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• EB-101 breakthrough therapy in pivotal Phase 3 study for RDEB, supported by evidence of multi-year wound healing data

• AAV gene therapies with early 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 intravitreous and subretinal 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

• $104M in cash, cash equivalents, receivables and marketable securities (Sept 30, 2020)
Robust Pipeline

EB-101
RECESSIVE DYSTROPHIC EPIDERMOLYSIS BULLOSA (RDEB)

ABO-102
SANFILIPPO SYNDROME TYPE A (MPS IIIA)

ABO-101
SANFILIPPO SYNDROME TYPE B (MPS IIIB)

ABO-201
JUVENILE BATTEN DISEASE (CLN3)

AIM™ VECTORS
UNDISCLOSED TARGETS

ABO-401
CYSTIC FIBROSIS

ABO-50X
RETINAL DISEASES

Regulatory Designations

- REGENERATIVE MEDICINE ADVANCED THERAPY (FDA)
- PRIORITY MEDICINES (EMA)
- BREAKTHROUGH THERAPY (FDA)
- FAST TRACK (FDA)
- RARE PEDIATRIC DISEASE (FDA)
- ORPHAN DRUG (FDA)
- ORPHAN DRUG (EMA)
EB-101: Gene-Corrected Cell Therapy for RDEB
Epidermolysis Bullosa (EB): Devastating Inherited Connective Tissue Disorder

Recessive Dystrophic Epidermolysis Bullosa (RDEB):

- Most severe form of EB
- Primarily characterized by skin blisters and erosions
- Caused by mutations in COL7A1 gene, which encodes type VII collagen
- Est. 2,500 U.S. patients

Up to 80% of patient’s body covered in wounds, leading to:

- Severe pain and widespread scarring
- Debilitating and life-threatening systemic complications
- Up to 90% of RDEB patients are at risk for squamous cell carcinoma (SCC)

50% of generalized severe patients die before 35 75% die before 40
Recognizing the Full Burden of RDEB

**Clinical**

Large, chronic wounds comprise the main clinical burden in RDEB and are correlated with pain.

Up to 90% of RDEB patients are at risk of developing SCC.

**Economic**

Annual wound dressing cost per patient is up to $245,000.

U.S. families characterized the economic impact of managing RDEB as “high” or “severe.”

**Humanistic**

Many patients have anxiety and depression.

67% of divorced parents reported RDEB as a major/primary factor.
Recurrent and Chronic Wounds Have Distinct Time Courses

Recurrent Wounds Over Time (N=25)

Chronic Wounds Over Time (N=25)

Natural history of chronic wounds in patients with recessive dystrophic epidermolysis bullosa; Solis, D. et al.; Journal of Investigative Dermatology, Volume 137, Issue 5, S37
Large, Chronic Open Wounds Cause Greatest Pain and Itch
Sequentially Photographed Wounds: N=25 patients, 62 wounds

- Wong Baker FACES™ Pain Rating Scale
  - Pain: Mean: 5.0
  - Itch: Mean: 2.3

- Itch Man Scale
  - Mean: 2.4

- Size and Duration Distribution
  - Recurrent: ≤19 cm²: 64%, 20-39 cm²: 21%, ≥40 cm²: 15%, Mean Size: 26 cm², Mean Duration: 5 years
  - Chronic Open: ≤19 cm²: 27%, 20-39 cm²: 20%, ≥40 cm²: 53%, Mean Size: 118 cm², Mean Duration: 7 years

Natural history of wounds in patients with recessive dystrophic epidermolysis bullosa; Teng et al., Abstract #251; Society of Investigational Dermatology Annual Meeting, 2019
EB-101: Ex-Vivo Autologous Gene Corrected Cell Therapy

EB-101 restores functional collagen VII to patient’s own keratinocytes and their progenitors

- Personalized treatment
- Biopsy to patient-ready in ~4 weeks
- 2 skin biopsies = 6 sheets = 240 cm²

EB-101 is transplanted onto patients' wounds in as little as 26 days.

Keratinocyte cells expanded and prepared for the next step.

10-12 days of continued cell maturation and growth.

Collagen Type VII gene-corrected keratinocytes expanded (3-5 days)

Keratinocyte cells transduced into functional Col7A1 gene transfer

Functional Type VII collagen transduced into keratinocyte cells; cell maturation leads to gene-corrected type VII collagen expression.

Two 8mm skin biopsies shipped to Abeona, produced into 6-8 ~40 cm² sheets.

Keratinocyte cells expanded and harvested as 5.5x7.5 cm² sheets.

PERSONALIZED TREATMENT

BIOPSY TO PATIENT-READY IN ~4 WEEKS

2 SKIN BIOPSIES = 6 SHEETS = 240 CM²
# EB-101 Pivotal Phase 3 VIITAL™ Study

### Study Design
- Multi-center, randomized trial led by Stanford University
- 10-15 RDEB patients, with approx. 30 chronic wound sites treated in total
- Follow-up visits 1-6 months, then in a long-term follow-up protocol until year 5

### Study Endpoints
- Proportion of wounds with >50% healing, comparing treated with untreated wound sites on the same patient
- Patient’s global impression of change in pain from baseline
- Patient-reported outcomes assessing pain during:
  - Dressing changes
  - Pain impact
  - Physical function

**Enrollment completion expected in first half of 2021**
**VIITAL™ Study Supported by EB-101 Phase 1/2a Study for RDEB**

### Study Description
- A Phase 1/2a Single Center Trial of Gene Transfer for Recessive Dystrophic Epidermolysis Bullosa (RDEB) using EB-101 for autologous tissue transplantation

### Study Design
- Open-label, interventional study
- Seven patients with RDEB (ages 18 to 45 years)
- Follow-up visits at 1-12 months post treatment; yearly thereafter until year 5

- Phase 1/2a study addressed wounds of increasing severity and complexity
- Study participants had challenging wounds representative of those most troublesome for the RDEB population
- Learnings from program provided essential guidance for future wound treatment, de-risked Phase 3 study
- Potential to address most wounds, regardless of size or duration
EB-101 Treated Large, Chronic Wounds in a Phase 1/2 Study

Baseline 9 months

Durable wound healing
- Healing lasted for 3+ to 5+ years after treatment
- Wound healing of large wounds was associated with no pain

Favorable safety profile 5 years after treatment
- Longest safety follow up of any gene therapy in development for RDEB

Evidence of treating the underlying cause
- Continuous Type VII collagen expression seen 2+ years after treatment
EB-101 Restored Collagen VII that Forms Functional Anchoring Fibrils
Phase 1/2 Study Results

Green line shows collagen expression post-treatment
### EB-101 Demonstrated Durable Efficacy

**Phase 1/2 Study Results**

<table>
<thead>
<tr>
<th>Participant</th>
<th>Site</th>
<th>Location</th>
<th>Wound Area (cm²)</th>
<th>3 months</th>
<th>6 months</th>
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**Notes:**

- 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
Proportion of Wounds with ≥ 50% and ≥75% Healing
Phase 1/2 Study Results

Average wound area healed per patient was 130 cm² and 120 cm² (up to 157 cm²) at 3- and 6-months, respectively

(Note: healed area was calculated based on minimum % healing per wound site, e.g. 50% used for wound sites that healed ≥ 50%)

Eichstadt et al. JCI Insight 2019
ABO-102* and ABO-101 Clinical Programs for MPS III
Sanfilippo Syndrome (MPS III)

Inherited monogenic disorders causing lysosomal enzyme deficiency

- Global incidence varies by regions and it is estimated 0.17-2.35 per 100,000 births*
- Two most common forms categorized by deficient enzymes:
  - MPS IIIA (SGSH), MPS IIIB (NAGLU)
- Abnormal accumulation of glycosaminoglycans (GAGs; heparan sulfate (HS))
- Language and cognitive decline, behavioral abnormalities, seizures, sleep disturbances
- Most children with MPS III have only ~60% of typical cognitive capacity by age 3 years
- 70% of children with MPS III do not survive to age 18 years

No approved treatments available

*Zelei et al. 2018. Orphanet Journal of Rare Diseases
ABO-102 and ABO-101: AAV Gene Therapies for MPS IIIA and MPS IIIB

Mechanism of Action

AAV9 Vectors Cross the BBB

Vector releases functional gene in cells

Functional Gene: SGSH or NAGLU

Neighboring cells take up SGSH or NAGLU

SGSH or NAGLU enzyme is secreted from transduced cells
## Phase 1/2 Open-label, Dose-escalation Clinical Trials in MPS IIIA and IIIB

### Study Design

<table>
<thead>
<tr>
<th>transpher A study® (ABT-001)</th>
<th>transpher B study® (ABT-002)</th>
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<tbody>
<tr>
<td><strong>Study Description</strong></td>
<td><strong>Study Description</strong></td>
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<tr>
<td>• Single IV dose of ABO-102 (scAAV9.U1.hSGSH) for MPS IIIA</td>
<td>• Single IV dose of ABO-101 (rAAV9.CMV.hNAGLU) for MPS IIIB</td>
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<tr>
<td><strong>Enrollment Status</strong></td>
<td><strong>Enrollment Status</strong></td>
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<td>✓ Cohort 1: 5 x 10^{12} vg/kg (n=3)</td>
<td>✓ Cohort 1: 2 x 10^{13} vg/kg (n=2*)</td>
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<tr>
<td>✓ Cohort 2: 1 x 10^{13} vg/kg (n=3)</td>
<td>✓ Cohort 2: 5 x 10^{13} vg/kg (n=5)</td>
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<td>• Cohort 3: 3 x 10^{13} vg/kg (n=11, up to 16)</td>
<td>• Cohort 3: 1 x 10^{14} vg/kg (n=2, up to 8)</td>
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<td><strong>Primary Endpoints</strong></td>
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<td>• Neurodevelopmental scores post treatment vs. untreated patients enrolled in natural history studies based on Mullen Scales of Early Learning (MSEL)</td>
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<td>• Product safety</td>
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<td><strong>Secondary Endpoints</strong></td>
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<tr>
<td>• Behavior evaluations, quality of life, enzyme activity, heparan sulfate levels, and brain and liver volume</td>
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</table>

*Enrollment completion expected in the first quarter of 2021*

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*Clinical study protocol states 3 subjects in Cohort 1; however, due to exceptional circumstances and following robust safety profile and positive review from DSMB, trial was cleared in Europe to advance to Cohort 2 dose*
Neurocognitive Development of Youngest Patients Preserved 18-24 months Post Treatment compared with Natural History

Cognitive Natural History Data (92 children)

- Black Solid Line: Typical developmental pattern for children with MPS IIIA (natural history)
- Gray Shaded Area: Variability from patient-to-patient differences and measurement error
- Black Dashed Line: Expected development for children without disease

Shapiro et al, J Pediatrics, 2016
Burhman et al, J Inherit Metab Dis 2014
Wijburg et al, WORLD Symposium, 2018
Post-treatment Improvement in Disease-Specific CNS Biomarkers in MPS IIIA and MPS IIIB

ABO-101 showed improvement in CSF heparan sulfate

ABO-102 showed rapid, dose-dependent, and sustained reduction in CSF heparan sulfate
Post-treatment Reduction in Liver Volume in MPS IIIA and IIIB

ABO-102 showed durable, dose-dependent reduction in liver volume post treatment

ABO-101 showed early signs of reduction in liver volume post treatment

NHS data: Truxal et al, 2016, Mol Genet Metab
Consistent Safety and Clinical Benefit in Phase 1/2 Studies with ABO-102 and ABO-101

A BO-102 was well-tolerated
• No infusion reactions
• No treatment-related SAEs
• No clinically significant AEs 0.5-50 months (n=16)

Preliminary evidence of clinical benefit
• Preservation of neurocognitive development in the 3 young patients treated <30 months of age in Cohort 3 (18-24 mos. of follow-up)
• Rapid and sustained, dose-related reduction in disease-specific biomarkers (e.g. heparan sulfate in cerebrospinal fluid and liver volume)

A BO-101 was well-tolerated
• No infusion reactions
• No treatment-related SAEs
• No clinically significant AEs 3-31 months (n=9)

Clear biologic effect
• Decreased CSF HS levels (up to 12 mos.)
• Reduction in plasma and urine HS and GAGs
• Reduction in liver volume
• Neurological assessments pending resumption of visits post-COVID

As of July 2020
In-House GMP Manufacturing
Fully-Integrated, Independent, and Scalable cGMP Manufacturing

Control of supply chain, including timelines and cost
• 26,000 sq. ft multi-purpose facility in Cleveland
• Scalable cGMP capacity
• State-of-the-art laboratories to support CMC development for process and analytics
• Experienced and trained staff in Quality, Validation, Process Development, and Assay Development

Clinical and commercial grade manufacturing capability
• EB-101 Phase 3 manufacture ongoing; retrovirus manufacturing in late stage development
• Scalable capacity to support EB-101 commercial launch
• 200L AAV manufacturing GMP upstream capacity; process development for 500L underway
• Supportive of development programs, capable of clinical and commercial AAV production
Anticipated Milestones

**EB-101**
- Complete enrollment in pivotal Phase 3 VIITAL™ study in H1 2021, depending upon impact from COVID-19 pandemic
- Topline results from VIITAL™ study in late-2021

**ABO-102 and ABO-101 (AAV-based Gene Therapies)**
- Complete enrollment in ABO-102 MPS IIIA and ABO-101 MPS IIIB studies in Q1 2021
- Updated neurocognitive data from MPS IIIA and clinical data from MPS IIIB studies in Q1 2021
- Update on U.S. regulatory pathway for ABO-102 in MPS IIIA
A Fully-Integrated Gene & Cell Therapy Company
Focused on Rare Diseases With No Approved Treatments

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