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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
Making Progress on Our Strategic Priorities

Bolstering Operational Experience
- New Head of Research & Clinical Development
- Added relevant regulatory BLA experience
- Appointed four new Board members with life sciences operations experience

Delivering Operational Excellence
- Completed two successful Type B meetings with FDA
- Activated second clinical site in pivotal Phase 3 study in RDEB
- Reported positive neurocognitive development results and MRI data in MPS IIIA
- Focused on delivering two pivotal data packages in 2022

Advancing Preclinical Pipeline
- Focused on advancing preclinical eye programs toward clinic
- Reported results of two NHP studies
- Moving to POC studies in H2’21 followed by tox studies in 2022
EB-101: Ex-Vivo Autologous Gene-Corrected Breakthrough Therapy for Large, Chronic Wounds

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
Phase 1/2a: Durable Healing and Pain Reduction Following EB-101 Treatment

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

- ≥50% healing
- ≥75% healing

Overall Wound Pain: Relief Associated with EB-101 Treatment

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

- 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
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
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 to 15 RDEB patients

Co-Primary 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 = IV

AAV9 Vectors Cross the BBB

Vector releases functional gene in cells

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

- *P < .0001
- **P = .0014

Paired t test

CSF GM2

- *P = .0113

Paired t test

CSF GM3

- *P = .0163

Paired t test

Plasma HS

- *P = .042

Paired t test

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

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Cohort 3: Younger Children Track Along Normal Development Range

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

DQ=100
DQ=60

Age (months)

Cognitive age equivalent
Cohort 3: Younger Children Track Along Normal Development Range

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

Age (months)

Cognitive age equivalent

DQ=100

DQ=60
Transphere 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 VIITALù study in January
- **✓** Complete enrollment in VIITALù study in 2021
- **✓** Topline results from VIITALù study in mid-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
- **✓** Requested meeting with FDA in late-Q1 2021 to discuss MPS IIIA data and potential BLA submission path in MPS IIIA
- **✓** Additional follow-up visits and neurocognitive assessments of patients with MPS IIIA treated in high dose cohort 3
- **✓** Make first lot of Abeona-produced ABO-102 clinical grade product
- **✓** Complete enrollment in ABO-101 MPS IIIB study

**Preclinical**

- **✓** Complete NHP studies validating AIM capsid library in intraocular administration
- **✓** Execute POC studies in H2 2021 for undisclosed eye indications
- **✓** Initiate toxicology/IND-enabling studies in 2022
Phase 1/2a: EB-101 Treatment of Chronic, Large RDEB Wounds

Baseline

9 months

Treated wound

Treated wound

Untreated wound

Green line shows collagen expression post-treatment