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Company Overview

- EB-101 breakthrough therapy in pivotal Phase 3 study for RDEB, supported by clinical evidence of long-term wound healing and pain reduction
- AAV gene therapies with signs of clinical benefit in MPS IIIA and clear biologic effect in MPS IIIB in Phase 1/2 trials
- Novel AIM AAV capsid platform: in vivo proof of concept data for efficient delivery in NHP as well as tropism for other organs, including CNS
- State-of-the-art cGMP manufacturing for clinical and commercial grade gene and cell therapy products
EB-101 Restores Normal Functional Collagen VII to Keratinocytes and Their Progenitors

- Personalized Treatment
- Biopsy to Patient-Ready in ~4 Weeks
- 2 Skin Biopsies = 6 Sheets = 240 cm²

EB-101: Ex-Vivo Autologous Gene-Corrected Breakthrough Therapy for Large, Chronic Wounds
Phase 1/2a: Durable Healing and Pain Reduction Following EB-101 Treatment

% of Wounds with ≥ 50% or ≥ 75% Healing

- Wound area healed, average per patient: ≥ 130 cm² and ≥ 120 cm² (up to 157 cm²) at 3- and 6-months, respectively
- EB-101 treatment of chronic, large RDEB wounds resulted in considerable and durable reduction in wound burden
- Reduced wound burden associated with symptomatic relief, including pain

Overall Wound Pain: Relief Associated with EB-101 Treatment

<table>
<thead>
<tr>
<th>% Painful Wounds (n/N)</th>
<th>Pre-application</th>
<th>3 months</th>
<th>6 months</th>
<th>12 months</th>
<th>24 months</th>
<th>36 months</th>
<th>48 months</th>
<th>60 months</th>
<th>72 months</th>
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</thead>
<tbody>
<tr>
<td></td>
<td>53.0% (20/38)</td>
<td>0.0% (0/38)</td>
<td>15.8% (6/38)</td>
<td>5.3% (2/38)</td>
<td>7.9% (3/38)</td>
<td>0.0% (0/26)</td>
<td>0.0% (0/15)</td>
<td>0.0% (0/15)</td>
<td>0.0% (0/5)</td>
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EB-101 Pivotal Phase 3 VIITAL™ Study

**SCREENING PERIOD**
- Clinic Screening Visit: Day -28 (+/- 1 day)
- Home Nurse Visit: Day -14 to -7
- Clinic Baseline Visit: Day -1

**TREATMENT**
- In-patient: Day 0 – 7 (+/- 3 days)

**FOLLOW-UP PERIOD**
- Clinic Visit or Phone Call: Day 14 (+/- 3 days)
- Home Nurse Visits: Weeks 4, 9, and 18 (+/- 14 days)
- Clinic Visits: Weeks 6, 12 (+/- 7 days), 24 (+/- 14 days)

**Registration Study Underway**
- Open-label, interventional study of one-time surgical application of EB-101 compared to matched untreated wounds
- ~35 treated large, chronic wounds across 10-15 RDEB patients

**Co-Pimary Endpoints**
- Proportion of chronic wounds with healing ≥ 50% from baseline in treated vs untreated wounds at Week 24
- Pain reduction at dressing changes assessed by mean differences in scores of Wong-Baker FACES scale between treated and untreated wounds at Week 24
ABO-102 and ABO-101: AAV Gene Therapies for MPS IIIA & MPS IIIB Mechanism of Action

Adeno-associated Virus 9 (AAV9) Vector + Functional Gene: SGSH or NAGLU = AAV9 Vectors Cross the BBB

IV

Vector releases functional gene in cells

AAV9 Vectors Cross the BBB

Neighboring cells take up SGSH or NAGLU

SGSH or NAGLU enzyme is secreted from transduced cells
Transpher A: Sustained Reduction in CNS and Systemic Biomarkers

**CSF HS**

- Cohort 1 (5.0E12 vg/kg)
- Cohort 2 (1.0E13 vg/kg)
- Cohort 3 (3.0E13 vg/kg)

* P < .0001
** P = .0014

**CSF GM2**

* P = .0113

**CSF GM3**

* P = .0163

**Plasma HS**

* P = .042

**Urinary GAGs**

* P = .001

Paired t test
Natural-History Disease Progression Model for MPS IIIA

Black solid line: Typical developmental pattern for children with MPS IIIA per Natural History data.

Gray shaded area: 95% confidence interval, incorporating variability from patient-to-patient differences and measurement error.

Black dashed line: Expected cognitive development for children without disease (DQ100) or with 60% of normal (DQ60). Development Quotient (DQ): ratio between age equivalent and actual age (chronological).

Age equivalent: Functional age of the child, calculated by comparison with the age at which a child in the normal population develops similar skills.

References:
- Berman et al, J Inherit Metab Dis 2014
- Shapiro et al, J Pediatrics, 2016
- Wijburg et al, WORLD Symposium, 2018
Cohort 3: Younger Children Track Along Normal Development Range

Cohort 3 patients (3x10^{13} vg/kg)

DQ=100

DQ=60
Cohort 3: Younger Children Track Along Normal Development Range
Transpher A: MRI Results
Increase in grey matter, corpus callosum and amygdala volumes with ABO-102 treatment compared to Natural History

Child with MPS-III A from ABT-001 at 38 months of age (baseline)

Child with MPS-III A from ABT-001 at 42 months of age (30 months post-treatment)
Transpher A: MRI Results - Increase in cortical grey matter, corpus callosum and amygdala volumes with ABO-102 treatment compared to Natural History

Completed Successful Type B Meeting with FDA

Aligned with FDA that current single-arm Transpher A study will serve as pivotal study for ABO-102 and potentially support a BLA, depending on data

• Could have evaluable data set in 2022 if see similar treatment effects in recently dosed children in Cohort 3 as those already presented

Aligned with FDA on definition of primary endpoint

• Neurocognitive assessment using raw score from Bayley Scales of Infant and Toddler Development (BSITD-3) up to 42 months development age (BSITD-3 maximum limit), followed by assessment using the Kauffman Assessment Battery for Children (KABC-2)
Consistent Safety and Clinical Benefit in Phase 1/2 Studies with ABO-102 and ABO-101

**ABO-102 was well-tolerated**
- No deaths
- No infusion-related adverse events
- No drug-related SAEs
- No clinically significant AEs, 0.3-59.7 months (n=21)

**Evidence of clinical benefit**
- Preservation of neurocognitive development in the 3 young patients treated <30 months of age in Cohort 3 (30-36 mos. follow-up, 43, 48 and 64 months of chronological age)
- A child treated at 1 year of age continues to track on the DQ100 line 2.5 years after treatment, showing normal development
- Sustained, dose-related and statistically significant reductions in disease-specific biomarkers 2 years post-administration

**ABO-101 was well-tolerated**
- No deaths
- No infusion-related adverse events
- One drug-related SAE
- No clinically significant AEs 6-40 months (n=11)

**Evidence of potent biologic effect**
- Decreased CSF HS levels sustained up to 24 mos.
- Dose-dependent normalization of plasma NAGLU activity up to Month 6 in Cohort 3
- Dose-dependent reduction in plasma and urine HS and GAGs
- Reduction in liver volume
- Cognitive evaluation requires longer follow-up in Cohorts 2 and 3
Fully-Integrated, Independent, and Scalable cGMP Manufacturing

Control of supply chain, including timelines and cost

- 40,000 sq. ft multi-purpose facility in Cleveland
- Scalable cGMP capacity
- State-of-the-art laboratories to support CMC process and analytical development
- Experienced and trained CMC staff in Quality, Validation, Process Development, and Assay Development

Clinical and commercial grade manufacturing capability

- EB-101 Phase 3 manufacture ongoing; GMP retroviral supernatant manufacturing capability
- Scalable capacity to support EB-101 commercial launch
- 200L AAV manufacturing GMP upstream capacity; process development for 500L in the works
- Supportive of development programs, capable of clinical and commercial AAV production
### Anticipated Milestones

#### EB-101
- ✔ Successful Type B meeting with alignment on co-primary endpoints for Phase 3 VIITAL™ study in Q1 2021
- ❑ Complete enrollment in VIITAL™ study in Q1 2022
- ❑ Top-line results from VIITAL™ study in Q3 2022, followed by BLA filing

#### ABO-102 and ABO-101
- ✔ Met target enrollment in ABO-102 MPS IIIA in Q4 2020
- ✔ Updated neurocognitive data in MPS IIIA and clinical data in MPS IIIB at WORLD in Q1 2021
- ✔ Successful Type B meeting with alignment on definition of primary endpoint for ABO-102 MPS IIIA study in Q2 2021
- ❑ Top-line results for ABO-102 MPS IIIA study between Q4 2022 and Q2 2023
- ❑ Make first lot of Abeona-produced ABO-102 clinical grade product
- ❑ 2-year neurocognitive data from ABO-101 MPS IIIB study in H2 2022

#### Preclinical
- ✔ Complete NHP studies validating AIM capsid library in intraocular administration
- ❑ Advancing multiple preclinical programs for undisclosed eye indications, anticipate animal POC data by mid-2022
Phase 1/2a: EB-101 Treatment of Chronic, Large RDEB Wounds

Baseline

9 months

Treated wound

Untreated wound

Green line shows collagen expression post-treatment
## Phase 1/2a: EB-101 Demonstrated Durable Efficacy

<table>
<thead>
<tr>
<th>Participant</th>
<th>Site</th>
<th>Location</th>
<th>Wound Size (cm²)</th>
<th>3 months</th>
<th>6 months</th>
<th>12 months</th>
<th>2 years</th>
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- **Enrolled patients with large wounds not eligible for clinical trials with other gene therapies in development**
- **Wounds up to 400 cm² and open 3-20 years**

### RDEB Wound Healing

- >75%
- 50%-75%
- <50%

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[Image of a chart showing participant data]

**Abeona Therapeutics**