPEL trial
- Encouraging early data available from one nGD patient and one PD-GBA patient
  - In both patients CSF GCase levels were undetectable at baseline; normalized at ~3-4 months after PR001 administration
- No PR001-related AEs reported in the two nGD compassionate use patients
- One PD-GBA patient dosed with PR001 experienced SAEs that markedly resolved with additional immunosuppression
- Based on early clinical data PROPEL trial protocol amended and enrollment expected to continue in 2H 2020
- Study startup activities continuing for Phase 1/2 trials of PR001 for GD2 and PR006 for FTD-GRN
- Leadership team strengthened with new General Counsel
Clinical Update

Jeffrey Sevigny, M.D., Chief Medical Officer
PR001 program update

**nGD Compassionate Use**

- Two nGD patients administered PR001 following compassionate use requests
  - ~22-month-old GD2 patient dosed in January 2020 (as previously disclosed)
  - Additional nGD patient recently dosed

**proPEL**

- Two patients enrolled in PROPEL Phase 1/2 trial for PD-GBA
  - One patient received $1.4 \times 10^{14}$ vg of PR001; second patient received sham procedure
- PROPEL enrollment expected to continue in 2H 2020

**proVIDE**

- PROVIDE Phase 1/2 trial for Type 2 Gaucher disease anticipated to initiate enrollment in 2H 2020

**proGRESS**

- Initiation of PROGRESS Phase 1/2 trial for Type 3 Gaucher disease postponed until additional clinical data from PROPEL and PROVIDE available
Type 2 Gaucher compassionate use: early patient data

Background

- Type 2 Gaucher disease patient dosed with PR001 (1.3x10^{14} vg) in Jan 2020 following compassionate use request
- Patient was ~22 months old at dosing

Biomarkers

- CSF GCase enzyme activity increased from undetectable at baseline to normal level at Month 4

<table>
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<th>GCase activity in CSF (μmol/L/d)</th>
<th>Day 0</th>
<th>Month 1</th>
<th>Month 4</th>
<th>Normal range (adult)</th>
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<td>1.0</td>
<td>4.7</td>
<td>1.1 - 8.1</td>
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</table>

Clinical Safety and Efficacy

- PR001 ICM administration well-tolerated; no AEs reported
- Patient clinically stable; no apparent worsening of the patient’s neurological symptoms since PR001 administration
- Follow-up clinical assessments are planned
PROPEL PD-GBA Phase 1/2 trial: early patient data

Background

- Two PD-GBA patients enrolled in PROPEL trial: one administered PR001; one received sham procedure
- Patient who received PR001 (1.4x10^{14} vg) also diagnosed with Gaucher disease (GBA1 mutations in both chromosomal copies)

Biomarkers

- CSF GCase enzyme activity increased from undetectable at baseline to normal level at ~Month 3

<table>
<thead>
<tr>
<th>GCase activity in CSF (μmol/L/d)</th>
<th>Day 0</th>
<th>~Month 3</th>
<th>Normal range (adult)</th>
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</thead>
<tbody>
<tr>
<td>Undetectable</td>
<td></td>
<td>3.0</td>
<td>1.1 - 8.1</td>
</tr>
</tbody>
</table>

Clinical Safety and Efficacy

- PR001 ICM administration well-tolerated
- Patient did not complete protocol-specified immunosuppression regimen due to steroid intolerance
- Three months following PR001 administration, patient experienced SAEs presumed to be AAV9-immune mediated
  - SAEs were worsening of the patient’s underlying hallucinations and orthostasis, and encephalitis (worsening of mental status and evidence of CSF inflammation)
  - MRI (with contrast) of brain and spinal cord showed no changes from baseline
- Patient’s condition responded to additional immunosuppressive treatment; SAEs have markedly resolved
• Amendment implemented to mitigate risk of AAV9-mediated immune reactions:
  o Immunosuppression regimen modified by adding sirolimus and reducing corticosteroid dose and duration
  o Trial converted to open-label design and sample size reduced to N=12 with removal of sham procedure

• Based on updated analytical methods with increased precision, dose levels of PR001 established to be:
  o $1.4 \times 10^{14}$ vg in low dose
  o $2.8 \times 10^{14}$ vg in high dose

✓ Protocol amendment endorsed by IDMC, discussed with and submitted to FDA
✓ Patient enrollment expected to continue in 2H 2020
✓ Next biomarker and safety analysis on subset of patients by mid-2021
Revised PR001 PD-GBA Phase 1/2 trial
Open label, ascending dose

- Single or biallelic GBA1 mutations
- Moderate to severe Parkinson’s disease
- Stable background PD medication

PR001 Low Dose (N=6)
PR001 High Dose (N=6)

- Safety and tolerability
- Key biomarkers: GCase, GluCer, GluSph (CSF and blood)
- Additional biomarkers: α-Synuclein, NfL, DAT SPECT, MRI
- Efficacy: MDS-UPDRS, cognition, ADLs

ICM: intra-cisterna magna; MDS-UPDRS: Movement Disorders Society Unified Parkinson's disease Rating Scale; ADLs: Activities of Daily Living; NfL: neurofilament light; DAT: Dopamine transporter; SPECT: single photon emission computed tomography
Goal of PR001 treatment is to deliver the *GBA1* gene to restore GCase activity in PD-GBA patients.

Pre-PR001 Treatment

~50% of healthy GCase

Goal of PR001 Treatment

~75% of healthy GCase

Upside of PR001 Treatment

100% of healthy GCase

Source: Liu et al., Annals of Neurology 2016; Thaler et al., Parkinsonism and Related Disorders 2017
PR001 Type 2 Gaucher disease Phase 1/2 trial
*Open label*

**Type 2 Gaucher Patients**
- Infants 0-24 months old
- Biallelic GBA1 mutations
- Neurological signs & symptoms consistent with Type 2 Gaucher disease
- Stable SoC background medications

**PR001 (Open label, N=15)**
- Safety and tolerability
- Key biomarkers: GCase, GluCer, GluSph (CSF and blood)
- Time to clinical event (e.g., tracheostomy, PEG placement, death)
- Efficacy: behavior, cognition, gross motor, function, QoL
PR006 FTD-GRN Phase 1/2 trial
*Open label, ascending dose*

**FTD-GRN Patients**
- 30-80 years old
- Single pathogenic GRN mutation
- Symptomatic disease stage
- Stable background medications

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**Single ICM injection**

**2 month biomarker readout**

**12 month clinical readout**

**5-year safety and clinical follow-up**

- PR006 Low Dose (N=5)
- PR006 Mid Dose (N=5)
- PR006 High Dose (N=5)

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**Safety and tolerability**

**Key biomarkers:** progranulin, NfL, volumetric MRI

**Efficacy:** CDR plus NACC FTLD; measures of behavior, cognition, language, function, QoL
Financial Update

Brett Kaplan, M.D., Chief Financial Officer
Second quarter 2020 financial update

- Cash (inclusive investment purchases) of $131.2 million as of June 30, 2020, as compared to $149.6 million as of March 31, 2020
- Cash runway remains into first half of 2022

- $12.9 million for second quarter of 2020, compared to $12.0 million for second quarter of 2019

- $9.2 million for second quarter of 2020, compared to $3.7 million for second quarter of 2019

- Filed prospectus supplement for $75M ATM offering
Q&A