



1Q'23 Portfolio Update Conference Call

May 24, 2023

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Agenda



Opening Remarks

Vish Seshadri, Ph.D., M.B.A., Chief Executive Officer



Additional Phase 3 VIITAL study results at ISID

Dmitriy Grachev, M.D., Ph.D., Chief Medical Officer



Animal proof-of-concept data from AAV ophthalmology program at ASGCT

Brian Kevany, Ph.D., Chief Technical Officer



Q&A

Vish Seshadri, Dmitriy Grachev, Brian Kevany, Joe Vazzano, Madhav Vasanthavada



Opening Remarks

Vish Seshadri, Ph.D., M.B.A., Chief Executive Officer

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
- Estimated 3,850 U.S. patients¹
- 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 few treatment options

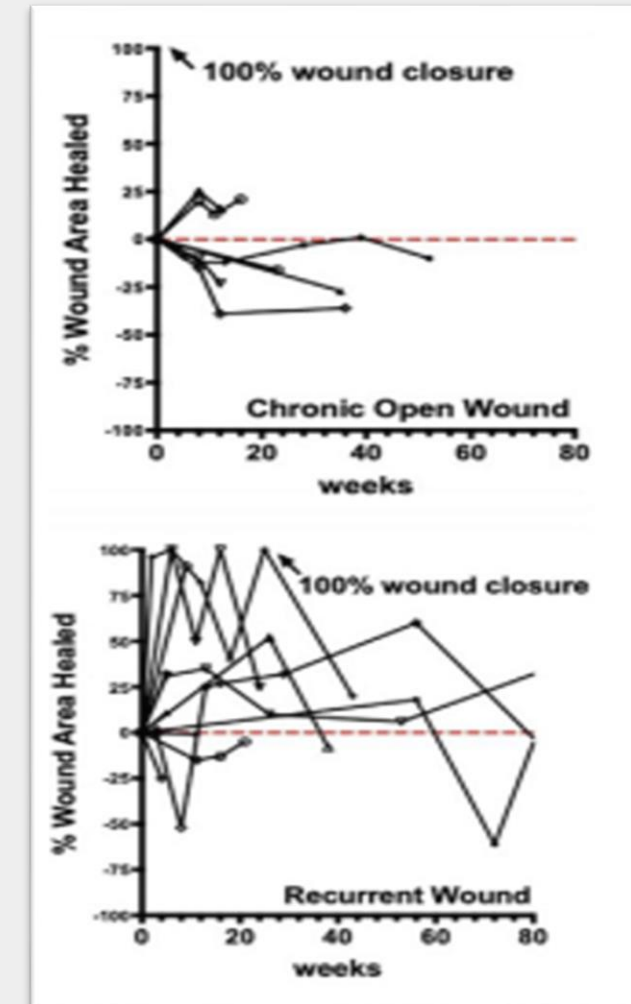
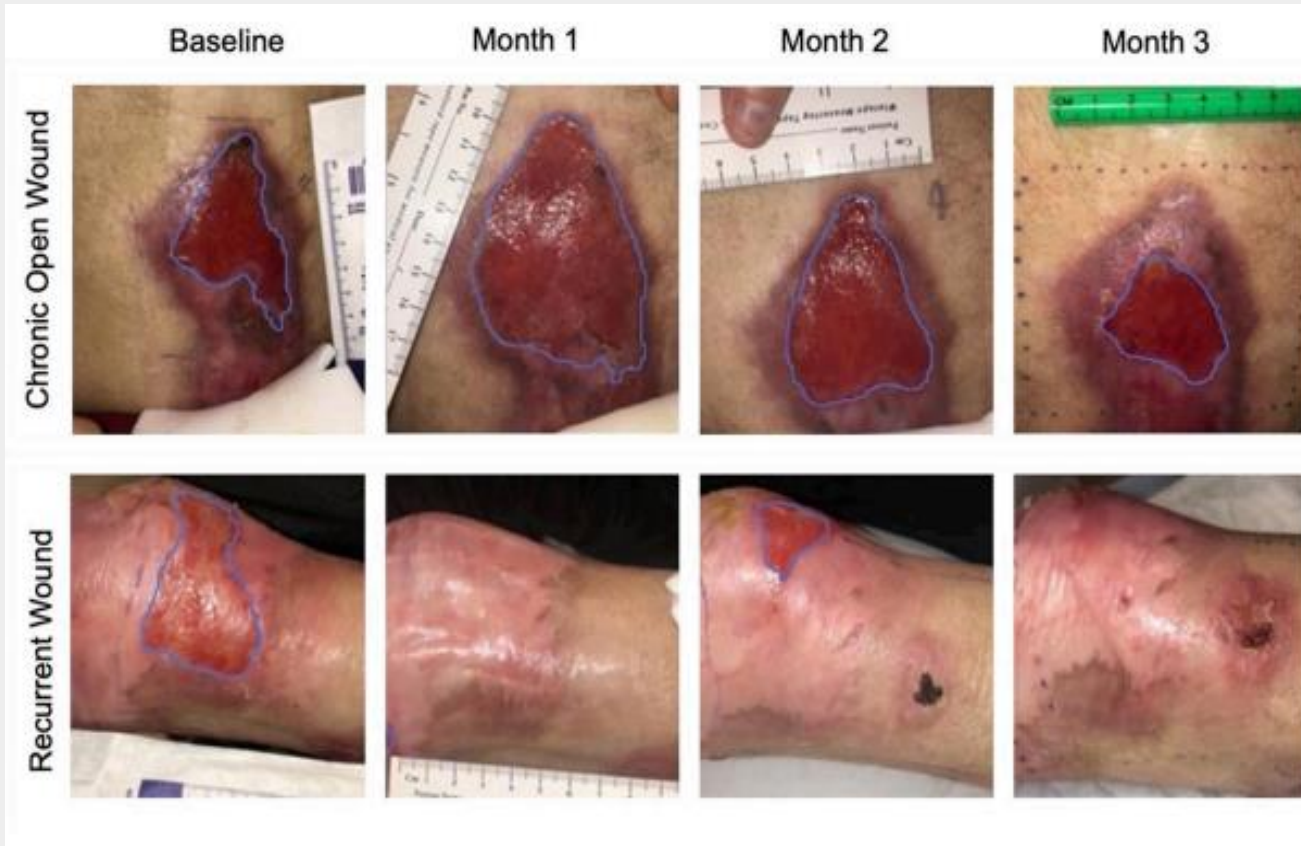


50% of generalized severe patients die before 35

75% die before 40

Emerging natural history data reveals large chronic wounds have reduced propensity for spontaneous healing vs recurrent wounds

- RDEB Natural History Study 2021 (251 wounds, retrospective analysis)¹



- RDEB Natural History Study 2023 (69 wounds, prospective analysis)² corroborates findings from 2021 study

¹ Classification of 2 distinct wound types in recessive dystrophic epidermolysis bullosa: A retrospective and cohort natural history study. Solis et. al., J. Am Acad Dermatol; 2021 85(5): 1296-1298;
² Harris et. Al: Natural history of spontaneous wound healing in recessive dystrophic epidermolysis bullosa wound types using a mobile photography application; Poster Presentation; ISID 2023, Tokyo

Example of $\geq 75\%$ healed after EB-101 treatment (upper left thigh)

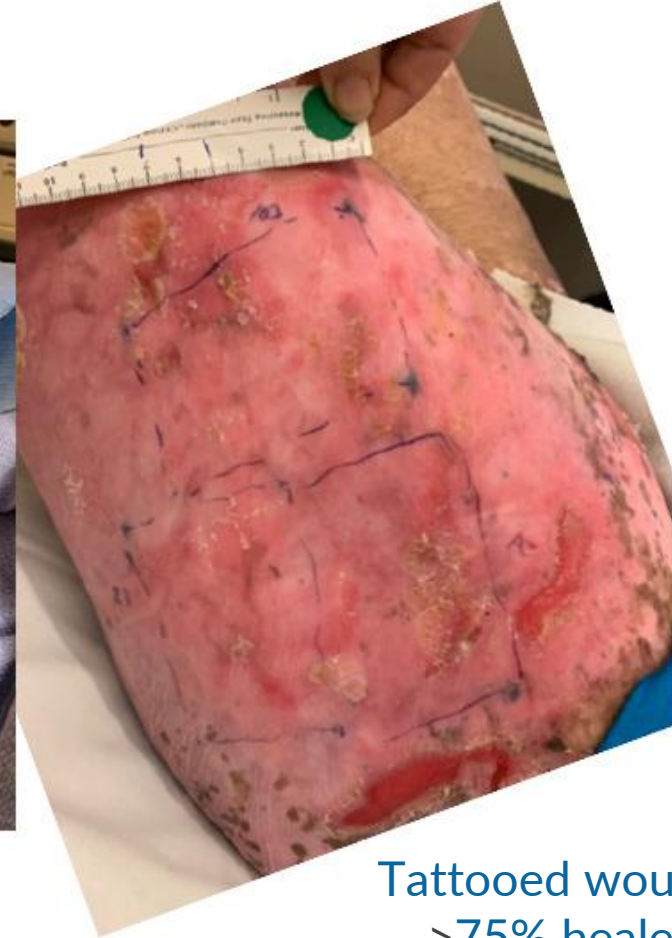
Baseline



Surgery

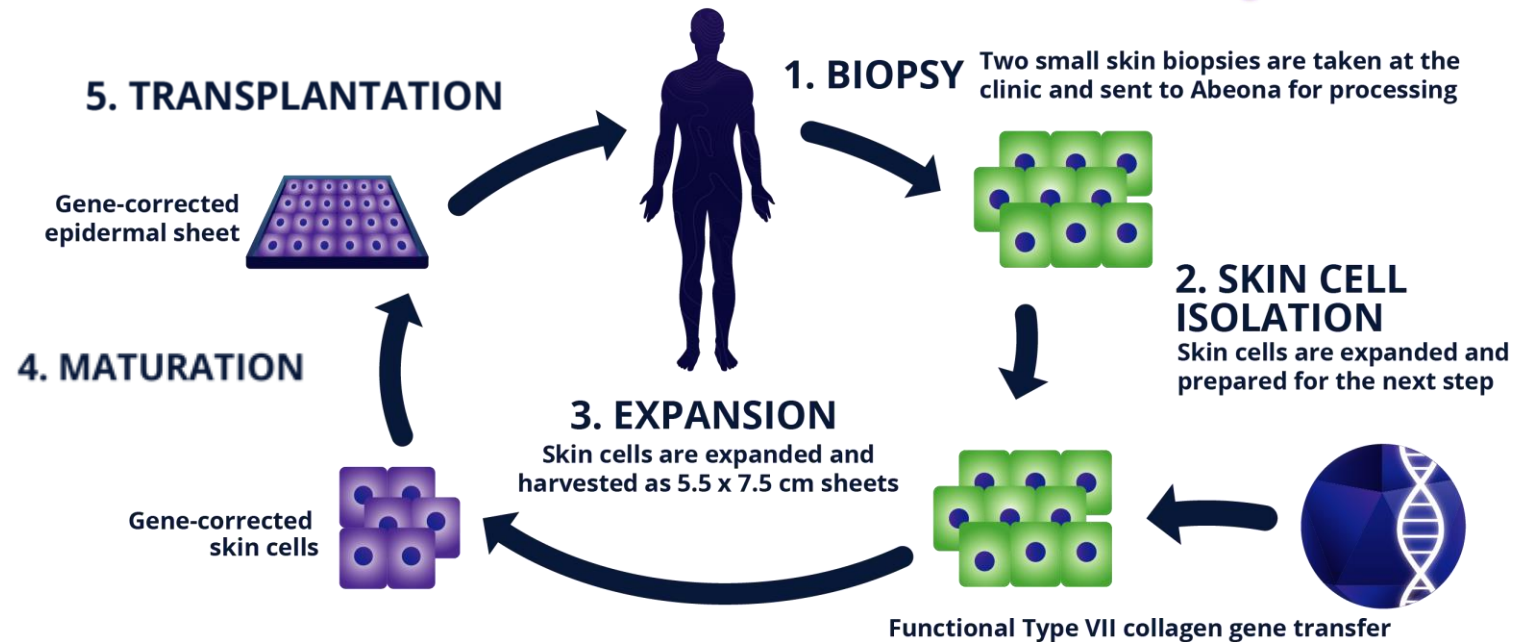
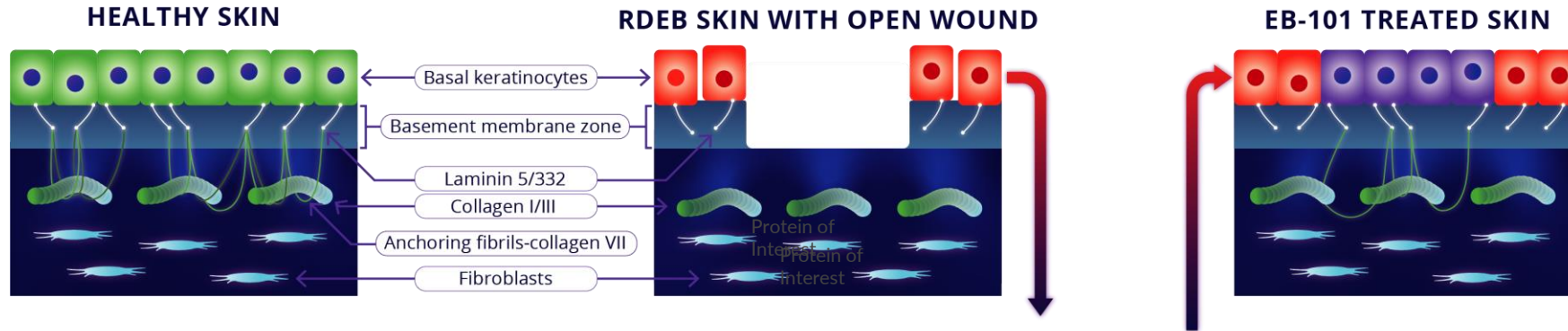


Week 24

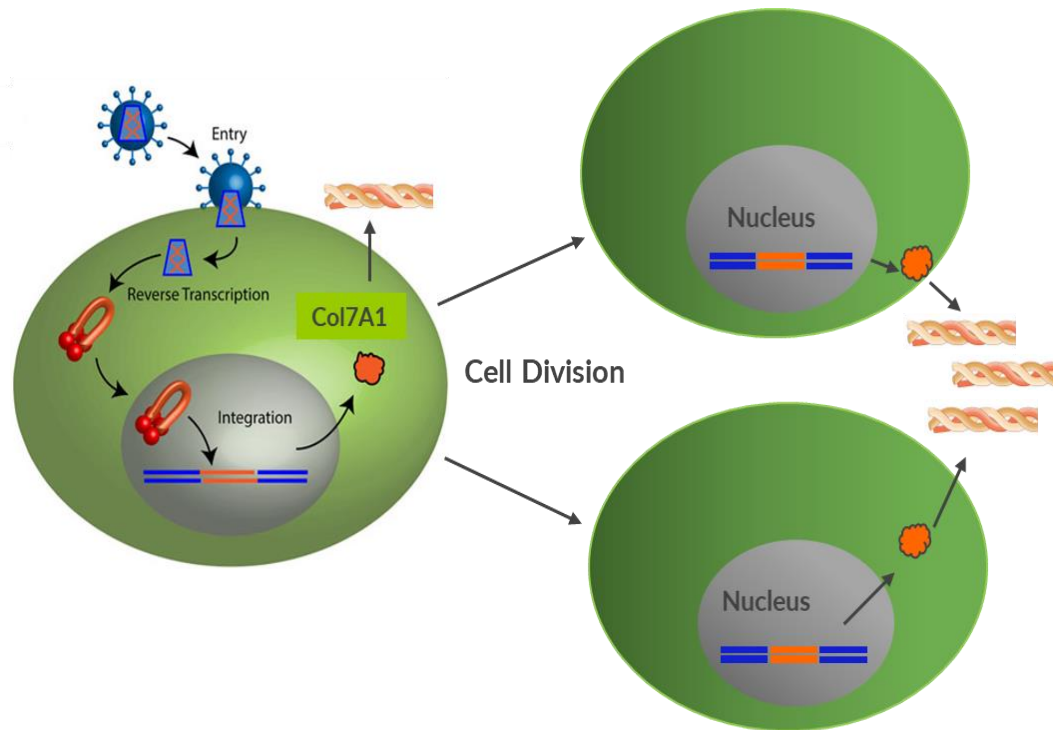


Tattooed wounds scored as
>75% healed at Week 24

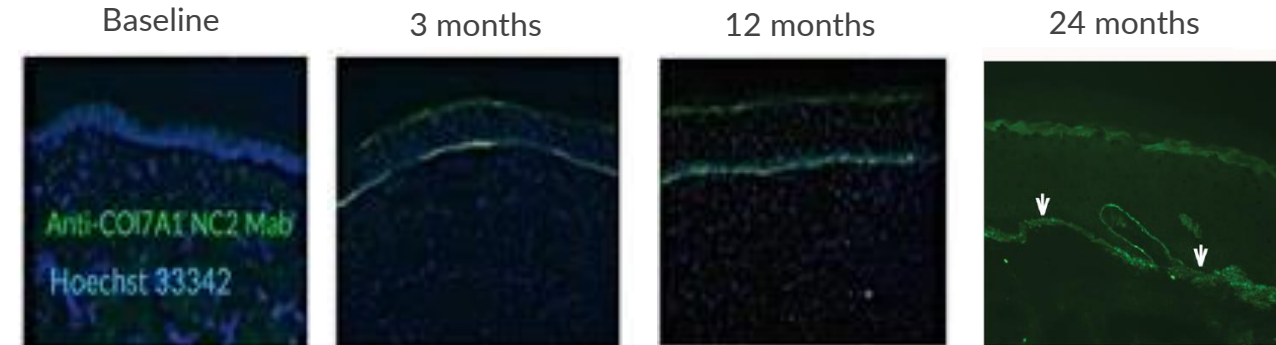
EB-101 restores functional collagen VII to patient's own cells



Transgene encoding functional COL7A1 stably integrates into host genome and is therefore maintained through cell division

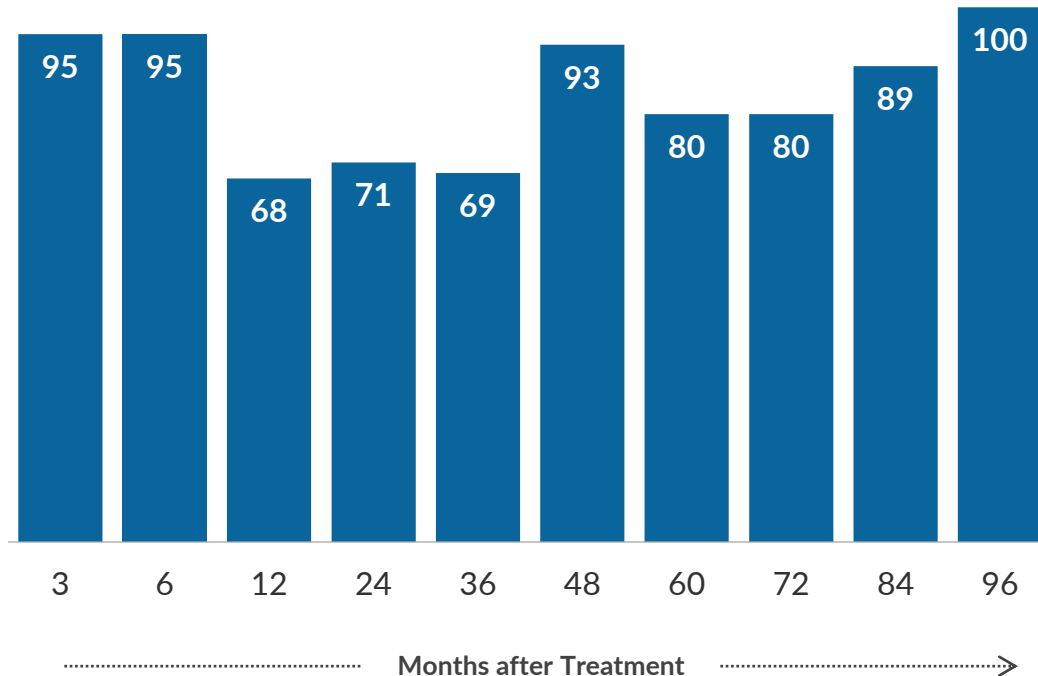


Long term Col7A1 expression at treated site detected by immunofluorescence

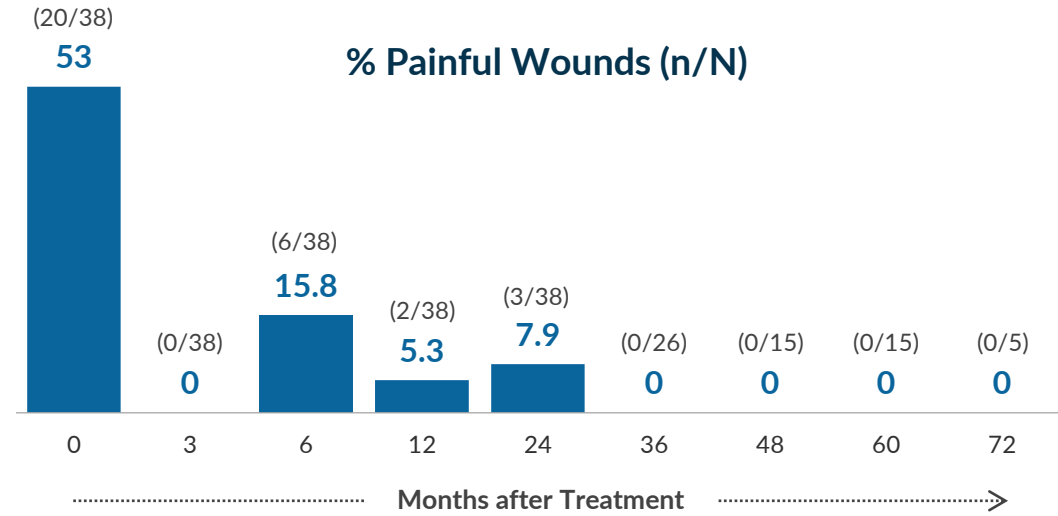


Evidence of multi-year wound healing and pain reduction after EB-101 in Phase 1/2a study

% of Wounds with $\geq 50\%$ Healing



Overall Wound Pain: Relief Associated with EB-101



Key Findings from Phase 1/2a Study

- Average surface area healed per patient: $>130 \text{ cm}^2$ and $>120 \text{ cm}^2$ at 3 and 6 months, respectively
- Evidence for healing of extremely large wounds (up to 400 cm^2) 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



Additional Phase 3 VIITAL™ Study Results for EB-101

**Presented at International Societies for Investigative Dermatology 2023 Meeting
May 10-13, 2023**

Dmitriy Grachev, M.D., Ph.D., Chief Medical Officer

VIITAL study¹: Designed to show wound healing and pain reduction in RDEB patients

Phase 3, randomized, inpatient controlled trial treating the most severe RDEB wounds

Eligibility:

- Age ≥6 years with confirmed RDEB
- ≥2 matched large, chronic^a wounds per patient
- No evidence or history of SCC in the area that would undergo EB-101 application

Randomized wound pairs
EB-101 (n=43) & Control (n=43)
across 11 RDEB patients

Nonrandomized wounds^b
EB-101 treated, not included in primary
analysis (n=14)

Co-primary Endpoints:

- ≥50% wound healing at week 24^c
- Pain reduction at week 24

Secondary Endpoint:

- Complete wound healing at weeks 12 and 24^c

Select Exploratory Endpoints:

- ≥75% wound healing at weeks 12 and 24^c
- ≥50% wound healing at week 12
- Pain reduction at week 12
- Pain reduction over time (at-home diary)
- Change in itch severity at week 24
- CrGI-Pain and PGIC-Blistering scores at week 24

Sample Size

A sample size of a minimum of 36 wound pairs in 10 to 15 participants was estimated using conservative assumptions based on wound healing and pain reduction observed in the phase 1/2a trial. Statistical power for each co-primary endpoint was initially calculated separately. **The joint statistical power for the trial was bounded by the multiplication of these 2 powers and was expected to be >80%.**

^aLarge = ≥20 cm² surface area; chronic = open for ≥6 months. ^bEligible wounds based on size and chronicity that either: (a) did not have a matched wound to pair with it, or (b) were initially randomized to control, but were ultimately treated with EB-101 because their matched pair was deemed untreatable during surgery due to the patient's position. ^cConfirmed at a subsequent visit ≥2 weeks later.

CrGI, Caregiver Global Impression; PGIC, Patient Global Impression of Change; RDEB, recessive dystrophic epidermolysis bullosa; SCC, squamous cell carcinoma.

1. ClinicalTrials.gov Identifier: NCT04227106

Demographics, baseline characteristics, and EB-101 exposure

Patients with RDEB

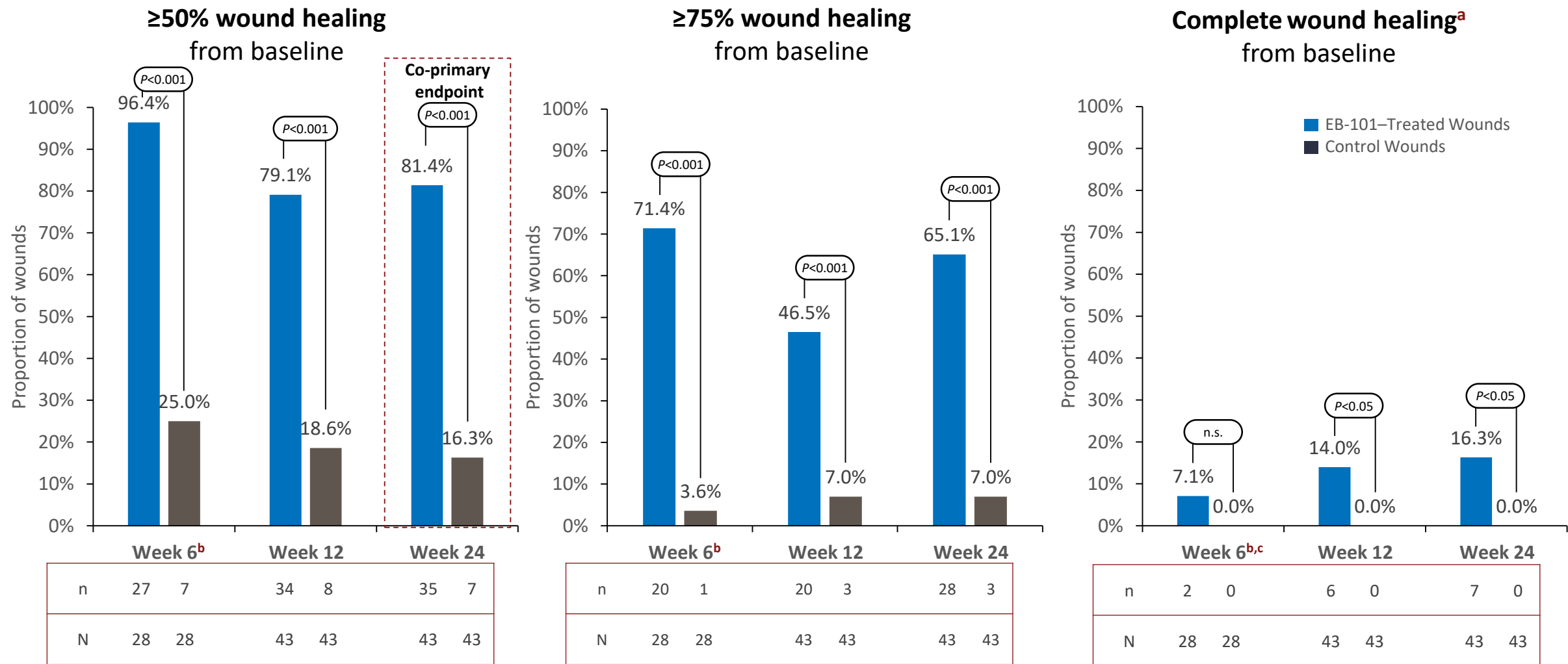
| | Total N=11 |
|--|----------------|
| Age , median (range) — y | 21.0 (6 to 40) |
| Sex — n (%) | |
| Male | 4 (36.4) |
| Female | 7 (63.6) |
| Race — n (%) | |
| White | 10 (90.9) |
| Other (Unknown) | 1 (9.1) |
| Ethnicity — n (%) | |
| Hispanic or Latino | 2 (18.2) |
| Not Hispanic or Latino | 8 (72.7) |
| Not reported | 1 (9.1) |
| Wound pairs per patient median (range) | 4 (2 to 5) |

Randomized Wounds

| | EB-101- Treated n=43 | Control n=43 |
|--|----------------------------|-----------------|
| Wounds by anatomical region , n (%) | | |
| Anterior trunk | 18 (41.9) | 4 (9.3) |
| Posterior trunk | 10 (23.3) | 23 (53.5) |
| Upper extremity | 8 (18.6) | 5 (11.6) |
| Lower extremity | 7 (16.3) | 11 (25.6) |
| Wound duration , median (range) — mo. | 60 (6 to 252) | 60 (6 to 252) |
| Pain severity , median (range) | 4.0 (0 to 10) | 4.0 (0 to 10) |
| Itch severity , median (range) | 4.0 (0 to 10) | 4.0 (0 to 10) |
| Total wound surface area covered by EB-101 per patient,^a median (range) — cm² | 160.0 (80 to 200) | 0 |

^aWound surface area was calculated after wound debridement.

EB-101 improved wound healing in as early as 6 weeks and across all timepoints

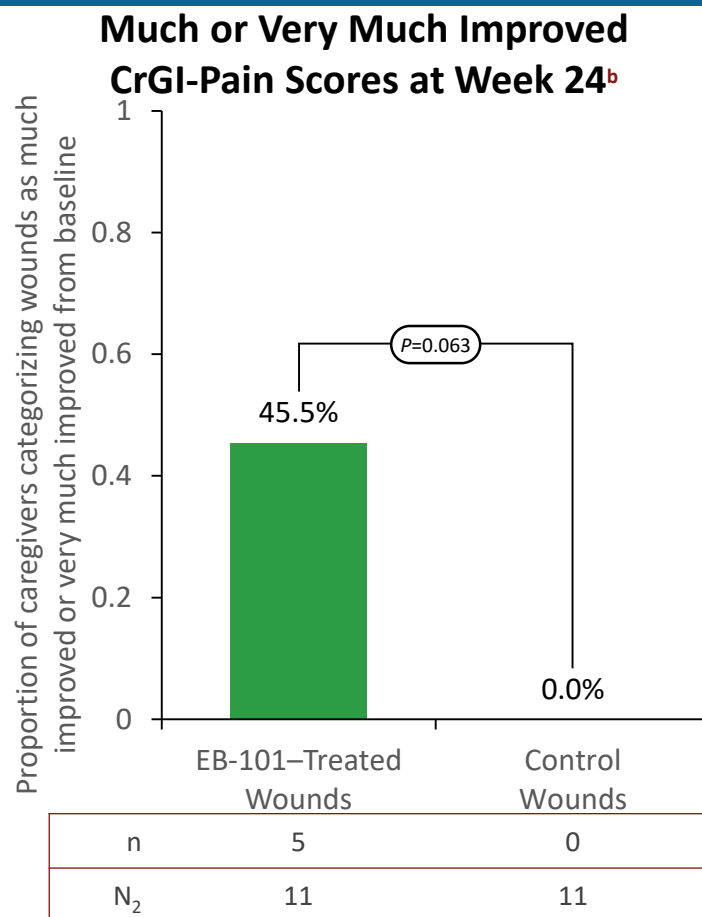
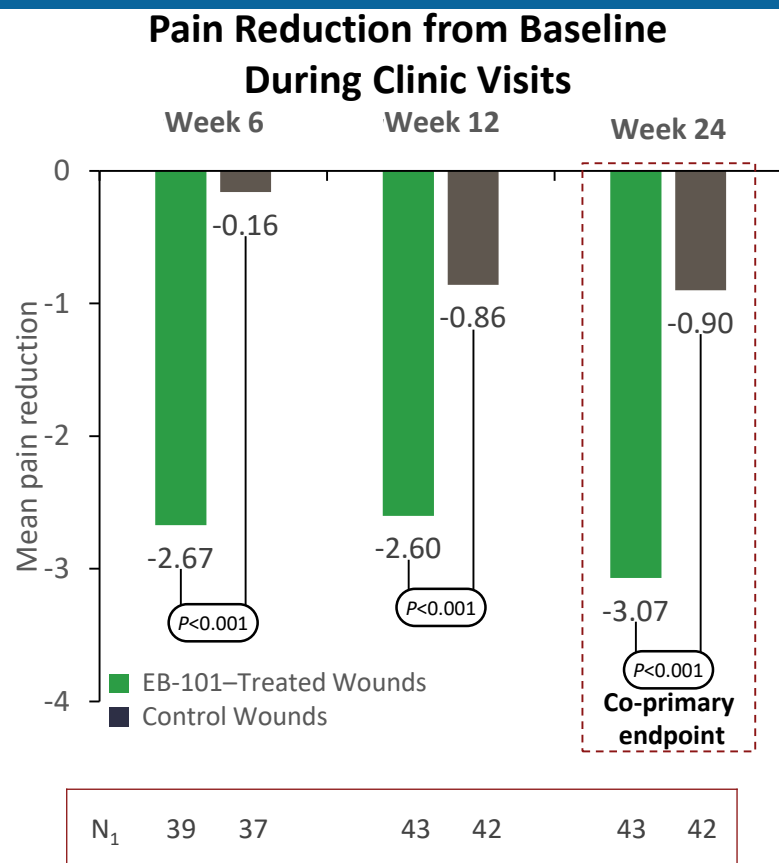


Wounds demonstrating healing at week 24 were required to be confirmed ≥2 weeks later to be included.

^aComplete wound healing was defined as re-epithelialization with no drainage or erosion and presence of only minor crusting. ^bPost hoc endpoint. ^cMissing data was not imputed; observed case only.

n, number of wounds in healing improvement category; N, number of total wounds with nonmissing healing improvement category; n.s., not significant.

Significant reduction in pain reported with EB-101^a



Additional Pain Measures

At-home pain severity assessments using the Wong-Baker FACES scale showed statistically significant **pain reduction with EB-101 treatment as early as week 3.**

Pain was also assessed using PROMIS^c, with a significantly greater improvement in pain quality-sensory scores achieved with EB-101 treatment.

^aPain was assessed via the Wong-Baker FACES scale or numeric rating scale. For every post-baseline assessment, the pain reduction was calculated as baseline pain score minus the post-baseline pain score.

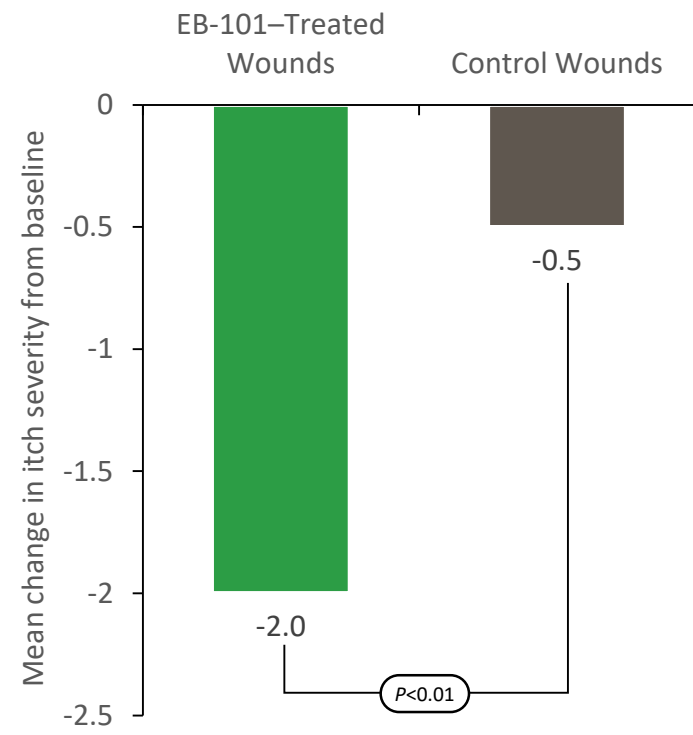
^bEach caregiver gave 2 responses on the CrGI-Pain, 1 for all EB-101-treated wounds (randomized and nonrandomized) and the other for all control wounds. ^cChange in pain quality and pain interference assessed using the PROMIS Pediatric Short Form 8a versions of Pain Quality (sensory and affective domains) and Pain Interference scales at week 24.

CrGI, Caregiver Global Impression; n, number of caregiver responses; N_1 , total number of wounds with nonmissing pain reduction score; N_2 , total number of caregiver responses at Week 24; PROMIS, Patient-Reported Outcomes Measurement Information System.

Patient- and caregiver-reported outcomes show quality of life improvements

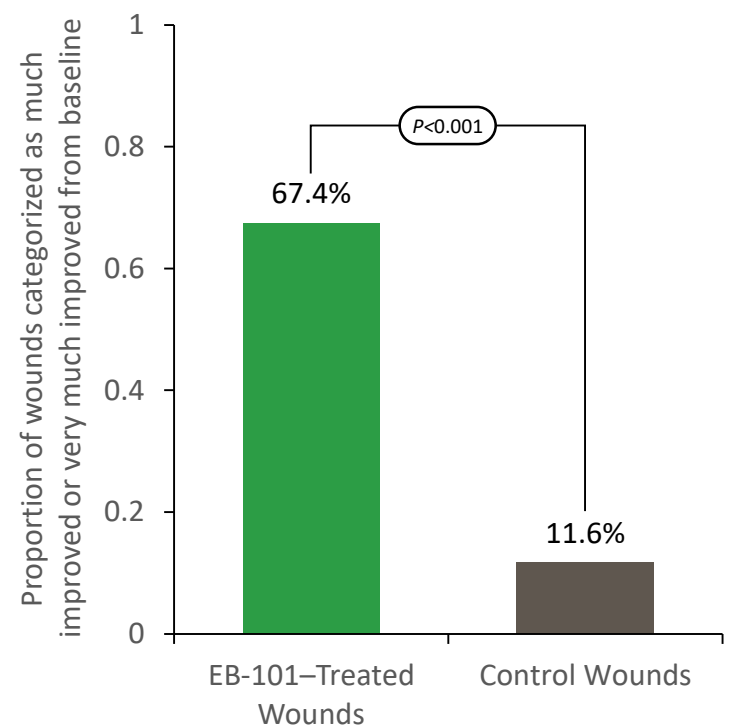


Change in Itch Severity at Week 24



| | | |
|----------------|----|----|
| N ₁ | 43 | 42 |
|----------------|----|----|

Much or Very Much Improved
PGIC-Blistering Scores at Week 24



| | | |
|----------------|----|----|
| n | 29 | 5 |
| N ₂ | 43 | 43 |

Wound-specific treatment-emergent adverse events^a

| | EB-101-Treated Wounds ^b | | Control Wounds | |
|---|------------------------------------|------------------|--------------------|------------------|
| | Patients (N=11) | Wounds (n=57) | Patients (N=11) | Wounds (n=43) |
| | n | n (%) E | n | n (%) E |
| All | 7 | 22 (38.6) 35 | 4 | 7 (16.3) 7 |
| Serious | 0 | 0 | 0 | 0 |
| Leading to new or prolonged hospitalization | 0 | 0 | 0 | 0 |
| Leading to trial discontinuation | 0 | 0 | 0 | 0 |
| Leading to death | 0 | 0 | 0 | 0 |
| Leading to infection | 5 | 12 (21.1) 21 | 3 | 4 (9.3) 4 |
| Related to EB-101 | 1 | 6 (10.5) 6 | 1 | 3 (7.0) 3 |

EB-101 shown to be well tolerated in VIITAL, consistent with past clinical trial experience

^aTEWAEs are defined as any study wound adverse event with an onset on or after the date of EB-101 application.

^bResults include both randomized and nonrandomized EB-101-treated wounds.

E, event.

**In VIITAL, EB-101 –
treated wounds
showed a favorable
risk-benefit profile in
patients with RDEB.**

- **Both co-primary endpoints met** with majority of EB-101–treated wounds demonstrating $\geq 50\%$ healing, and greater pain reduction observed in EB-101–treated wounds vs. control at six months
- **At earlier time points (6 and 12 weeks)**, percentage of wounds demonstrating healing ($\geq 50\%$, $\geq 75\%$, and complete) and pain reduction was greater in EB-101–treated wounds vs. control
- Patient-reported outcomes for **itch and blistering showed significantly greater improvement** with EB-101 treatment
- **Caregiver-reported outcomes** for wound care and overall impression of wound pain showed **consistent trends for improvement**
- EB-101 was **safe and well tolerated**, with no reports of patient-level or wound-specific serious EB-101–related TEAEs and only a small number of nonserious EB-101–related TEAEs, consistent with previous clinical trial experience¹⁻³

RDEB, recessive dystrophic epidermolysis bullosa; SAE, serious adverse event; TEAE, treatment-emergent adverse event.

1. Siprashvili Z, et al. *JAMA*. 2016;316(17):1808-17. 2. Eichstadt S, et al. *JCI Insight*. 2019;4(19):e130554. 3. So JY, et al. *Orphanet J Rare Dis*. 2022;17(1):377

Significant progress toward BLA submission



- **Recent accomplishments and next steps for BLA**
 - Completed three PPQ runs
 - Pre-BLA meeting scheduled on July 10, 2023
 - Anticipate BLA submission in early-3Q 2023
- **Commercial planning on-track to understand market access**
 - Encouraging initial feedback from stakeholders across healthcare system supports positive coverage
 - Payor research supports EB-101 pricing in-line with the value of a one-time treatment that delivers wound healing and pain reduction for years



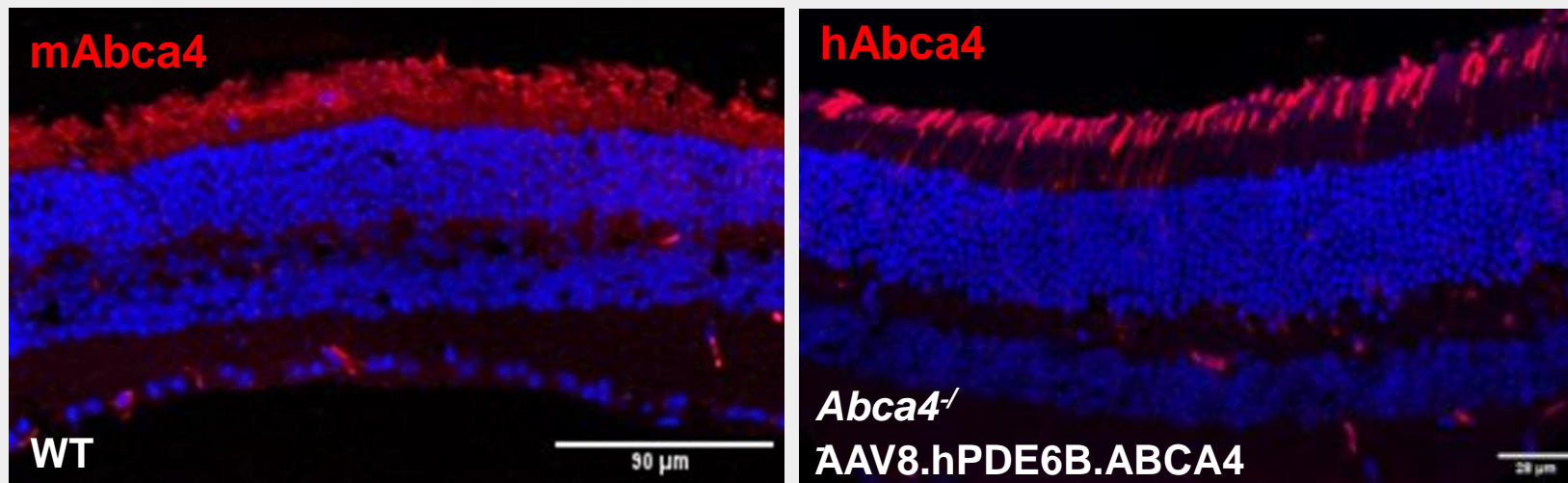
Animal Proof-of-Concept Data from AAV Ophthalmology Program

**Presented at Annual Meeting of American Society of Gene & Cell Therapy
May 16-20, 2023**

Brian Kevany, Ph.D., Chief Technical Officer

ABO-504 for Stargardt disease

“In Vivo Production of Full-Length ABCA4 Protein Following Cre-Mediated Recombination from Dual AAV Vectors in ABCA4^{-/-} Mice”



Key Findings:

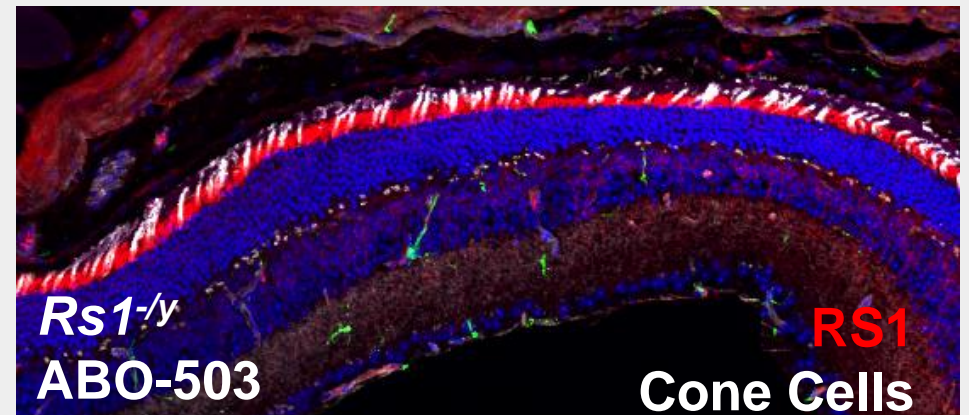
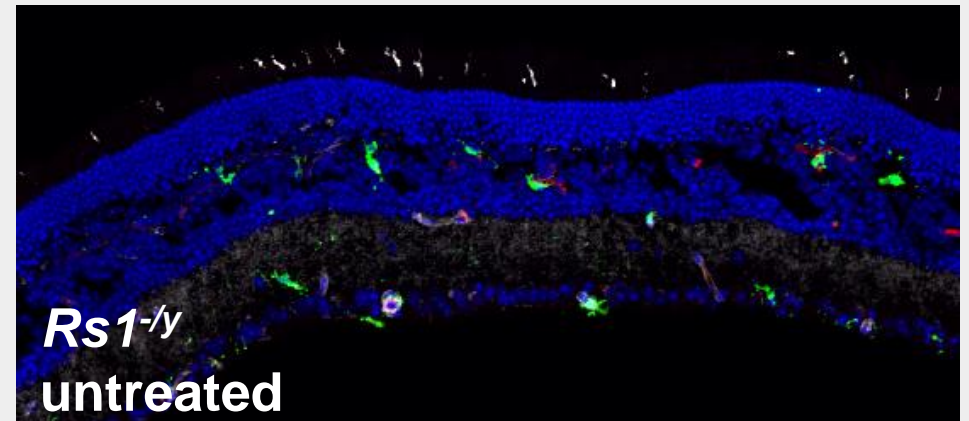
- A dual AAV vector strategy using Cre recombinase efficiently reconstituted the ABCA4 gene, leading to full-length hABCA4 protein expression *in vivo* in photoreceptor cells in ABCA4^{-/-} mouse eyes.
- Cre-mediated recombination of ABCA4 was confirmed by mRNA sequencing of RNA from cell culture and AAV-dosed animals.
- Immunohistochemistry confirmed correct localization of recombinant hABCA4 protein in photoreceptor cells.
- Studies of functional recovery are ongoing.

ABO-503 for X-linked retinoschisis

“ABO-503, a Novel Gene Therapy for Treatment of X-Linked Retinoschisis”

Key Findings:

- Robust RS1 expression observed in photoreceptor cells near injection site and in adjacent inner retina in mutant mice six months after treatment with ABO-503.
- RS1 expression associated with improvement in cone photoreceptor density and increased thickness of photoreceptor outer nuclear layer.
- Full-field flicker electroretinogram (ERG) analysis showed significant improvement in cone photoreceptor function.

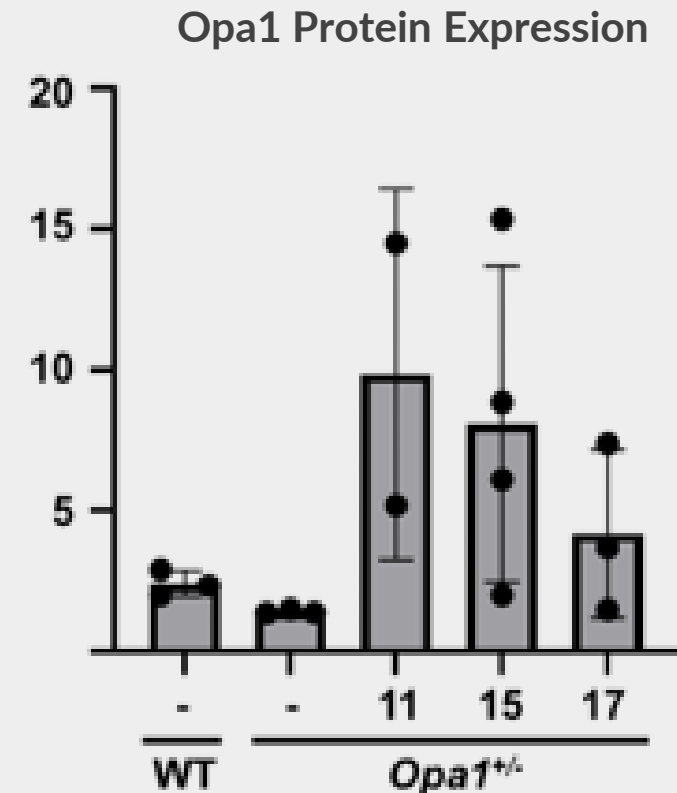


AAV gene therapy for autosomal dominant optic atrophy

“AAV Gene Therapy for Autosomal Dominant Optic Atrophy Caused by Mutation in the Opa1 Gene”

Key Findings:

- Vectors expressing Opa1 showed robust expression at both RNA and protein levels both *in vitro* and *in vivo*.
- In Opa1 knockout mouse fibroblasts, isoform variants 1, 5 and 7 expressed both RNA and protein with each variant corresponding to the expected cleavage pattern.
- Following intravitreal injection in wild type mice, variants 1 and 7 showed robust protein expression.
- Visual acuity assessments demonstrate function recovery in treated Opa1 mutant mice.





Q&A