AISLINN
Living with MPS IIIA

Corporate Presentation

February 2019
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Abeona: building a fully-Integrated gene & cell therapy company

- Pivotal Phase 3 preparation underway in severe form of Epidermolysis Bullosa (EB)
- Ongoing Phase 1/2 clinical trials in MPS IIIA and MPS IIIB
- Additional programs approaching the clinic in Batten disease (CLN1 and CLN3)
- Next generation AIM™ AAV vector platform with *in vivo* proof of concept data in cystic fibrosis, retinal disorders, Pompe’s and Fabry’s
- GMP facility established in Cleveland
  - Seamless transition from concept-to-commercial across multiple modalities
  - Quality systems and staff in place to support EB and AAV programs
Robust Pipeline

<table>
<thead>
<tr>
<th>Pipeline</th>
<th>Description</th>
<th>Preclinical</th>
<th>Phase I/II</th>
<th>Phase III</th>
<th>Designations</th>
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<tbody>
<tr>
<td>EB-101</td>
<td>RECESSIVE DYSTROPHIC EPIDERMOLYSIS BULLOSA (RDEB)</td>
<td><img src="#" alt="Preclinical" /></td>
<td><img src="#" alt="Phase I/II" /></td>
<td><img src="#" alt="Phase III" /></td>
<td>- Regenerative Medicine Advanced Therapy Designation (FDA) - Breakthrough Therapy Designation (FDA) - Rare Pediatric Disease Designation (FDA) - Orphan Drug Designation (FDA) - Orphan Drug Designation (EMA)</td>
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<tr>
<td>ABO-102</td>
<td>SANFILIPPO SYNDROME TYPE A (MPS II A)</td>
<td><img src="#" alt="Preclinical" /></td>
<td><img src="#" alt="Phase I/II" /></td>
<td><img src="#" alt="Phase III" /></td>
<td>- Regenerative Medicine Advanced Therapy Designation (FDA) - Fast Track Designation (FDA) - Rare Pediatric Disease Designation (FDA) - Orphan Drug Designation (FDA) - Orphan Drug Designation (EMA)</td>
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<tr>
<td>ABO-101</td>
<td>SANFILIPPO SYNDROME TYPE B (MPS II B)</td>
<td><img src="#" alt="Preclinical" /></td>
<td><img src="#" alt="Phase I/II" /></td>
<td><img src="#" alt="Phase III" /></td>
<td>- Orphan Drug Designation (FDA) - Orphan Drug Designation (EMA) - Rare Pediatric Disease Designation (FDA)</td>
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<td>ABO-202</td>
<td>INFANTILE BATTEN DISEASE (CLN1)</td>
<td><img src="#" alt="Preclinical" /></td>
<td><img src="#" alt="Phase I/II" /></td>
<td><img src="#" alt="Phase III" /></td>
<td>- Orphan Drug Designation (FDA) - Orphan Drug Designation (EMA) - Rare Pediatric Disease Designation (FDA)</td>
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<td>ABO-201</td>
<td>JUVENILE BATTEN DISEASE (CLN3)</td>
<td><img src="#" alt="Preclinical" /></td>
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<td><img src="#" alt="Phase III" /></td>
<td>- Orphan Drug Designation (FDA) - Orphan Drug Designation (EMA)</td>
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<td>ABO-401</td>
<td>CYSTIC FIBROSIS (CF)</td>
<td><img src="#" alt="Preclinical" /></td>
<td><img src="#" alt="Phase I/II" /></td>
<td><img src="#" alt="Phase III" /></td>
<td>- Orphan Drug Designation (EMA)</td>
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<td>ABO-50X</td>
<td>RETINAL DISEASES</td>
<td><img src="#" alt="Preclinical" /></td>
<td><img src="#" alt="Phase I/II" /></td>
<td><img src="#" alt="Phase III" /></td>
<td>- Orphan Drug Designation (EMA)</td>
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</table>

UNDISCLOSED TARGETS

AIM™ VECTORS
NEXT GENERATION AAV PRODUCTS
RDEB Clinical Program

EB-101

- Orphan Drug Designation (FDA)
- Orphan Drug Designation (EU)
- Rare Pediatric Disease Designation (FDA)
- Breakthrough Therapy Designation (FDA)
- Regenerative Medicine Advanced Therapy Designation (FDA)
Group of devastating inherited connective tissue diseases; characterized by skin blisters and erosions

- Recessive Dystrophic Epidermolysis Bullosa (RDEB): Absence of the COL7A1 gene (encodes type VII collagen to anchor skin to the underlying stroma)

No approved therapy

Phase 1/2 clinical trial completed
- Conducted at Stanford University School of Medicine
- Primary endpoint: Safety, wound healing vs. baseline
- Secondary endpoint: Expression of type VII collagen, pain, itching, quality-of-life

Preparing for pivotal Phase 3
Supportive Natural History Study Helps Shape Clinical Program

128 RDEB Subjects - Clinical Trial Readiness

Multi-year Enrollment complete:
• 1,436 wounds (1,041 recurrent wounds/395 chronic open wounds)

100% of patients reported a history of either chronic open wounds or recurrent wounds with no healing >12 weeks

Average duration of untreated chronic wounds >7 years

Allograft application prior to Natural History Study demonstrated lack of efficacy in healing RDEB wounds
• 13 patients (15 chronic wounds) were treated with an allograft product, including Apligraf® and Dermagraft®
• Only 7% (1/15 treated wounds) remained healed after 12 weeks, and 0% (0/15 treated wounds) remained healed after 24 weeks
EB-101: Ex-Vivo Autologous Gene-Corrected Cell Therapy

1. Biopsy
2. Keratinocyte Isolation (5-10 days)
3. Expansion (3-5 days)
4. Maturation (10-12 days)
5. Transplantation

EB-101: 26-days post growth

EB-101: ready for patients
Gene therapy skin grafts restore collagen 7 that forms functional anchoring fibrils

<table>
<thead>
<tr>
<th>Site</th>
<th>Location</th>
<th>Descript</th>
<th>Estimated duration</th>
<th>3m</th>
<th>6m</th>
<th>12m</th>
<th>24m</th>
</tr>
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<tbody>
<tr>
<td>A</td>
<td>R lateral hand</td>
<td>Erosion</td>
<td>3-5 yrs</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>B</td>
<td>R medial hand</td>
<td>Scar tissue</td>
<td>3-5 yrs</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>C</td>
<td>L ventral foot</td>
<td>Erosion and scar</td>
<td>3-5 yrs</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>D</td>
<td>L hand</td>
<td>Scar tissue</td>
<td>3-5 yrs</td>
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<td>3-5 yrs</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Z</td>
<td>L ventral foot</td>
<td>Induced wound</td>
<td>New</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

- Green: ≥75% healed
- Yellow: 50-70% healed
- White: < 50% healed
EB-101 significantly improved patient-reported outcomes

<table>
<thead>
<tr>
<th></th>
<th>Pre-Grafting</th>
<th>3 Month</th>
<th>6 Month</th>
<th>9 Month</th>
<th>12 Month</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Pain at wound site (% reported yes)</strong></td>
<td>58%</td>
<td>0%</td>
<td>17%</td>
<td>20%</td>
<td>0%</td>
</tr>
<tr>
<td><strong>Itch at wound site (% reported yes)</strong></td>
<td>67%</td>
<td>5%</td>
<td>17%</td>
<td>50%</td>
<td>25%</td>
</tr>
<tr>
<td><strong>Lack of durability at wound site (% reported yes)</strong></td>
<td>90%</td>
<td>0%</td>
<td>0%</td>
<td>0%</td>
<td>0%</td>
</tr>
<tr>
<td><strong>Ease of blistering at wound site (% reported yes)</strong></td>
<td>83%</td>
<td>0%</td>
<td>0%</td>
<td>0%</td>
<td>0%</td>
</tr>
</tbody>
</table>
EB-101 summary and next steps

Successful Phase 1/2
• Favorable safety profile with no product-related SAEs to date
• Significant and durable wound healing, with up to 5 years of follow-up
• Continuous type VII collagen expression 2+ years post treatment

Established GMP manufacturing capability at Abeona
• Manufacture both clinical and commercial product in Cleveland
• Scalable capacity to support commercial launch

VITAL: Phase 3 Trial
• Regulatory CMC review 1H19
• First patient expected to enroll mid-2019
MPS III Clinical Program

ABO-102  ABO-101

✓ Orphan Drug Designation (FDA)
✓ Orphan Drug Designation (EU)
✓ Rare Pediatric Disease Designation (FDA)
✓ Fast Track Designation* (FDA)
✓ Regenerative Medicine Advanced Therapy Designation* (FDA)

*ABO-102
Inherited monogenic disorders causing lysosomal enzyme deficiency

- Two most common forms categorized by deficient enzymes:
  - MPS IIIA (SGSH), MPS IIIB (NAGLU)
- Abnormal accumulation of glycosaminoglycans (GAGs; heparan sulfate (HS))
- Loss of speech/vision, cognitive decline, behavioral abnormalities, seizures, sleep disturbances
- 70% of children with MPS III do not reach age 18 years

No approved therapy

Estimated incidence of 1 in 70,000 births

Two ongoing global clinical trials

- ABO-102 (AAV-SGSH) for MPS IIIA: USA, EU, Australia clinical sites
- ABO-101 (AAV-NAGLU) for MPS IIIB: USA and EU clinical sites
MPS IIIA Natural History: Cognitive & Developmental Assessments

Shapiro et al. 2016

Truxal, K.V. et al. 2016
A Phase 1/2 Clinical Trial (AAV9-SGSH) for MPS IIIA

2-Year, Open-label, dose-escalation clinical trial

**Intravenous Dosing**
- **Cohort 1**: $5 \times 10^{12}$ vg/kg (n=3)
- **Cohort 2**: $1 \times 10^{13}$ vg/kg (n=3)
- **Cohort 3**: $3 \times 10^{13}$ vg/kg (9-12) 8 subjects treated

**Primary Endpoint**
- Safety

**Secondary Endpoints**
- Cerebrospinal Fluid (CSF) and/or urinary HS and/or GAGs
- CSF and serum SGSH enzyme activity
- Liver, spleen and brain volume by MRI
- Neurocognitive function as measured by Leiter International Performance Scale and the Mullen Scales of Early Learning
- Adaptive functioning, by Vineland Adaptive Behavior Scale (caregiver report)

ClinicalTrials.gov: NCT02716246; Study Sponsor Abeona Therapeutics Inc
Reductions in CSF and Urine Heparan Sulfate

[Graphs showing reductions in CSF HS and Urine Heparan Sulfate with age (in months).]
Reductions in Liver Volume vs Natural History Study

1. (Truxal et al., 2016, Mol. Genet. Metab.)
Mullen Developmental Age: ABT-001 vs. NHS

*DQ100* Approximate typical development trajectory (Eapen et al. BMC Pediatrics 2013)

1. (Truxal et. al., 2016, Mol. Genet. Metab.)
Summary of MPS IIIA ABO-102 Phase 1/2 Study Data

• N=14 as of November 2018
  – Length of follow up in cohort 3: 6 months (N=6 of 8 total), 12 months (N=3 of 8 total)
• Clear dose-response, and sustained improvement in biomarkers (HS CSF, Liver Volume)
• Encouraging neurocognitive signals seen in younger, higher functioning patients in cohort 3
  – Caregiver observations include:
    – Reports of improved attention, interaction with siblings/schoolmates/environment
    – Improved sleep
    – Improved speech in young patients
      • Enrolled at age 2.3 years: “putting adjectives and adverbs into complete sentences”
• Safety: ABO-102 has been well tolerated to date
  – No serious drug related adverse events (n=14 subjects)
  – SGSH ELISpot negative
  – Length of Follow up as of November 2018:
    • Cohort 1: 27-30 months; Cohort 2: 19-21 months; Cohort 3: 1-16 months
A Phase 1/2 Clinical Trial (AAV-NAGLU) for MPS IIIB

2-year, Open-label, dose-escalation global clinical trial

Intravenous Dosing
- **Cohort 1**: $2 \times 10^{13}$ vg/kg (n=3 subjects) 1 patient treated (4.2 y/o) and 1 patient enrolled
- **Cohort 2**: $5 \times 10^{13}$ vg/kg (n=3-6 subjects planned)

Primary Endpoint
- Safety

Secondary Endpoints
- CSF and/or urinary HS and/or GAG
- CSF and serum NAGLU enzyme activity levels
- Liver, spleen and brain volume (MRI)
- Neurocognitive function as measured by Leiter International Performance Scale and the Mullen Scales of Early Learning
- Adaptive functioning, by Vineland Adaptive Behavior Scale (caregiver report)
Reductions in Liver Volume, CSF and Urine Heparan Sulfate

Liver Volume

ABT-002 vs NHS (Nationwide Children's Hospital)

CSF and Urine HS

*Gray lines indicate natural history study patients¹

1. (Truxal et. al., 2016, Mol. Genet. Metab.)
Developmental Age vs. Natural History

1. (Whitley et al., 2018)

*Gray lines indicate natural history study patients¹
Next Generation AIM™ AAV Platform
AIM™: Next Generation AAV Vector Platform

- **AIM™ Vector Platform**: AAV viral vector platform selected to target CNS, lung, skin, muscle, liver and other tissues

- **Key Advantages of AIM™**
  - First generation demonstrated increased gene delivery efficiency to specific tissues
  - Second and third generations have increased tissue tropisms
    - Over 100 capsids under evaluation

- **Potential for redosing previously treated AAV subjects**

- **Cystic Fibrosis and Ocular programs demonstrate proof-of-concept to support pre-clinical studies**
AAV GMP MANUFACTURING
Abeona’s Gene Therapy Manufacturing and Quality Capabilities

Advantages of internal cGMP manufacturing
- Control of supply chain, including timelines and cost
- Internal Abeona quality systems and personnel

Multi-use production platform yields flexibility & risk reduction
- Manufacturing suspension and adherent upstream mammalian and insect platforms
- High quality, high titer material supporting clinical development & commercial scalability

Abeona’s large-scale cGMP capacity and deep expertise
- 26,000 sq. ft facility in Cleveland—stage 1 of 2 completed, stage 2 underway
- State-of-the-art laboratories to support CMC development for process and analytics

Commercial readiness of Abeona’s facility
- Designed for seamless transition from concept-to-commercial
- Conversion of GMP to commercial scalability
Abeona’s Gene Therapy Manufacturing and Quality Capabilities

- FDA Division of Manufacturing and Product Quality (DMPQ) supports clinical or single use commercial facility

- 46 highly trained staff members in instrumentation and equipment Quality, technical operations, process development, assay development, management of contract manufacturing

- AAV processing GMP facility supportive of clinical translation
  - Separate Upstream and Downstream Suites
  - Capable of Clinical and Commercial Production

- Cell processing facility
  - Commercially viable GMP suites
  - Dedicated to the production of EB-101
  - Capacity for Commercial launch and scalability
Leadership

Management Team

João Siffert, M.D.
Chief Executive Officer

Timothy J. Miller, Ph.D.
President, Chief Scientific Officer

Christine Silverstein
Chief Financial Officer

Max Colao
Chief Commercial Officer

Edward Carr
Chief Accounting Officer

S. Kaye Spratt, Ph.D.
SVP, Regulatory Affairs

Juan Ruiz, M.D., Ph.D.
Head of European Medical Affairs

Neena Patil
SVP, General Counsel

Jay Bircher
SVP, Quality & Technical Operations

Scientific Advisors

Maria Escolar, M.D.
Pittsburgh Children’s Hospital

John Cooper, Ph.D.
University of California, San Diego

Jonathan Mink, M.D., Ph.D.
University of Rochester

Erika Augustine, M.D.
University of Rochester

Steven Gray, Ph.D.
University of Texas Southwestern

Kevin Flanigan, M.D.
Nationwide Children’s Hospital