Developing cell and gene therapies for serious diseases

December 2022
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Clinical-stage cell & gene therapy company with near-term catalysts

<table>
<thead>
<tr>
<th>EB-101</th>
<th>BLA submission expected in 2Q '23, potential approval in 1Q '24</th>
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<tbody>
<tr>
<td></td>
<td>• Unique product profile with est. $2B+ cumulative U.S. revenues over product lifecycle</td>
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<td>• Priority review voucher (PRV) opportunity worth ~$100M upon BLA approval</td>
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<td>• Only investigational product positioned as “one-and-done” solution for large chronic RDEB wounds</td>
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<tr>
<th>AAV-based gene therapies</th>
<th>advancing toward clinic with focus on ophthalmology</th>
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<td></td>
<td>• Proprietary next-generation AIM™ capsids show strong transduction levels in macula and optic nerve using safer routes of administration in non-human primates</td>
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<td>• Clinical candidate nomination for ophthalmic programs, each with 5,000 to 15,000 est. US prevalence, expected in 1Q ’23; possible pre-IND meeting in 1H ’23</td>
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<thead>
<tr>
<th>Longer-term value potential of royalties and milestones</th>
<th>from out-licensed programs</th>
</tr>
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<tr>
<td>ABO-102 (to Ultragenyx), CLN-1 and Rett Syndrome (to Taysha Gene Therapies)</td>
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Cash runway into 3Q ‘24, well beyond EB-101 BLA submission and potential approval
EB-101: Engineered cell therapy for RDEB

- Orphan Drug Designation (FDA)
- Orphan Drug Designation (EU)
- Rare Pediatric Disease Designation (FDA)
- Breakthrough Therapy Designation (FDA)
- Regenerative Medicine Advanced Therapy Designation (FDA)
Recessive dystrophic epidermolysis bullosa (RDEB) is a painful disease with lifelong burden afflicting thousands of U.S. patients.

- Inherited connective tissue disorder with debilitating pain and systemic complications leading to early death.
- Primarily characterized by skin blisters and erosions.
- Caused by mutations in COL7A1 gene, which encodes type VII collagen.
- Est. 3,850 U.S. patients
- Up to 80% of patient’s body covered in wounds, leading to:
  - Severe pain and widespread scarring.
  - Numerous debilitating and life-threatening systemic complications.
  - Inflammation, infections, loss of heat - high metabolic rate and malnutrition.
  - 75-90% risk of developing squamous cell carcinoma (SCC).
- Heavy clinical, economic and humanistic burden with no approved treatment or cure.

50% of generalized severe patients die before 35.
75% die before 40.

EB-101 restores functional collagen VII to patient’s own cells

Biopsy

Keratinocytes extracted from biopsied samples

Keratinocytes grown and expanded

Keratinocytes transduced with corrected collagen gene

Gene-corrected cells grown to prepare for seeding

Cells seeded in plates for maturation into sheets

Media change to support stratification

Fully formed sheets 5-7 cell layers thick produced

Coordinate operating room time / resources

Cell sheets ready for packaging and delivery
EB-101 administration uses a standard surgical procedure

1. Wound bed prepared and antibiotics administered while surgeon prepares EB-101 for transplant.

2. Surgeon applies and sutures EB-101 on wound.

3. Covered wound treated with antibiotics and wrapped with gauze and surgical netting.
Significant unmet need for treating large chronic RDEB wounds

- Large chronic RDEB wounds:
  - Size: ≥20 cm² of body surface area (BSA)
  - Chronicity: ≥6 months (often open for years)
  - Cover >30% of body surface area on average
  - Severe daily pain leads to chronic opioid use
- No current treatment or cure for RDEB wounds; standard of care involves daily wound care, pain management, and protective bandaging

EB-101 is the only investigational product with promise as ‘one-and-done’ instantaneous therapy for large chronic RDEB wounds providing durable wound healing and pain reduction
Phase 3 VIITAL study topline results
Phase 3 VIITAL study evaluated EB-101 for wound healing and pain reduction using intra-patient randomization of wounds

Target Enrollment:
• ~36 wound pairs in 10–15 patients
• Age ≥6 years
• Minimum two large chronic* wounds per patient

Randomized wound pairs
EB-101 & Control

Co-Primary Endpoints:
• ≥50% wound healing at Week 24***
• Reduction in pain severity (Wong-Baker FACES scale) associated with wound dressing changes at Week 24

Secondary Endpoint:
• Complete wound healing at Week 24***

Select Exploratory Endpoint:
• ≥75% wound healing at Week 24***

Non-randomized wounds**
EB-101 treated, not included in primary analysis

FDA-aligned endpoints include ≥50% wound healing and mean pain reduction after 6 months

*Large = >20 cm² surface area; Chronic = Open for >6 months
** Wounds with no matching control wound
***Week 24 result confirmed at Week 26
Significantly more wounds achieved ≥50% healing and showed pain reduction with EB-101

% Wounds with ≥50% Healing at six months vs. baseline

- **EB-101**: 81%
- Control: 16%

n=43 wound pairs
p-value: <0.0001

Mean Pain Reduction* from baseline at 6 months

- **EB-101**: n=43
- Control: n=42

The mean pairwise difference across patients in pain reduction was 2.23 with p=0.0002 and sample size of 42 wound pairs in 11 patients.

* Pain severity on 0-10 scale with scoring in increments of 2 (ie. 0, 2, 4, 6, 8, 10).
**EB-101 showed greatest pain reduction benefit in wounds with severe baseline pain**

**Mean Pain Reduction in EB-101 Treated Wounds**
(incl Randomized and Non-randomized)
from baseline at 6 months

<table>
<thead>
<tr>
<th></th>
<th>All treated wounds</th>
<th>All treated wounds with baseline pain ≥6</th>
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<tbody>
<tr>
<td></td>
<td>n=53</td>
<td>n=27</td>
</tr>
<tr>
<td>3.51</td>
<td></td>
<td>5.70</td>
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</tbody>
</table>

12
Greater wound healing is associated with greater magnitude in pain reduction

<table>
<thead>
<tr>
<th>Healing Stage</th>
<th>n</th>
<th>Mean Pain Reduction from baseline at 6 months</th>
</tr>
</thead>
<tbody>
<tr>
<td>&lt;50% healing</td>
<td>8</td>
<td>1.75</td>
</tr>
<tr>
<td>≥50% healing</td>
<td>35</td>
<td>3.37</td>
</tr>
<tr>
<td>≥75% healing</td>
<td>28</td>
<td>3.86</td>
</tr>
<tr>
<td>Complete healing</td>
<td>7</td>
<td>5.14</td>
</tr>
</tbody>
</table>
EB-101 significantly improved wound healing vs. control across all levels of healing

% Wounds that Met or Exceeded Healing Threshold Indicated at six months vs. baseline (n=43)

- **≥50% Healing**: EB-101: 81%, Control: 16%
  - p-value: <0.0001
- **≥75% Healing**: EB-101: 65%, Control: 7%
  - p-value: <0.0001
- **Complete Healing**: EB-101: 16%, Control: 0%
  - p-value: 0.0160

* Complete wound healing is defined as re-epithelialization with no drainage or erosion and presence of only minor crusting.
Stringent criteria applied to score wounds as completely healed

- Complete re-epithelialization with no drainage or erosion
- No major crusting as adjudged by investigator (subjective)
  - In VIITAL, with any crusting, inability to verify underlying epithelial formation led to wound scored as not having met complete healing
- No control wounds were scored as completely healed at week 24 (with week 26 confirmation)
- Following slides show examples of wounds that were ≥75% healed but not scored as completely healed
Examples of ≥75% and complete wound healing after EB-101 treatment (upper trunk)

Baseline
B4 scored as >75% healed at Week 24
E9 scored as complete wound healing at Week 24

Surgery
B4 (treated wound)
E9 (treated wound)

Week 24
B4
E9

Source: VIITAL patient
Positive VIITAL results reinforce EB-101 value proposition

- Statistically significant and clinically meaningful results across endpoints in VIITAL
  - Wound healing by investigator assessment at all levels vs. control
  - Pain reduction reported by patient vs. control
- More pronounced pain reduction for wounds with severe baseline pain
- EB-101 was shown to be well tolerated and no serious treatment-related adverse events observed, consistent with past clinical experience
- Further details with additional exploratory endpoints will be presented at a future scientific meeting
- VIITAL results along with the Phase 1/2a long term follow-up results\(^1\) form the basis for the value proposition of EB-101 with potential for durable wound healing and pain reduction with a one-time treatment

Phase 1/2a data complements VIITAL with evidence of multi-year wound healing and pain reduction after EB-101

Key Findings from Phase 1/2s Study
- Average surface area healed per patient: >130 cm² and >120 cm² at 3 and 6 months, respectively
- Evidence for healing of extremely large wounds (up to 400 cm²) that were open for 16+ years
- Considerable reduction in wound burden at mean 5.9 years follow-up
- Long-term symptomatic relief, including reduction in pain
KOLs prefer EB-101 for large chronic wounds that impact patient’s quality of life

- Surveyed KOLs* unanimously opine that almost all patients will need both products in course of the disease
- EB-101 preferred for large chronic wounds that impact patient’s quality of life
- EB-101 is only currently investigated therapy evaluated for pain reduction as a co-primary endpoint in pivotal registrational study

Critical Wound Characteristics Driving First-Choice Gene Therapy Allocations

- **Size**
  - Larger (>40 cm²)
  - Midsized (20-40 cm²)
  - Smaller (<20 cm²)

- **Chronicity**
  - Older (>12 weeks) or recurring
  - Newer (<12 weeks)

- **Location**
  - Back, posterior neck / shoulders
  - Extremities excluding joints (arm, thigh)
  - High-friction areas (axilla, groin, lower abdomen)
  - High functional impact areas (fingers, soles of feet)
  - Low functional impact areas (face, toes, neck)
  - Extremities at joints (elbow, knee, wrist)

- **QOL Impact**
  - Severe pain / pruritus
  - Urgency to treat, high risk of infection
  - Chronically or currently infected or colonized
  - Scarring, early signs of SCC

(1) Advisors agreed they would hesitate to use any gene therapy option on a patient with a history of SCC, particularly if advanced or invasive

Note: First-choice gene therapy allocations based on in-meeting polling and follow-up discussions with n = 5 pediatric dermatologists specializing in EB

* Reflects advisory board comprised of Mercedes Gonzalez, MD (Pediatric Dermatology), Irene Lara-Corrales, MD (Pediatric Dermatology), Moise Levy, MD (Pediatric Dermatology), Marissa Perman, MD (Epidermolysis), Joyce Tang, MD, PhD (Dermatology)
$2B+ U.S. cumulative revenue opportunity

>3,000 EB-101 Transplants

Est. 1,600 patients with each receiving ~2 cycles of EB-101 transplant over time
- **Epidemiology:** ~3,850 U.S. prevalent patients with incidence rate of 95 per million live births\(^1\)
- **Access:** 60% have access & are willing to try gene therapy
- **Market Share:** 70% choose EB-101 to treat their large / chronic and most painful / debilitating wounds based on best efficacy / safety / durability profile
  - No restriction foreseen on using other investigational therapies to treat smaller/ recurrent wounds

~2 transplants per patient can heal all addressable large chronic wounds
- BSA to heal all large & chronic wounds: ~960 cm\(^2\)
- EB-101 Average Expected Transplant BSA Coverage (i.e., 12 sheets): 480 cm\(^2\)

For a therapy backed by evidence for pain reduction and durable wound healing, a price between $300K and $800k\(^2\) per treatment may be attainable leading to revenue of $2B+ over product lifecycle\(^3\)

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1. Eichstadt et. al., From Clinical Phenotype to Genotypic Modelling: Incidence and Prevalence of Recessive Dystrophic Epidermolysis Bullosa (RDEB) Clinical, Cosmetic and Investigational Dermatology 2019:12 933–942
2. Based on initial payer insights (Medicare & Medicaid) and value proposition for one-time treatment; potential pricing of competition; benchmarking vs. cost of standard of care. Additional payer research ongoing. Recurring annual cost of wound care for RDEB-generalized severe subtype $112,000. Not accounting for chronic pain medication and impact on life-style.
3. With additional upside from incidence patients of 95 per million live births in the US\(^2\); and EB-101 treated-patient needing new treatment for RDEB wound in a different body area
Preclinical AAV-based gene therapies
Novel AAV capsids for use in gene therapies for multiple ophthalmic conditions with high unmet medical need

- **AIM™** novel capsids designed to selectively target delivery of genetic payloads with **improved tissue/cell tropism** and **reduced immune response** to natural AAV capsids (potential for redosing in patients with prior AAV therapy)

- Abeona-invented novel capsids designed for **systemic delivery** or **direct ocular injection**

- Investigating **AIM™** capsids and Abeona-invented capsids in **undisclosed ophthalmic conditions**, each with 5,000 to 15,000 est. US prevalence
  - **AIM™** capsid AAV204 showed high transduction levels in macula and optic nerve compared to AAV8 using less invasive and safer route of administration than subretinal surgical delivery in NHPs
  - Clinical candidate nomination for ophthalmic programs, each with 5,000 to 15,000 est. US prevalence, expected in 1Q 2023; possible pre-IND meeting with FDA in 1H 2023

- Exploring licensing opportunities for Abeona’s proprietary capsids in a variety of ocular conditions
Anticipated milestones
## 2023 anticipated milestones

### EB-101

<table>
<thead>
<tr>
<th>Milestone</th>
<th>Timeframe</th>
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<tbody>
<tr>
<td>✓ Top-line results from VIITAL study</td>
<td>4Q 2022</td>
</tr>
<tr>
<td>❑ Full results from VIITAL study in publication and/or medical congress</td>
<td>2Q 2023</td>
</tr>
<tr>
<td>❑ BLA submission</td>
<td>2Q 2023</td>
</tr>
<tr>
<td>❑ Commercial preparations focusing on value proposition (payer and provider discussions)</td>
<td>1H 2023</td>
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### Preclinical

<table>
<thead>
<tr>
<th>Milestone</th>
<th>Timeframe</th>
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<tr>
<td>✓ Animal POC completed</td>
<td>4Q 2022</td>
</tr>
<tr>
<td>❑ Clinical candidate nomination</td>
<td>1Q 2023</td>
</tr>
<tr>
<td>❑ Possible pre-IND meeting</td>
<td>1H 2023</td>
</tr>
<tr>
<td>❑ Initiate IND-enabling studies</td>
<td>2H 2023</td>
</tr>
<tr>
<td>❑ Exploring licensing opportunities for our next-generation AAV capsids</td>
<td>Ongoing</td>
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