Corporate presentation

Abeona THERAPEUTICS

January 2024

Note regarding forward-looking statements

This presentation contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. We have attempted to identify forward-looking statements by such terminology as “may,” “will,” “believe,” “anticipate,” “expect,” “intend,” and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including, but not limited to, the timing and outcome of our Biologics License Application submission to the FDA for pz-cel; the FDA’s grant of a Priority Review Voucher; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with the FDA or other regulatory agencies, including those relating to preclinical programs; the ability to achieve or obtain necessary regulatory approvals; the impact of any changes in the financial markets and global economic conditions; risks associated with data analysis and reporting; and other risks disclosed in the Company’s most recent Annual Report on Form 10-K and subsequent periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

Cell and gene therapy company with PDUFA date in May 2024

Differentiated, de-risked transformational asset highlights growth potential

Pz-cel for recessive dystrophic epidermolysis bullosa (RDEB)

Differentiated profile with transformational potential

- Demonstrated wound healing and pain reduction
- Years of benefit after one treatment in clinical trials
- Ability to cover ~500 cm^2 with a one-time application post-approval

Largely de-risked asset

- Positive pivotal phase 3 study
- BLA accepted with priority review and potential PRV opportunity
- PDUFA date is May 25, 2024

Substantial revenue potential and profitable business model

- >$500M US peak revenue potential by year 5
- Non-CDMO dependent end-to-end manufacturing

Gene therapy platform

- Proprietary AAV capsid platform with unique properties for genetic ocular disorders
- Compelling proof-of-concept in Stargardt disease, X-linked retinoschisis, and autosomal dominant optic atrophy in animal models
- IND-enabling studies expected to begin in 2024

Out-licensed gene therapies

- MPS IIIA program with Ultragenyx Pharmaceutical
- Rett syndrome and CLN-1 programs with Taysha Gene Therapies

Current cash runway well beyond expected timing of BLA approval and PRV grant

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Pz-cel:
autologous COL7A1 gene-corrected epidermal sheets 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)

Pz-cel has the potential to deliver unique value for patients with RDEB

Only investigative therapy designed to address **large body surface areas**, even **toughest-to-treat EB** wounds and establish **wound healing** and **pain reduction** with years of **durability** after a **one-time treatment**[1,2]

1. https://clinicaltrials.gov/ct2/show/NCT04227106
2. So et al. Orphanet Journal of Rare Diseases (2022) 17:377

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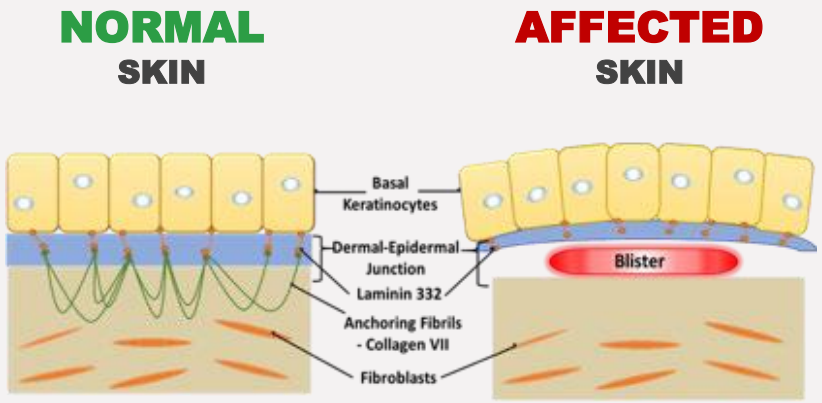
RDEB is a painful, debilitating disease with lifelong burden and poor quality of life

- Inherited connective tissue disorder with severe 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 3850 patients in the United States[1]
- Vast majority of patient's body covered in painful wounds
- Life-threatening complications including 75%-90% risk of developing squamous cell carcinoma (SCC)

50% of generalized severe patients die before age 35

75% die before age 40

Lack of functioning anchoring fibrils leads to skin blistering and tears from minor trauma



1. Eichstadt et al. Clin Cosmet Investig Dermatol. 2019 Dec 24;12:933-942. doi: 10.2147/CCID.S232547. Erratum in: Clin Cosmet Investig Dermatol. 2021 Jun 21;14:679. PMID: 31920360; PMCID: PMC6935313.

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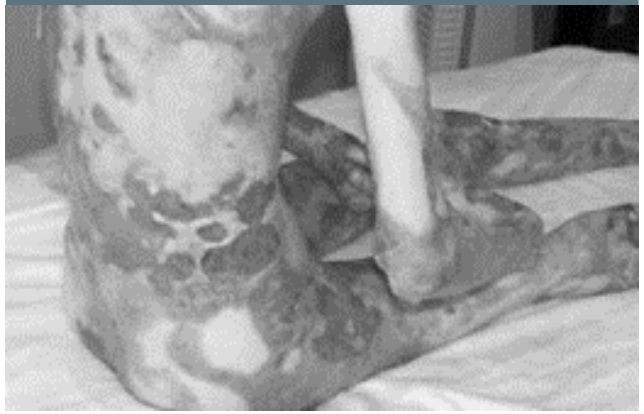
RDEB is debilitating across clinical, economic, and humanistic dimensions

Clinical



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

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

Economic

Average annual cost of wound care for patients with RDEB is ~$1M (DEBRA of America[1]).

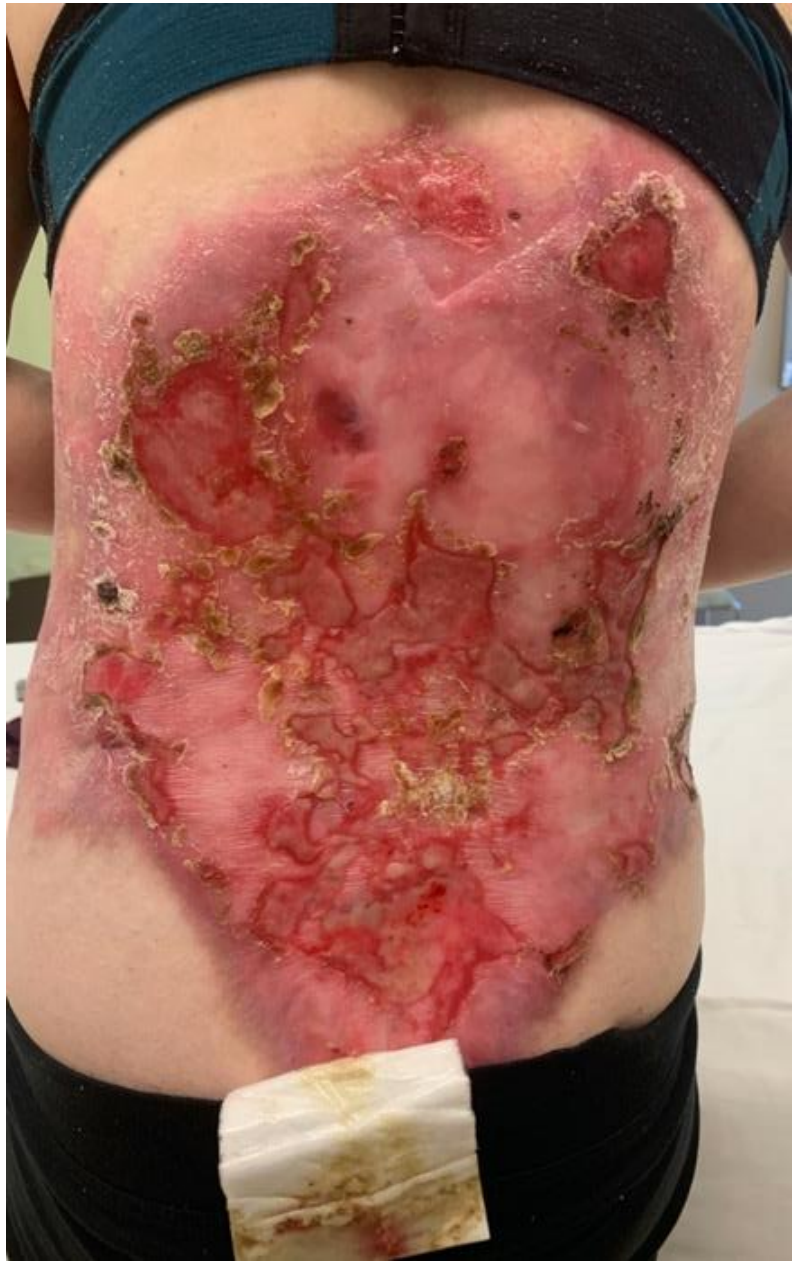
US families characterized the economic impact of managing RDEB as “high” or “severe.”

Humanistic

Many patients have anxiety and depression resulting in suicides.

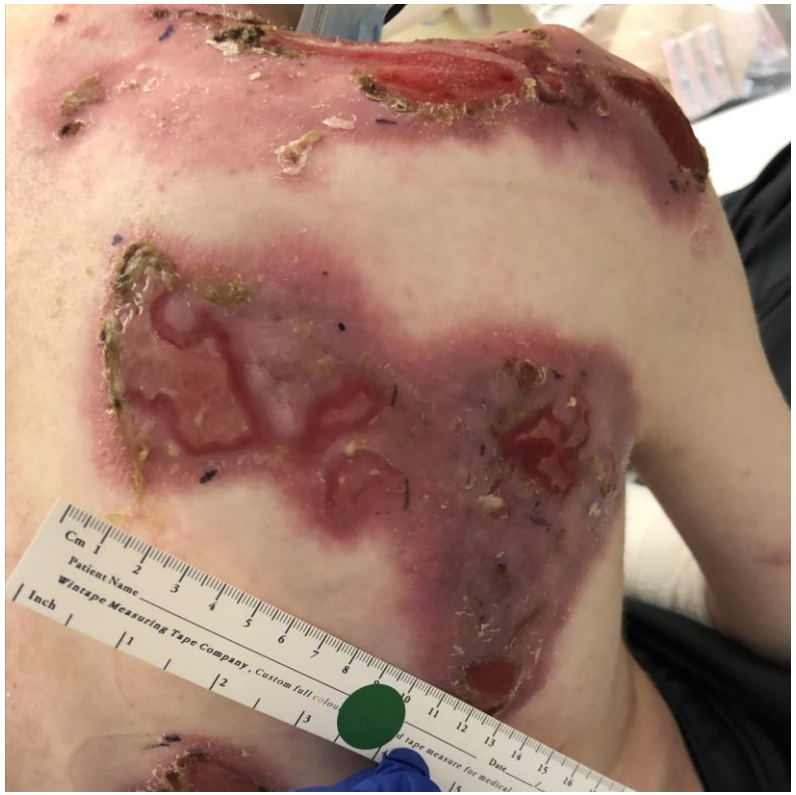
67% of divorced parents of a patient with RDEB reported the disease as a major/primary factor in divorce.

1. Tang et al. Society for Pediatric Dermatology 2020 Poster Presentation
[1]https://www.debra.org/sites/default/files/2021-05/2021%20debra%20of%20America%20Brochure-Website_0.pdf



Examples of RDEB wounds treated with pz-cel

Wounds had been open for a **median of 5 years (range of 0.5 to 21 years)** prior to study enrollment.



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Pz-cel: autologous epidermal sheet derived from patient skin and genetically engineered to secrete functional collagen 7

8-mm biopsies

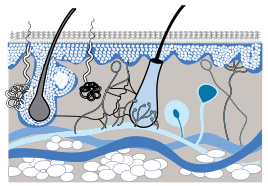
Coordinate operating room time/resources

Cell sheets ready for packaging and delivery

2-3 days | **~8 days** | **2 days** | **3-4 days** | **9 days**

Keratinocyte expansion

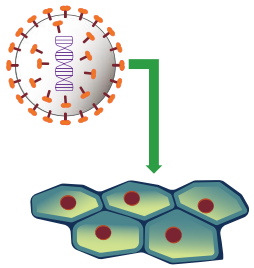
Keratinocytes extracted from biopsied samples



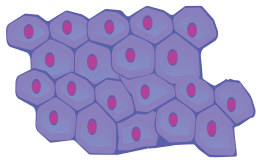
Keratinocytes grown and expanded

Retroviral transduction

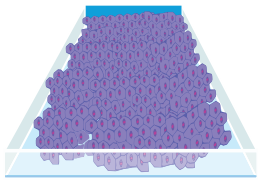
Keratinocytes transduced with corrected collagen gene



Gene-corrected cells grown to prepare for seeding



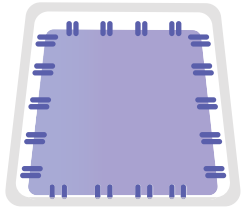
Cells seeded in plates for maturation into sheets



Maturation into epidermal sheets

Media change to support stratification

Fully formed sheets produced, 1 to 3 cell layers thick



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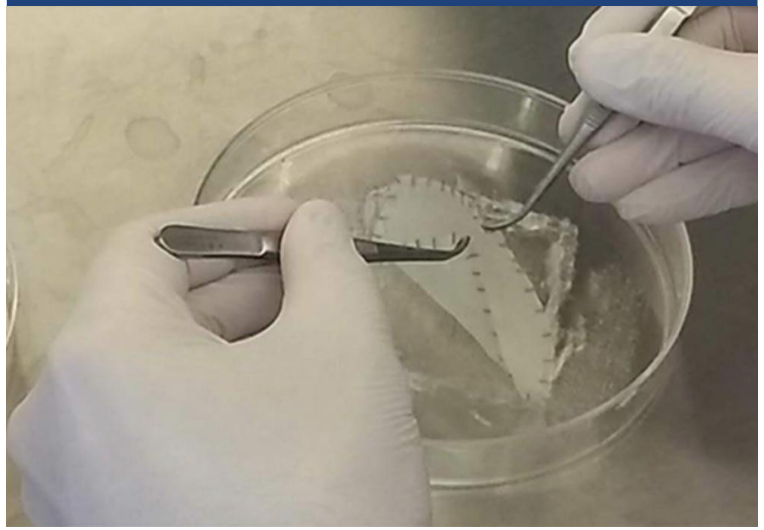
Pz-cel is applied using a surgical procedure

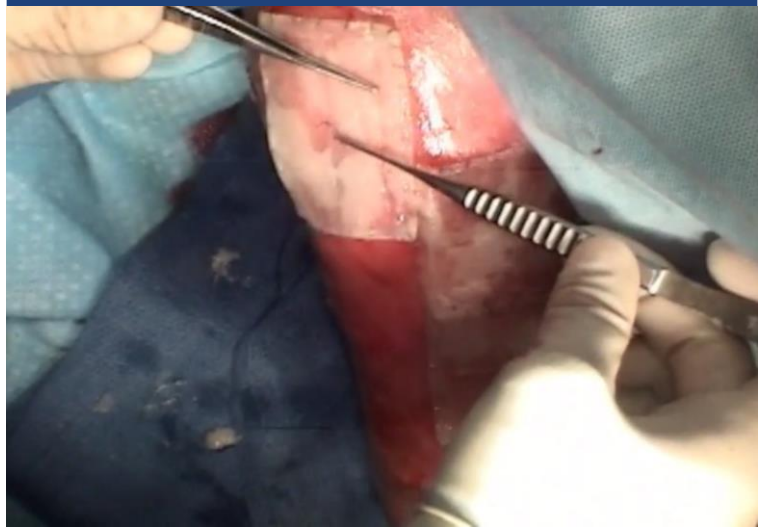
1

Wound bed prepared and antibiotics administered while surgeon prepares pz-cel for transplant.



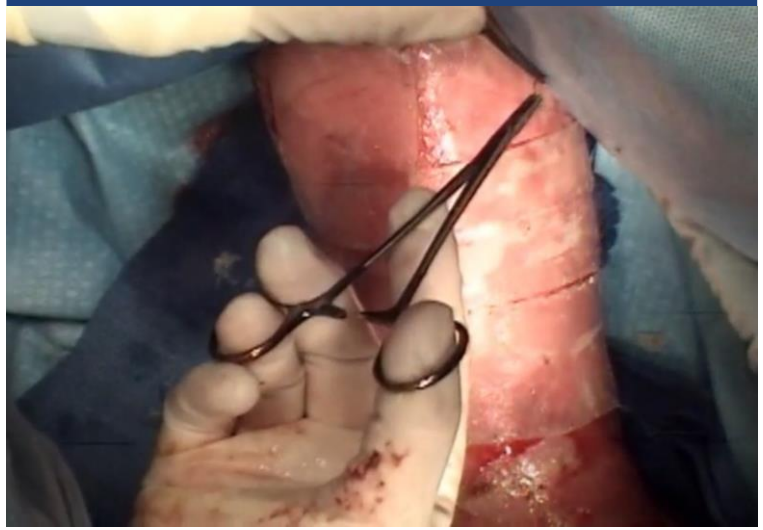
2

Surgeon applies and sutures pz-cel on wound.



3

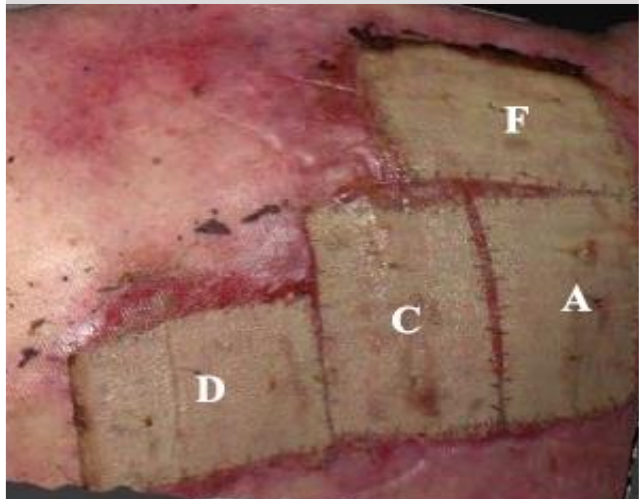
Covered wound treated with antibiotics and wrapped with gauze and surgical netting.



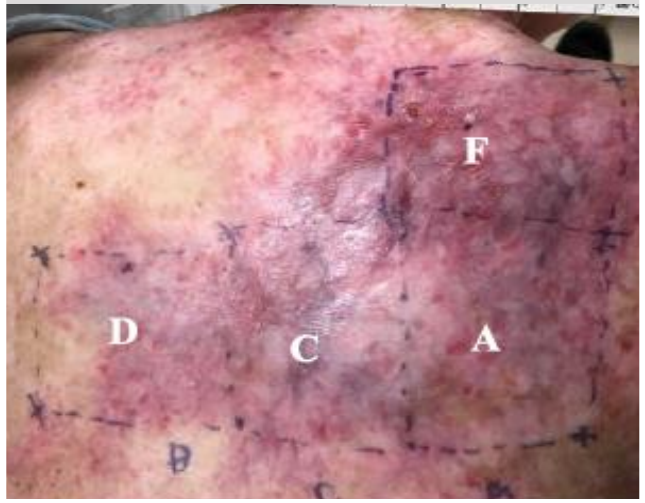
Pz-cel is the only investigational product that instantaneously covers RDEB wounds

Multiple pz-cel sheets applied in a quilt-like fashion can provide instantaneous coverage for larger wound areas

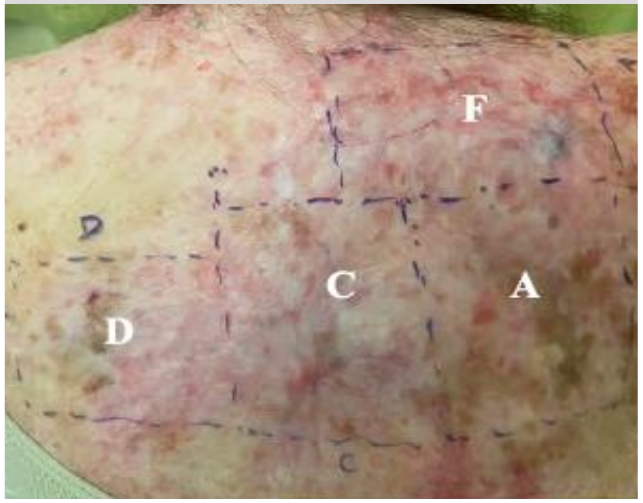
Day 0



Year 2



Year 5



- **320 cm^2** of body surface area has been instantaneously covered in a single application in clinical trials
- Trial patients are **followed for up to 15 years after a one-time pz-cel application**
- **560 cm^2** maximum wound area covered by pz-cel in a given patient with two applications (14 sheets total)

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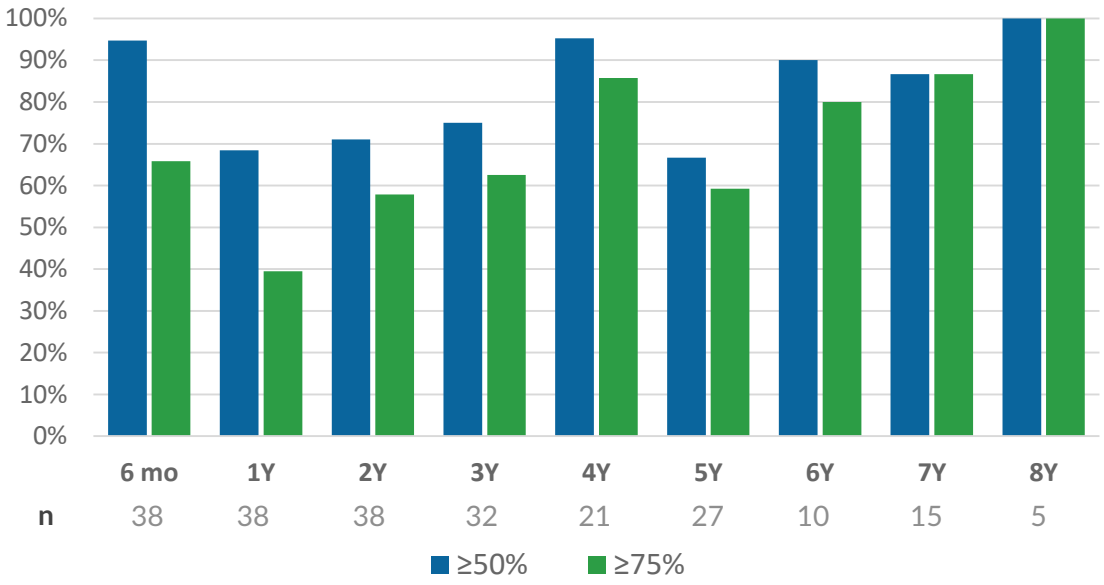
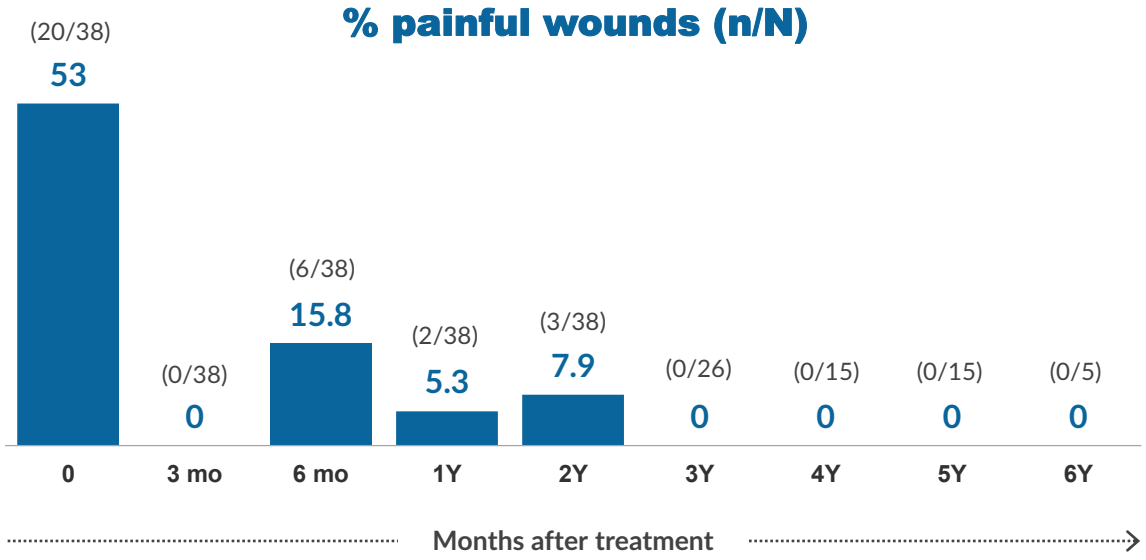
Evidence of multi-year wound healing and pain reduction with pz-cel after one-time application in phase 1/2a study

% of wounds with ≥50% or ≥75% healing



Overall wound pain relief



Key findings from phase 1/2a study

- Sustained wound healing and safety data up to 8 years at latest follow-up
- Long-term symptomatic relief, including reduction in pain
- Average surface area healed per patient: >130 cm^2 and >120 cm^2 at 3 and 6 months, respectively
- Healing of large wounds that were open for 16+ years

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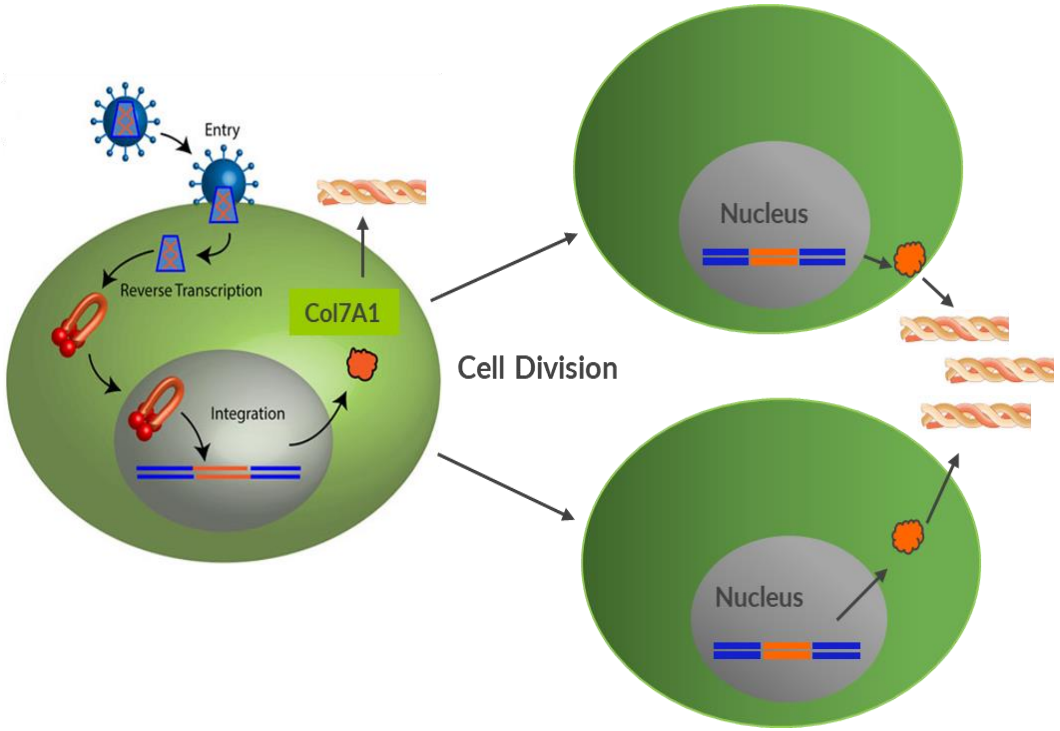
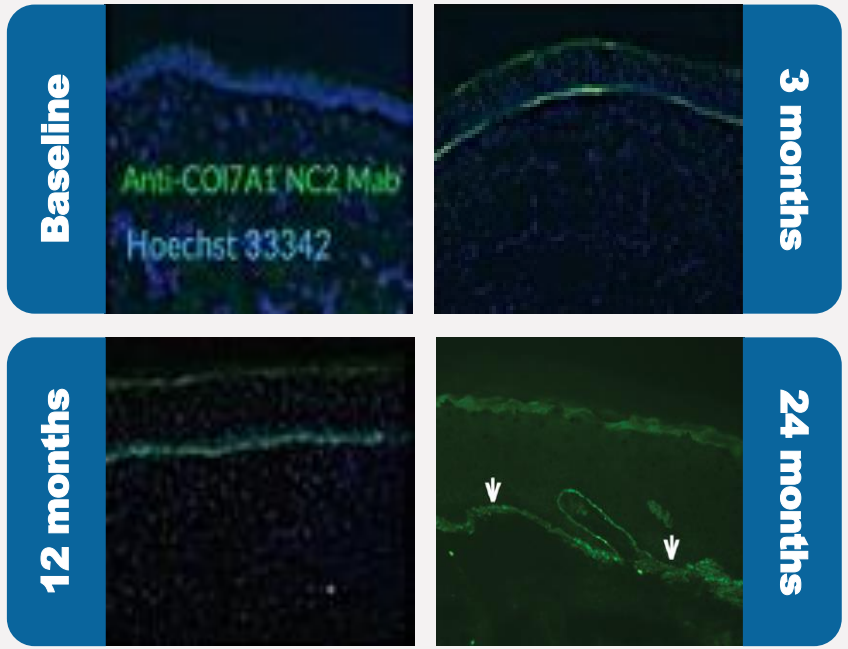
Transgene encoding functional COL7A1 integrates into host genome and is therefore stably maintained through cell division



Long-term COL7A1 expression at treated site detected by immunofluorescence



Eichstadt et al. JCI Insight 2019

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Phase 3 VITAL study results

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VIITAL study summary

Phase 3 randomized, intrapatient, controlled trial conducted at Stanford and UMass Memorial

Eligibility

- Aged ≥6 years with confirmed RDEB
- Two or more matched large chronic wounds[1] per patient
- No evidence or history of SCC in the area that would undergo pz-cel application

43 large wound pairs randomized
14 nonrandomized wounds[2]
across 11 patients

1:1 randomized wound pair

43 wounds pz-cel treated

43 wounds standard of care

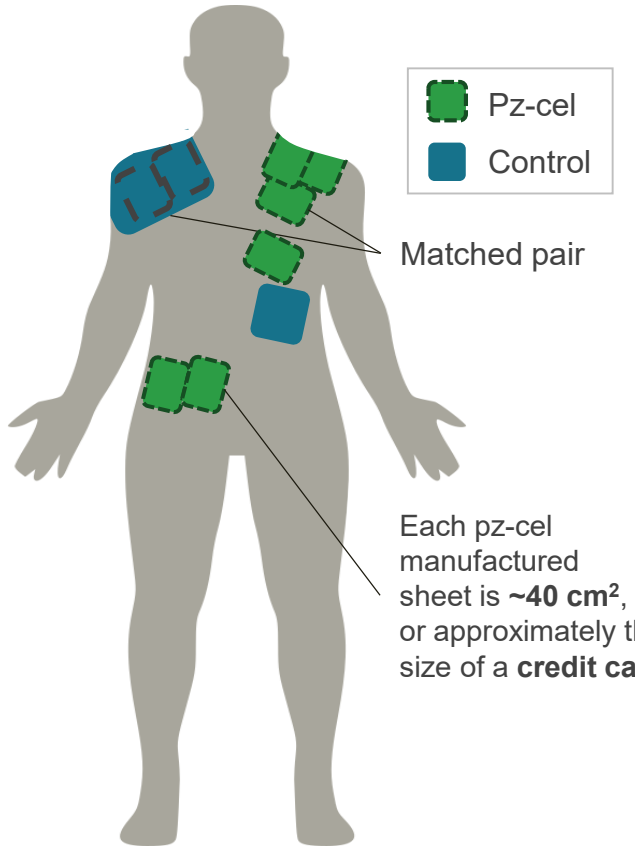
Patient follow-up at weeks 6, 12, 24, and 26

Coprimary endpoints:

- ≥50% wound healing at week 24[3]
- Pain reduction at week 24 assessed using Wong-Baker FACES scale

VIITAL study patient

(illustrative)



1. Large: ≥20 cm² surface area; chronic: open for ≥6 months.
2. Wounds with no matching controls and were not included in primary analysis.
3. Week 24 result confirmed at week 26.

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Wounds treated in VIITAL were ≥20 cm^2 and open for at least 6 months and up to 21 years

Baseline characteristics	Treated	Control
# patients treated	11	
# large chronic wounds, randomized	43	43
# large chronic wounds, nonrandomized	14	0
Patient age (mean, range)	22.5 years old, 6 to 40	
Mean body surface area covered by pz-cel per patient	>200 cm^2	n/a
Wound duration (mean years remained chronically open)	6.2	6.3
Baseline pain severity (0-10 scale)	5.12	4.38

Key inclusion criteria

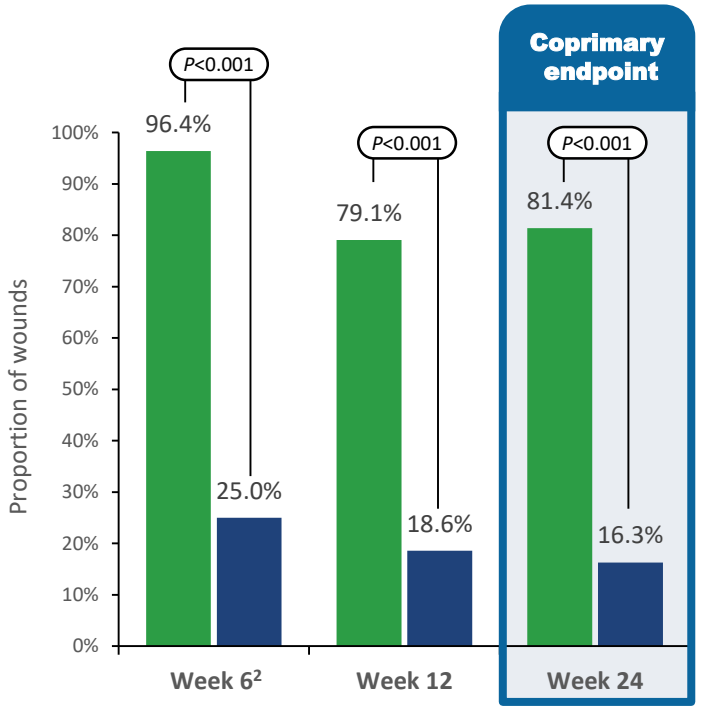
- Wound **≥20 cm^2**
- Patient **aged ≥6** years
- 2 confirmed **RDEB C7 mutations** with recessive inheritance patterns
- Wounds **open for ≥6 months**

Key exclusion criteria

- Current evidence or a history of **SCC** in the area that will undergo pz-cel application
- Evidence of systemic **infection**

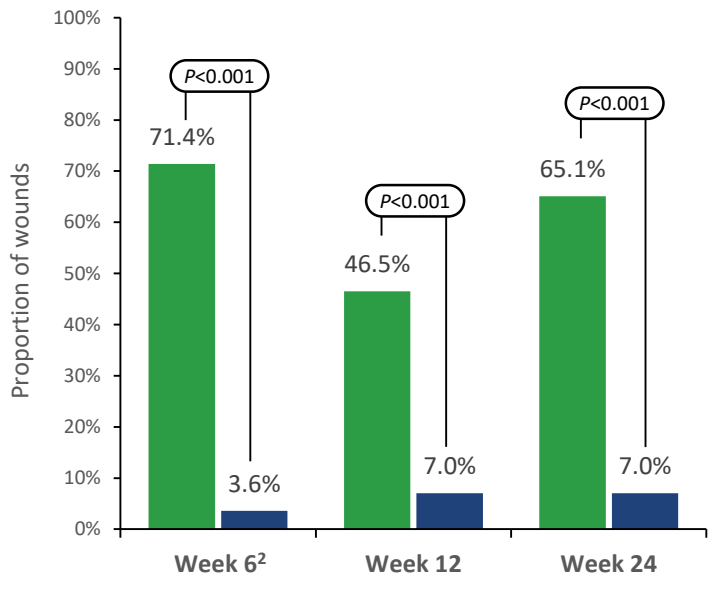
Pz-cel improved wound healing as early as 6 weeks and across all time points

≥50% wound healing from baseline



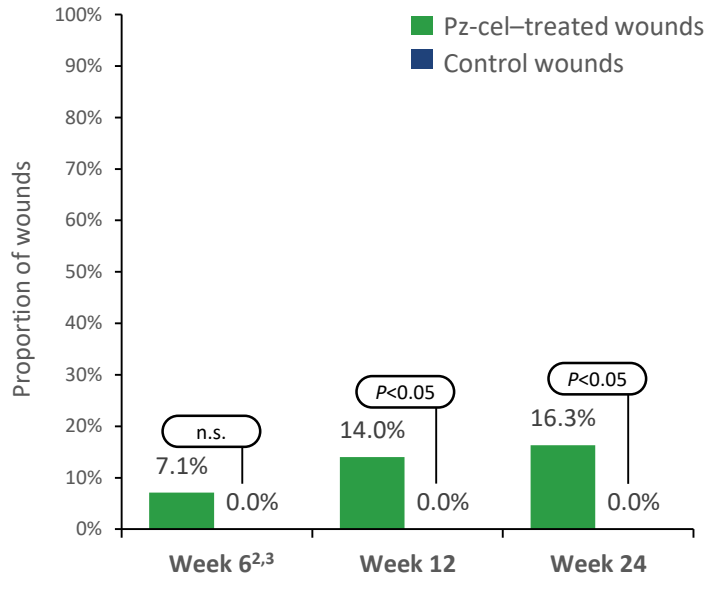
n	27	7	34	8	35	7
N	28	28	43	43	43	43

≥75% wound healing from baseline



n	20	1	20	3	28	3
N	28	28	43	43	43	43

Complete wound healing[1] from baseline



n	2	0	6	0	7	0
N	28	28	43	43	43	43

Wounds demonstrating healing at week 24 were required to be confirmed ≥2 weeks later to be included.
1. Complete wound healing was defined as re-epithelialization with no drainage or erosion and presence of only minor crusting. **2.** Post hoc endpoint. **3.** Missing data were not imputed; observed case only.
Abbreviations: n, number of wounds in healing improvement category; N, number of total wounds with non-missing healing improvement category; n.s., not significant.

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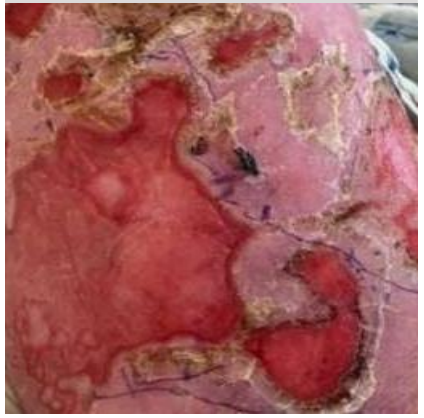
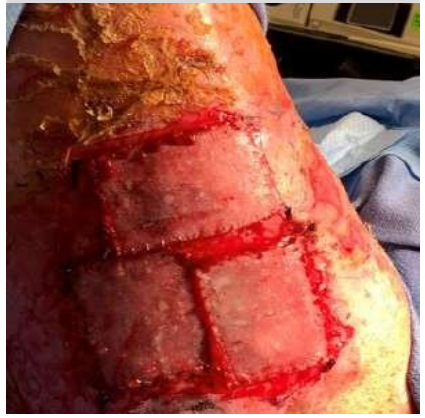
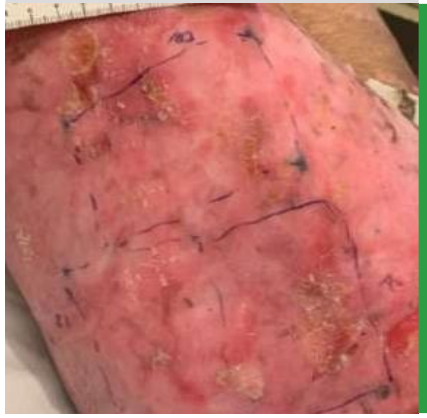
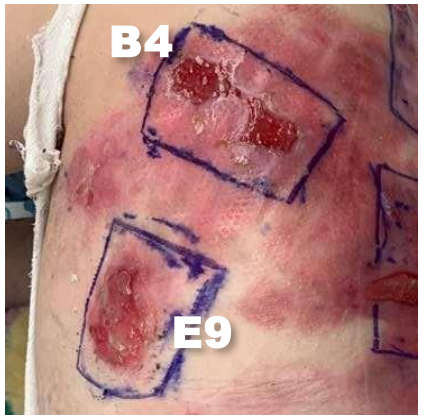
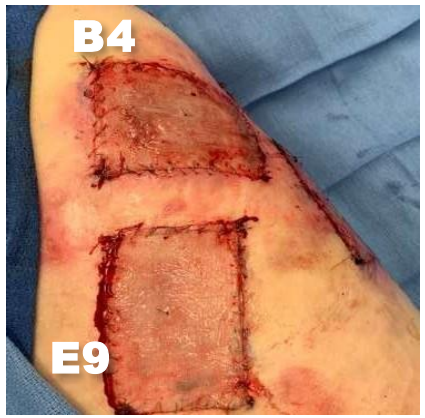
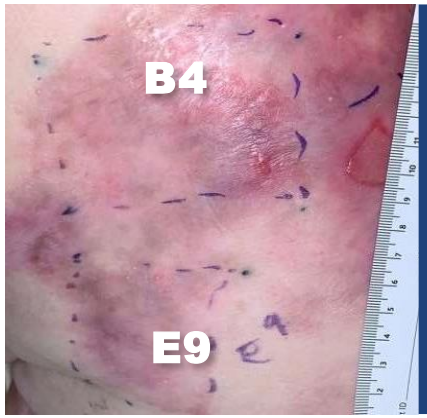
Examples of ≥75% and complete wound healing after pz-cel treatment

Baseline	Surgery	Week 24	
			Upper left thigh
			Upper trunk

Tattooed wounds scored as ≥75% healed but not complete wound healing at week 24

Wound healing measurement

- Wounds in VIITAL trial were investigator-assessed based on predefined criteria to score healing
- Complete healing was defined as complete re-epithelialization with no drainage or erosion, no major crusting

B4 scored as ≥75% healed at week 24; E9 scored as complete wound healing at week 24

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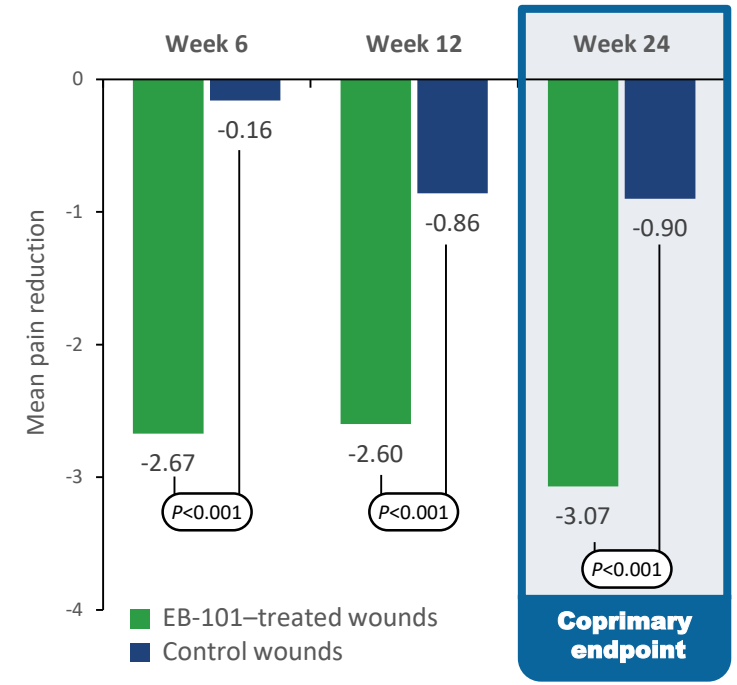
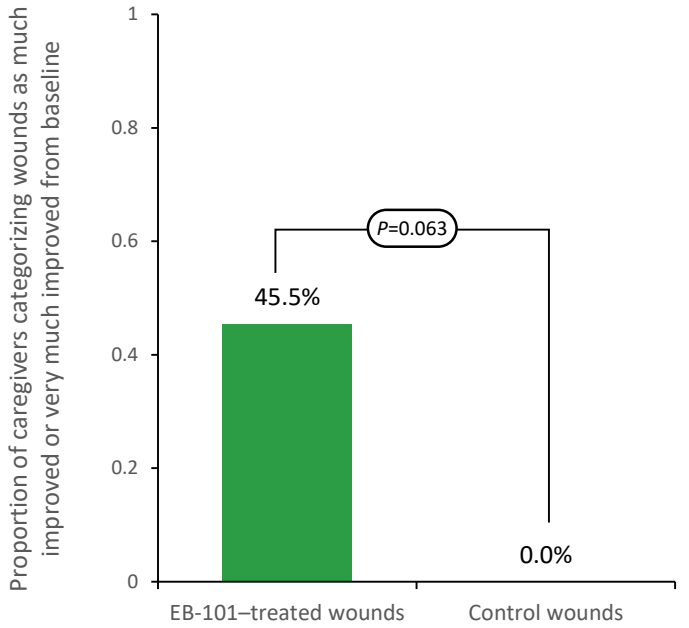
Significant reduction in pain reported with pz-cel[1]

Pain reduction from baseline during clinic visits



N_1	39	37	43	42	43	42

Much or very much improved CrGI-Pain scores at week 24[2]



n	5	0
N_1	11	11

Additional pain measures

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

Pain was also assessed using PROMIS,[3] with a significantly greater improvement in pain quality-sensory scores achieved with pz-cel treatment.

1. Pain was assessed via the Wong-Baker FACES scale or numeric rating scale. For every postbaseline assessment, the pain reduction was calculated as baseline pain score minus the postbaseline pain score.
2. Each caregiver gave 2 responses on the CrGI-Pain, 1 for all pz-cel–treated wounds (randomized and nonrandomized) and the other for all control wounds.
3. Change 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.
Abbreviations: CrGI, Caregiver Global Impression; n, number of caregiver responses; N_1, total number of wounds with non-missing pain reduction score; PROMIS, Patient-Reported Outcomes Measurement Information System.

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Pz-cel had a favorable safety profile with no serious treatment-emergent adverse events (TEAEs) related to study treatment in clinical trials

System organ class preferred term Wound type	Patients n (%)
Injury, poisoning, and procedural complications	7 (38.9)
Procedural pain	7 (38.9)
Treated study wound	1 (5.6)
Control study wound	1 (5.6)
Non-study wound	6 (33.3)
General disorders and administration site conditions	3 (16.7)
Local reaction	3 (16.7)
Treated study wound	2 (11.1)
Control study wound	0 (0.0)
Non-study wound	1 (5.6)
Infections and infestations	3 (16.7)
Wound infection	3 (16.7)
Treated study wound	1 (5.6)
Control study wound	0 (0.0)
Non-study wound	2 (11.1)
Skin and subcutaneous tissue disorders	2 (11.1)
Pruritus	2 (11.1)
Treated study wound	1 (5.6)
Control study wound	0 (0.0)
Non-study wound	2 (11.1)

Adverse reactions are sorted by descending frequency of system organ class and preferred term.
Adverse events were coded using MedDRA 23.0.

- Across phase 1/2a and VIITAL phase 3 studies, 99 total wounds were treated in 18 patients; data presented here are aggregated from these studies and the long-term follow-up
- 10 patients (55.6%) had adverse reactions related to pz-cel
- 2 TEAEs with a fatal outcome, 1 event each of sepsis and failure to thrive, both were unrelated to study treatment
- No serious TEAEs or TEWAEs related to pz-cel were reported
- No instances of positive replication-competent retrovirus (RCR) results were reported

Market opportunity for pz-cel and commercial launch preparedness

We estimate >1500 treatment opportunities for pz-cel in RDEB based on current prevalent pool

3850 Patients based on genetic modeling[1]

~1200 US patients with DEB already identified; 90% RDEB

2 treatment cycles per patient with RDEB, on average (see next slide for assumptions)

Pricing

expected per each treatment cycle, in line with value of a one-time therapy offering multiyear clinical benefit

Payer willingness

to cover RDEB lives[2]

Estimated peak annual revenue of >$500M

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. 2023 US. Payor Research conducted by Trinity Research Partners

On average, two pz-cel treatment cycles needed to cover all existing large chronic wounds

Body surface area (BSA) assumptions		Source
Avg. human BSA	17,000 cm^2	18,000 cm^2 (men) or 16,000 cm^2 (women)[1]
Avg. BSA of patient with RDEB	~10,200 cm^2	~60% of a normal human[2]
% BSA wounded	>30% (10%-80%)	Patient and caregiver survey[3]
% RDEB wounds that are large and chronic	40%	Natural history study[4]
Non-flexion areas (eg, trunk, thighs, back, etc)	80%	Pz-cel suitable/assumption
Therefore, BSA covered by large chronic wounds	**~960 cm^2**	
Pz-cel BSA coverage (12 sheets)	**480 cm^2**	

We believe an average patient with RDEB will require ~2 treatment cycles to cover their existing wound areas. Additional pz-cel treatment may be needed for new wounds arising from previously untreated areas and has not been factored into our analysis.

1. Bender, Arnold E. & David A. Bender. Body Surface Area. A Dictionary of Food and Nutrition. New York: Oxford University Press, 1995.
2. EB Research Partnership. https://www.ebresearch.org/dystrophic.html
3. Bruckner et al. Orphanet Journal of Rare Diseases 2020 (https://doi.org/10.1186/s13023-019-1279-y)
4. Solis et. al., J. Am Acad Dermatol; 2021 85(5): 1296-1298

Abeona THERAPEUTICS

Pz-cel offers a uniquely differentiated value proposition

Instantaneous closure of large RDEB wound areas

Demonstrated **significant pain reduction** in wounds

Multiple years of wound healing after **one-time application**

Improved quality of life outcomes, including itch and blistering

Well tolerated with years of documented safety profile

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Key opinion leader (KOL) advisory board[1] reinforces pz-cel value proposition

1. KOLs confirm high unmet need in patients with RDEB and their families due to severe wound burden
   - The strategy for skin care has been largely protection and risk management (SCC screening, infection)
2. Experts note critical need for a treatment that provides long-term wound closure
3. Anticipate using multiple therapies, including pz-cel, based on RDEB wound characteristics
   - Clear preference for using pz-cel for large wounds
   - Advisors are willing to treat patients as young as possible for greatest impact
4. 65% of wounds demonstrating “>75% wound healing” for large chronic wounds viewed as highly meaningful
5. KOLs were happy to note that all 4 patients in phase 3b study were repeat patients now receiving pz-cel for previously untreated wound areas
6. Experts express hope that wound closure and pain reduction will lead to additional long-term benefits

> “There is a notion that maybe it’s not just one single magic bullet, but maybe we can use these treatments in a complementary way.”
>
> **– US KOL**

> “I think it's much more important to focus on after a wound closes, does it remain closed for the next 2 years with incidental trauma or activities of daily life? I think that'll be the most important measurement.”
>
> **– US KOL**

1. Abeona conducted physician advisory board meeting in September 2023.

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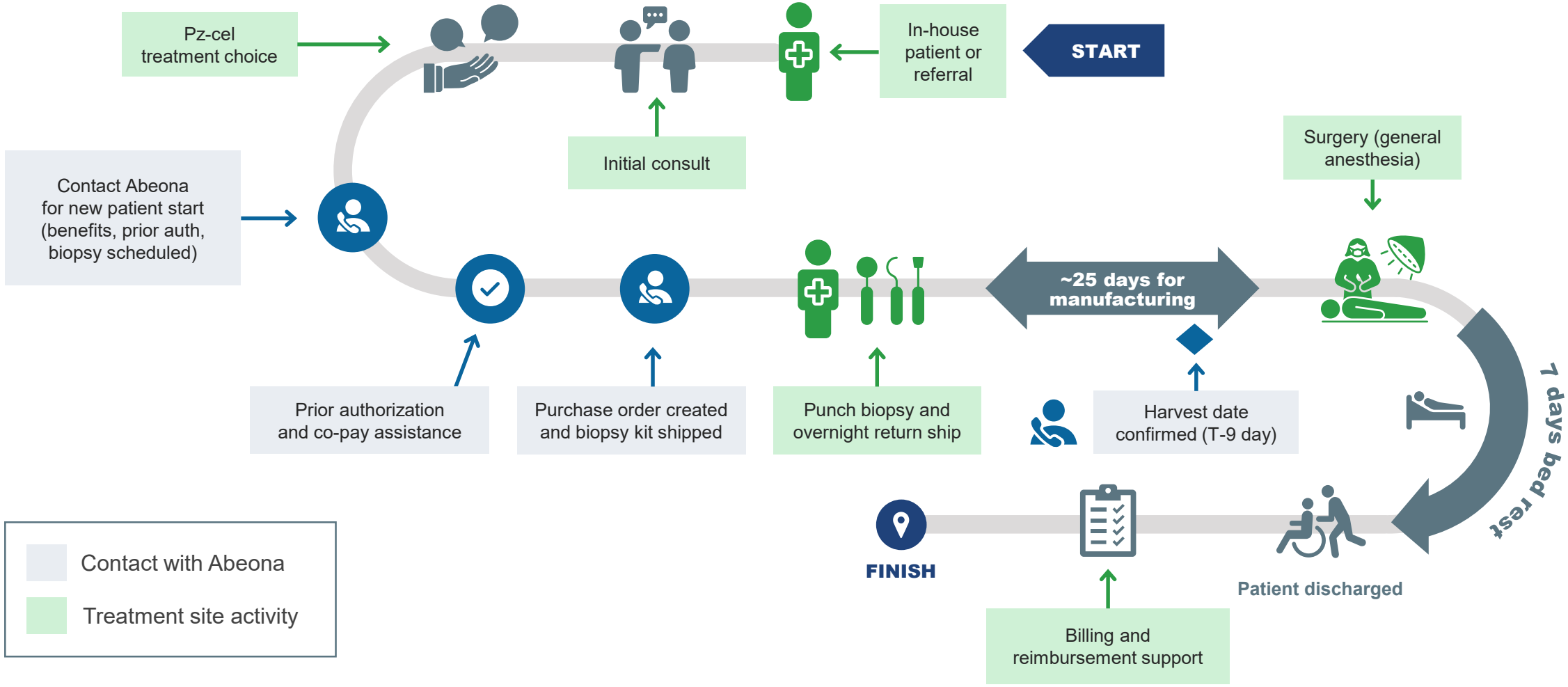
Pz-cel patient journey is typical of autologous cell therapies



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Pz-cel launch strategy focuses on partnering with select number of high-volume EB centers and expanding reach over time

Strategic goal at launch

- Onboard 5 to 7 high-volume EB centers
- Identify 1 to 2 patients per center per month following initial ramp-up
- Ensure timely market access

Executing on multiple launch readiness activities:

Provider and site onboarding

- Multiple sites have begun onboarding process and discussion with multidisciplinary teams initiated
- Interest from burn centers in making pz-cel procedure available for RDEB patients
- Initiated peer-to-peer education

Payer engagement

- Field-based teams in place to engage private and Medicaid payers
- Developing patient assistance programs to deliver best-in-class patient and provider experience

Highly focused, nimble field team and infrastructure being built to support May 25, 2024 PDUFA

Abeona THERAPEUTICS

Focusing payer engagement on key commercial and Medicaid payers to enable pz-cel coverage post-approval

"You know how they say a picture is worth 1000 words? Yeah, that's pretty impressive...getting 50% [wound healing] is probably enough to impress the committee."

– STATE MEDICAID PAYER

"The number of patients that had greater than 50% wound healing, that 81.4% is fairly telling. The reduction in pain scores is fairly significant…. The fact that this can reduce pain speaks to the value of the product."

– HOSPITAL ADMIN

"This is most like the other one-time gene therapies like SPINRAZA, HEMGENIX, or even CAR Ts since they are inpatient."

– NATIONAL MCO PAYER

- Feedback from payers and healthcare providers supports positive coverage decisions for pz-cel, and pricing in line with the value of a therapy that provides instantaneous coverage for large wound areas and demonstrates years of wound healing and pain reduction even in the "toughest-to-treat" RDEB wounds after one-time application
- Continuing payer engagement to ensure broad access and reimbursement while recognizing value of pz-cel

Pipeline

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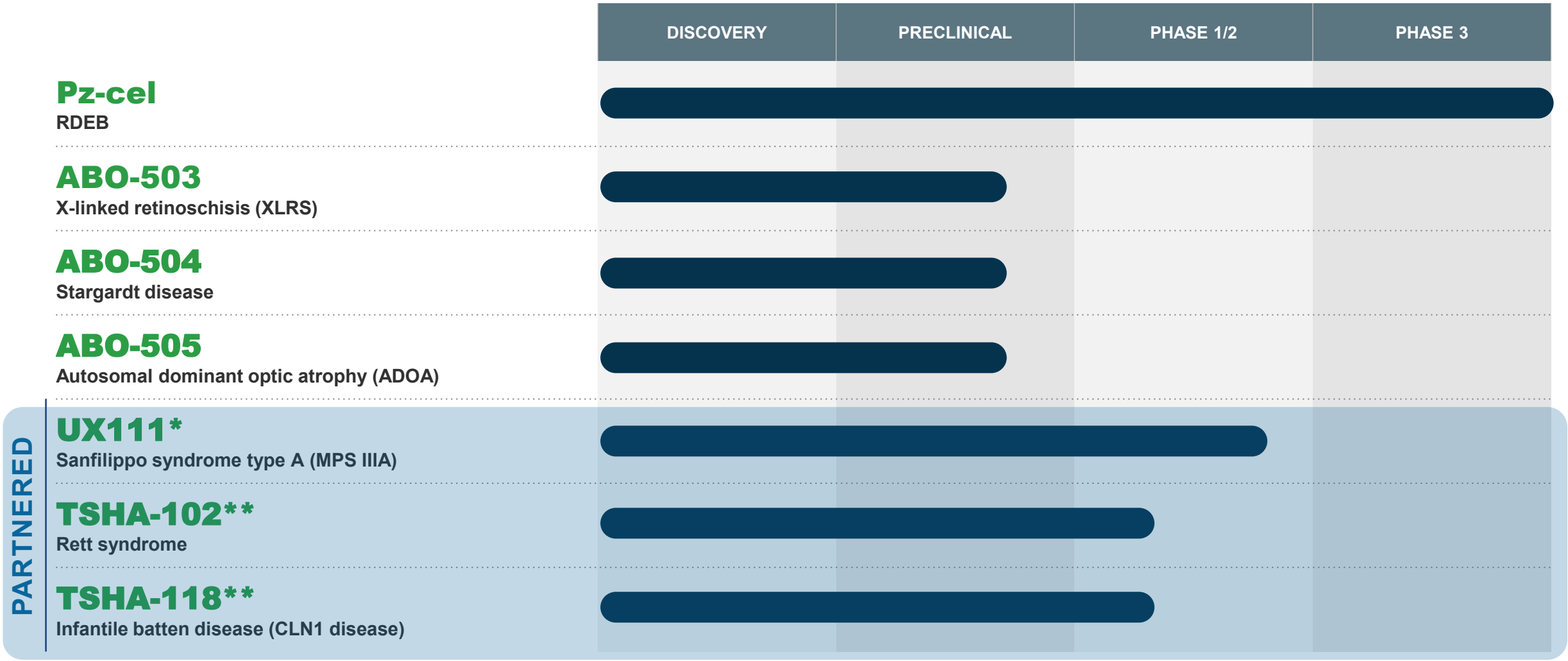
Pipeline of differentiated cell and gene therapies



Partnered with *Ultragenyx Pharmaceutical Inc. **Taysha Gene Therapies, Inc.

Abeona THERAPEUTICS

Differentiated solutions for genetic ophthalmic conditions with high unmet medical need that leverage novel capsids and gene constructs

AAV capsids

- Novel AIM™ capsids designed to selectively target delivery of genetic payloads with **improved tissue/cell tropism** and **reduced immune response** compared with natural AAV capsids
- AIM™ and Abeona-developed novel capsids designed for distinct routes of administration including **systemic, ocular, and direct CNS administration**
- Investigating AIM™ and Abeona-developed capsids in **ophthalmic conditions—including Stargardt disease, X-linked retinoschisis, and autosomal dominant optic atrophy—**each with 5000 to 15,000 estimated US prevalence
- **Licensing opportunities** for Abeona's proprietary capsids in a variety of **ocular conditions**

Gene constructs

- Abeona developed delivery of large genes utilizing AAV-packaged classical Cre-Lox system for **Stargardt disease**
- Utilizing para-retinal injection of AIM™ capsid AAV204, a potentially safer route of administration in fragile retinas, for **X-linked retinoschisis**
- Targeting of retinal ganglion cells using para-retinal injection of AAV204 capsid for **autosomal dominant optic atrophy**

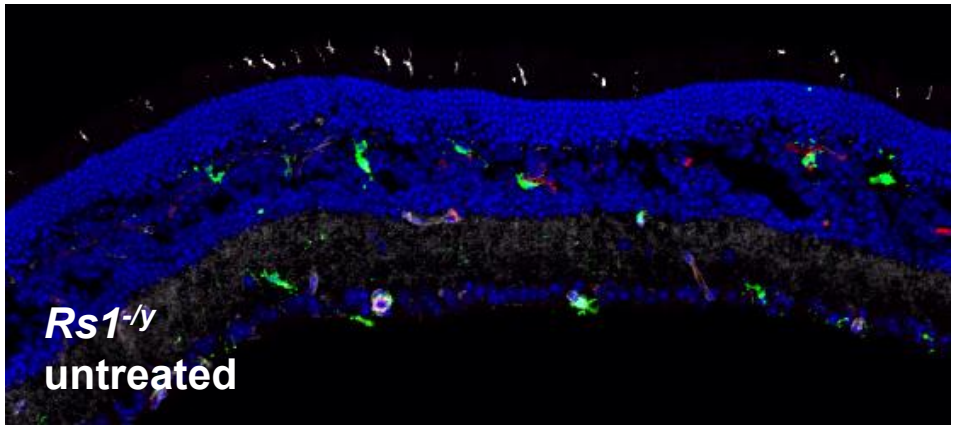
ABO-503 for X-linked retinoschisis

First-in-human opportunity in 1H 2025

"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 6 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



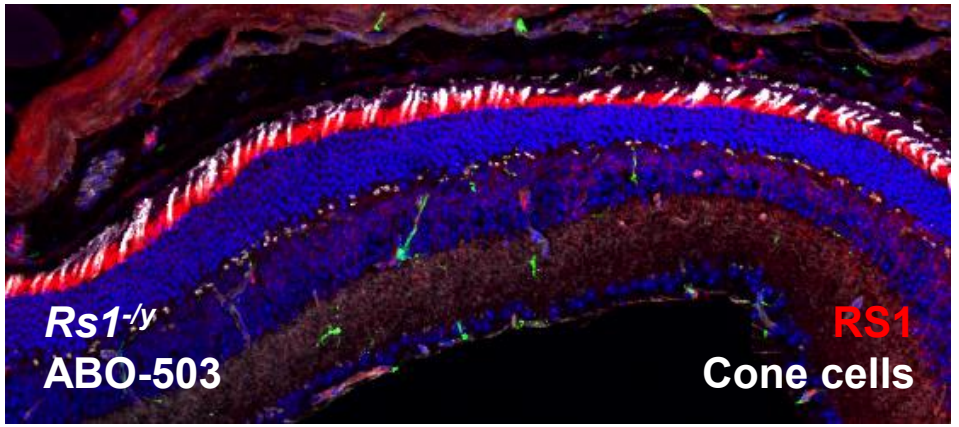


Image of mutant mice 6 months after treatment

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ABO-504 for Stargardt disease
First-in-human opportunity in 1H 2025

"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

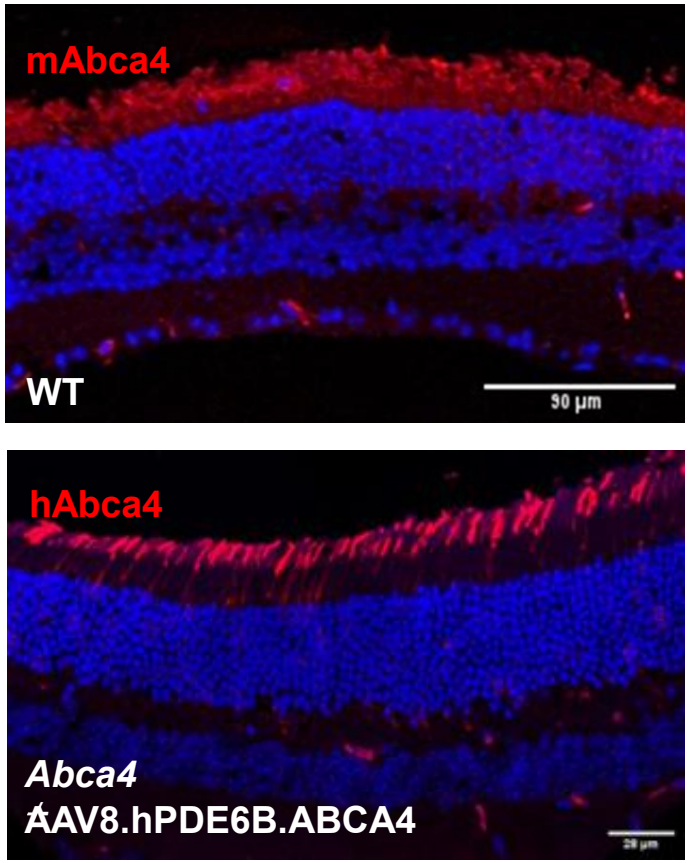

Image of mutant mice 6 months after treatment

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AAV gene therapy for autosomal dominant optic atrophy

Preclinical proof-of-concept demonstrating functional recovery in mutant mice

Key findings

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

Opa1 protein expression

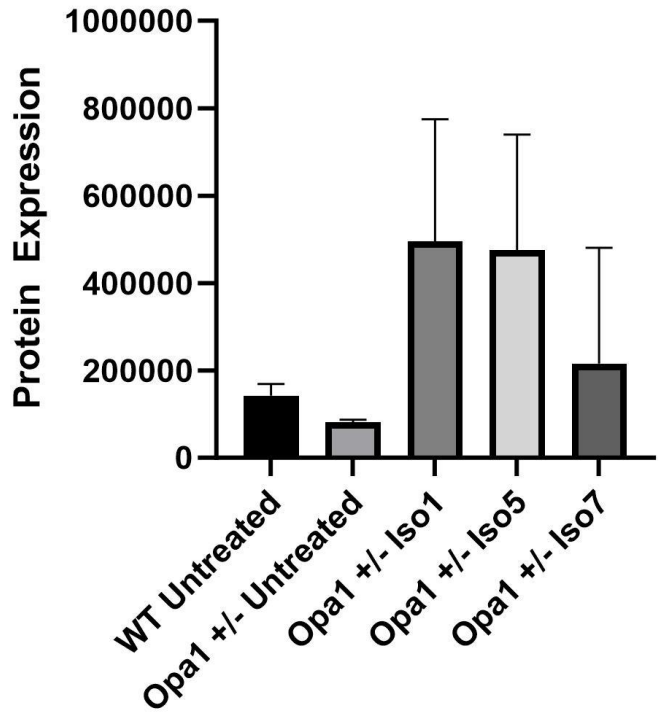

Image of mutant mice 10 months after treatment

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Anticipated milestones

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Transition from clinical to commercial stage in 2024

2023 de-risking accomplishments

- Reported positive pivotal phase 3 study results
- Completed process performance qualification
- Completed positive pre-BLA meeting with FDA
- Submitted BLA
- BLA accepted with priority review by FDA
- Initiated commercialization efforts for potential US launch

2024 anticipated milestones

- Potential approval of pz-cel and grant of priority review voucher (2Q 2024)
- VIITAL study peer-review publication (1H 2024)
- Potential commercial launch of pz-cel (2H 2024)
- Initiate AAV ophthalmology IND-enabling studies (2024)

Current cash runway well beyond expected timing of BLA approval and PRV grant