Abeona THERAPEUTICS

Prademagene Zamikeracel:

An Investigational Genetically Engineered Autologous Cell Therapy

March 19, 2024

Forward-Looking Statements Disclaimer

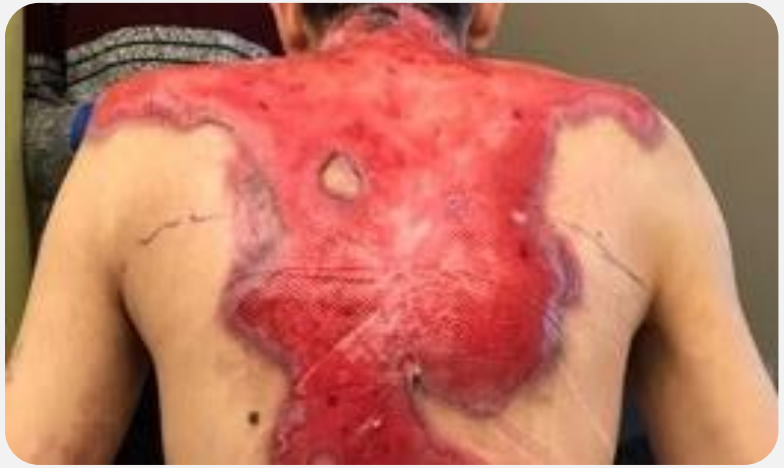
Prademagene zamikeracel is an investigational therapy that has not been approved by the Food and Drug Administration (FDA). This information is not intended to make safety or efficacy claims about prademagene zamikeracel or be used to make clinical decisions.

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.

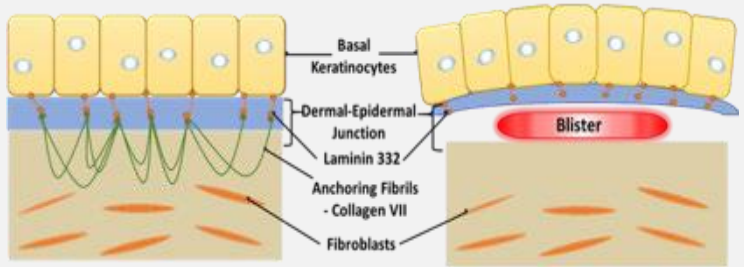
Recessive Dystrophic Epidermolysis Bullosa (RDEB) is a painful, lifelong debilitating connective tissue disease

Disease Overview

- **Ultra-rare genetic disease** with an estimated fewer than 1,500 patients in the US[1]
- Caused by the absence of the patient's functional copy of ***COL7A1* gene** which encodes **collagen 7**, a protein necessary to form anchoring fibrils and keep patient's skin intact
  - On average >30% of patient's body surface covered in wounds[3]
  - Chronic wounds remain open for years with up to 90% risk of developing **squamous cell carcinoma (SCC)**[2]
  - 76% likelihood of death by the age 40[4]
- Significant clinical, economic and humanistic burden on patients and caregivers



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

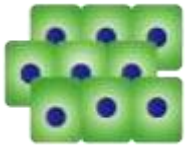


[1] Fine. JAMA Dermatol. 2016 Nov 1;152(11):1231-1238.
[2] South, et al. J Invest Dermatol. 2023 Nov;143(11):2108-2119.
[3] Bruckner, et al. Orphanet J Rare Dis. 2020 Jan 3;15(1):1.
[4] Tang, et al. Orphanet J Rare Dis. 2021 Apr 13;16(1):175.

Prademagene zamikeracel (pz-cel) is an autologous cell-based gene therapy that targets the underlying cause of RDEB disease

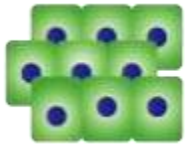
1. BIOPSY

Two 8 mm skin biopsies are taken at the clinic and sent to Abeona for manufacturing



2. KERATINOCYTE CELL ISOLATED & TRANSDUCED

Transduction with functional COL7A1 transgene



3. CELL EXPANSION

Transduced cells are expanded

4. MATURATION

Gene-corrected cells are grown into epidermal sheets (5.5 x 7.5 cm); up to 12 sheets may be produced per manufacturing run

5. SURGICAL APPLICATION

Pz-cel sheets are returned to the hospital after approximately 25 days and surgically applied onto wounds

***COL7A1* gene transduction** using a retrovirus vector (LZRSE-COL7A1) resulting in adequate expression and secretion of **collagen 7 protein**

VIITAL Study Summary

Phase 3 randomized, intra-patient, 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:

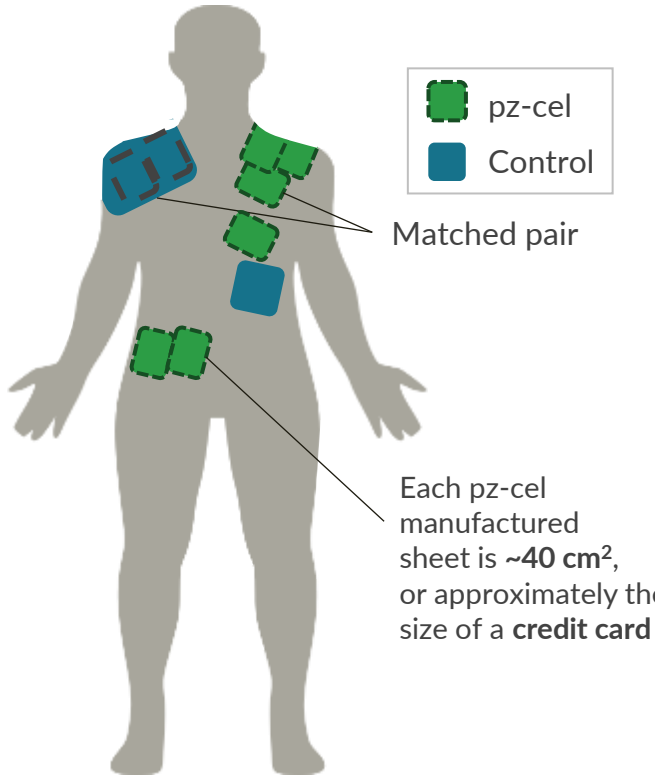
- ≥50% wound healing at week 24[3]
- Pain reduction at week 24 assessed using Wong-Baker FACES® Pain Rating Scale

Secondary endpoint:

- Complete wound healing at weeks 12 and 24

VIITAL study patient

(illustrative)



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

[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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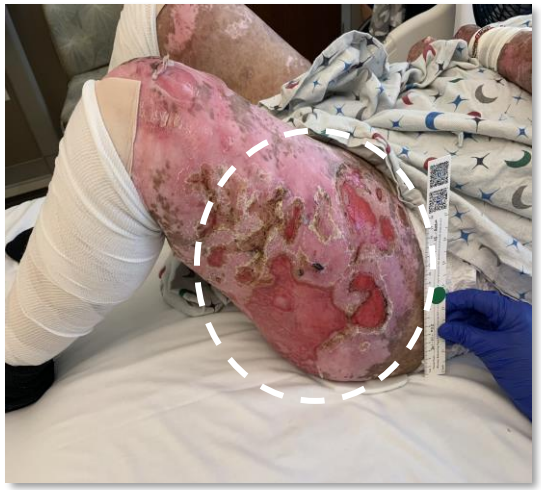
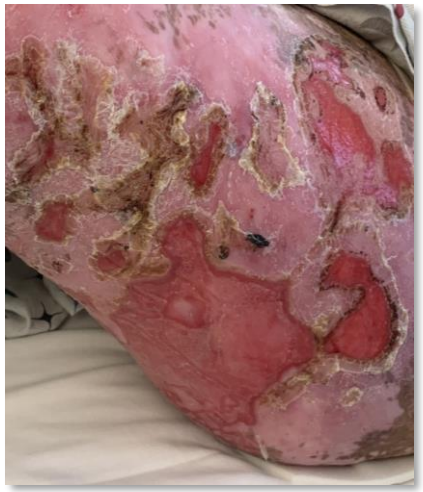
In clinical trials, genetically engineered epidermal sheets were surgically applied under general anesthesia within 36 hours of manufacturing, followed by 5-7 days of wound immobilization

Pz-cel Surgical Application

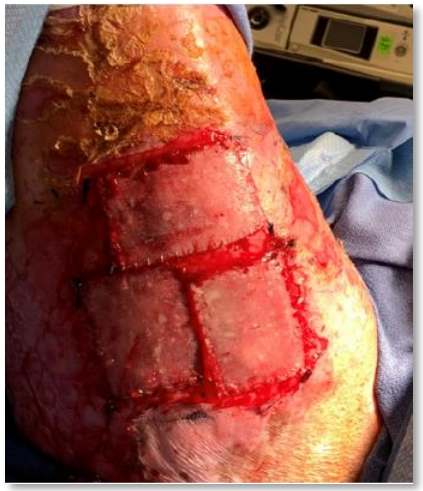
1. Wound bed prepared and antibiotics applied while surgeon prepared pz-cel for application
2. Surgeon sutured pz-cel on wounds
3. Covered wounds were treated with antibiotics and wrapped with gauze and surgical netting



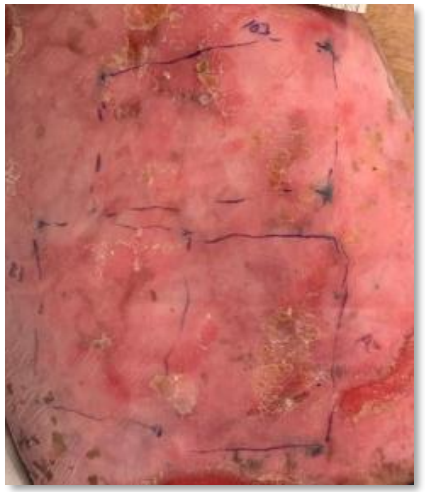
Patient from Pivotal Phase 3 Study



Baseline



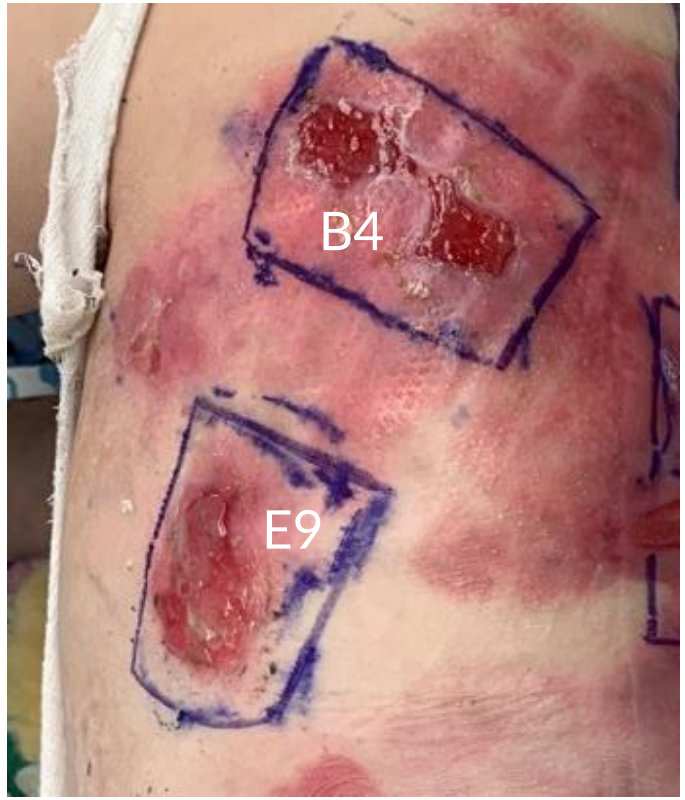
Surgery



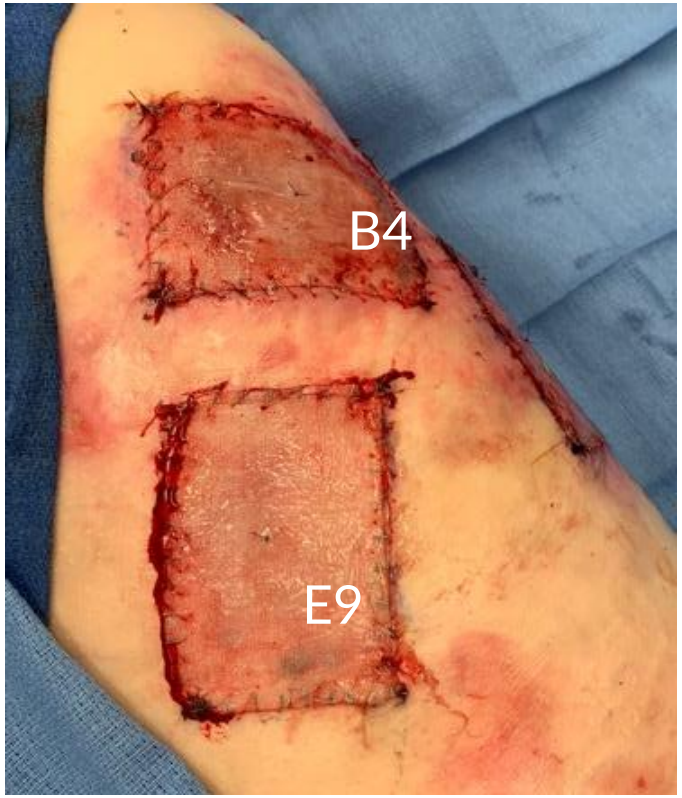
Week 24

Examples of ≥75% and complete wound healing after pz-cel treatment (upper back)

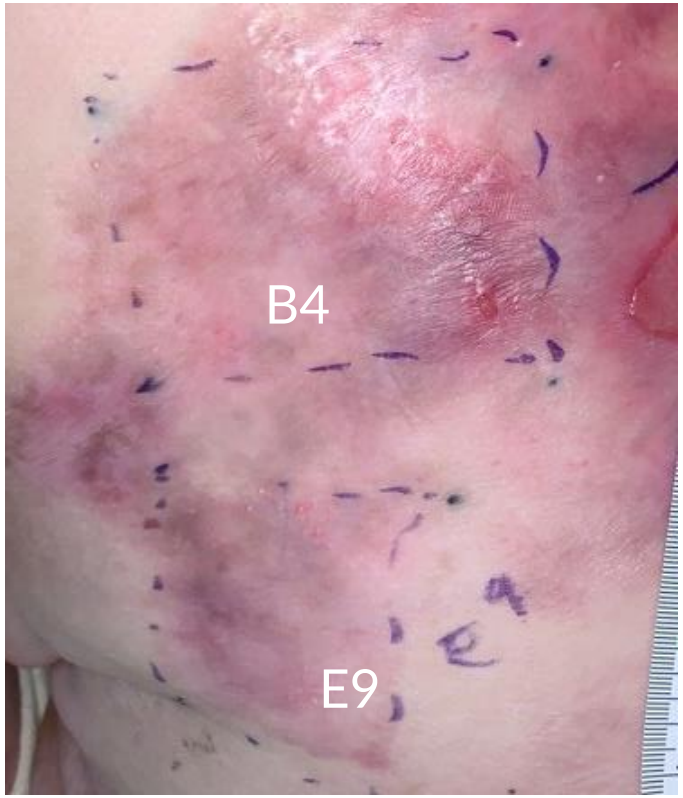
Baseline



Surgery



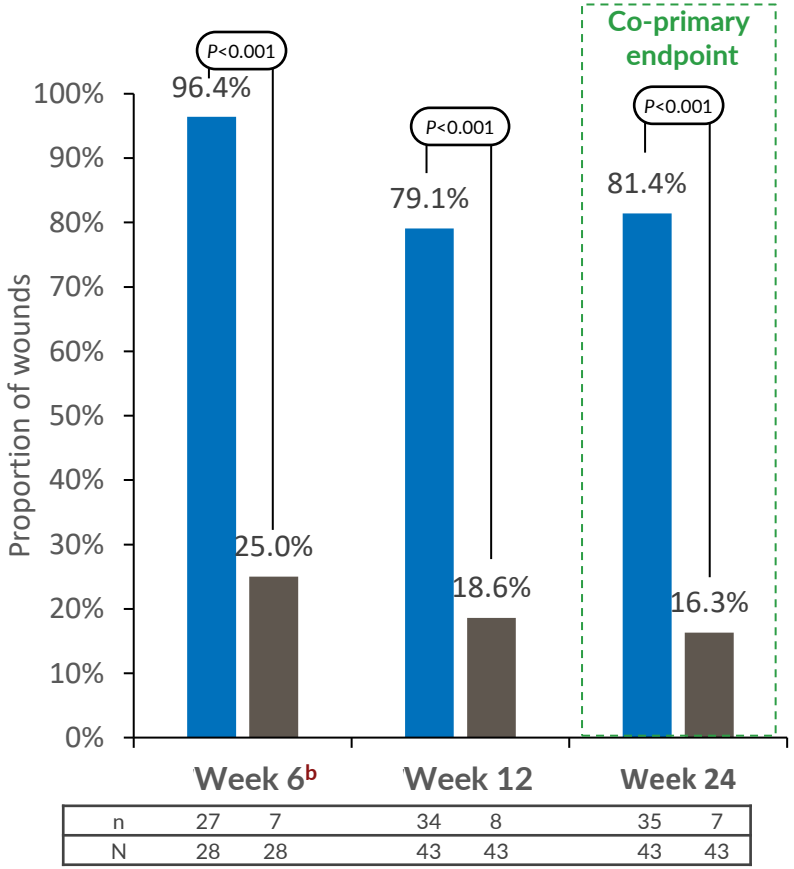
Week 24



B4 scored as >75% healed
E9 scored as complete wound healing

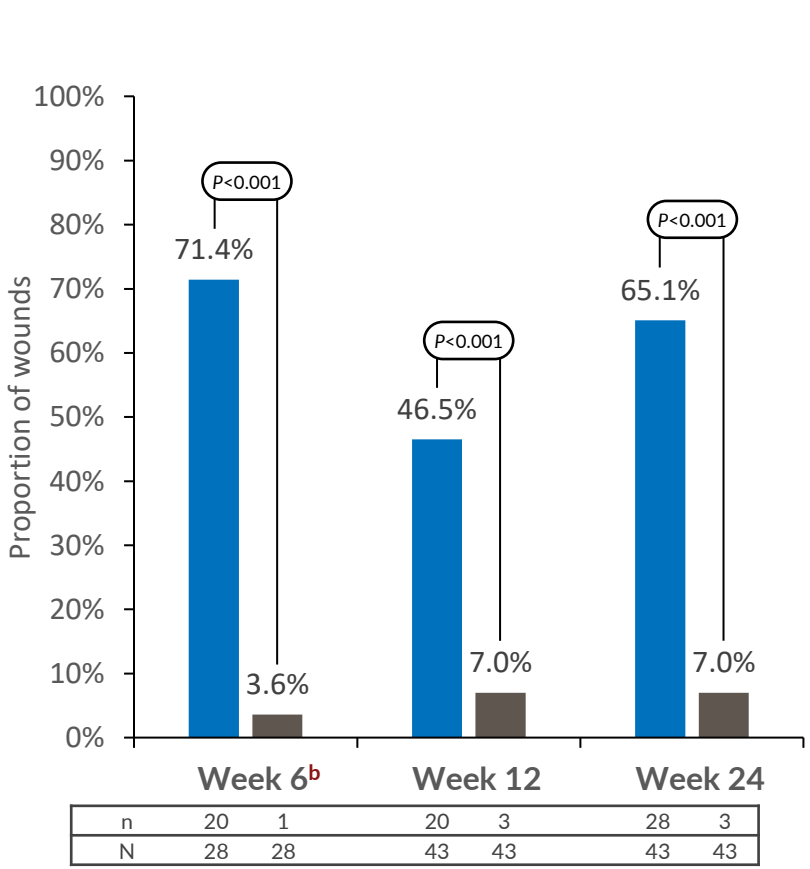
Pz-cel improved wound healing in as early as 6 weeks and across all timepoints in Phase 3 clinical trial

≥50% Wound Healing
from baseline



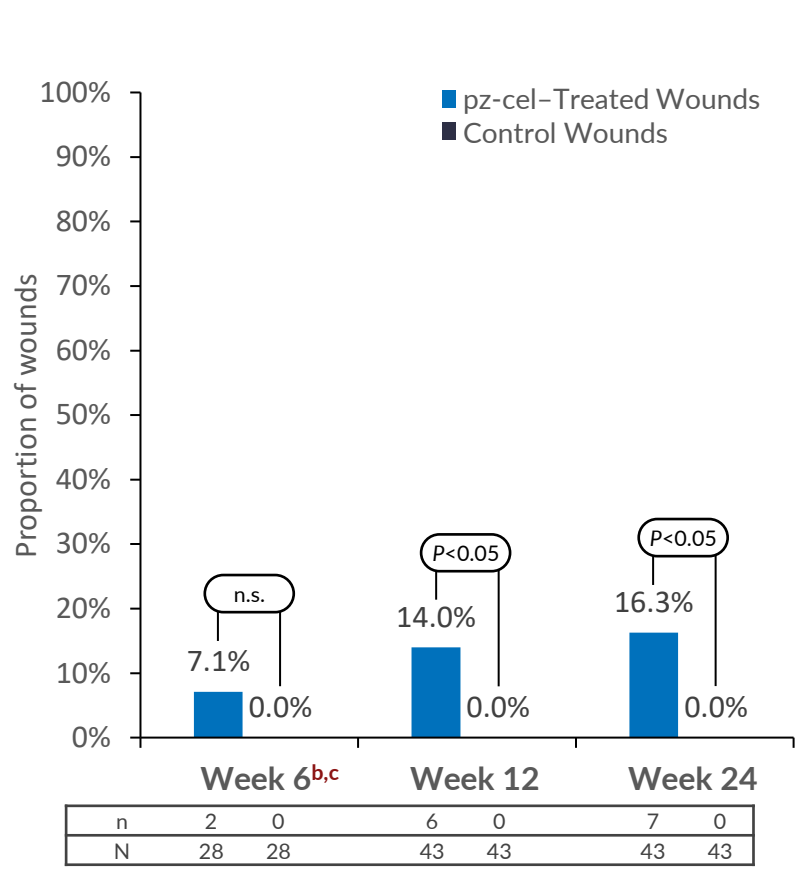
n	27	7	34	8	35	7
N	28	28	43	43	43	43

≥75% Wound Healing
from baseline



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

Complete Wound Healing[a]
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.
[a] Complete wound healing was defined as re-epithelialization with no drainage or erosion and presence of only minor crusting. [b] Post hoc endpoint. [c] Missing 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.

Source: Data on file (Phase 3 VIITAL study results)

Pz-cel had a favorable safety profile with no serious TEAEs related to study treatment in clinical trials

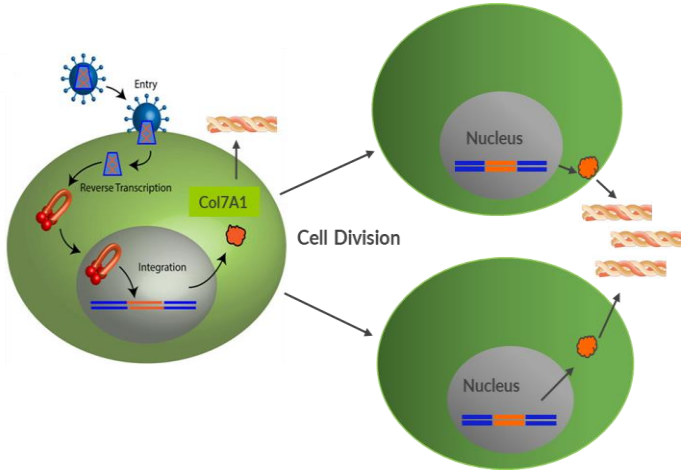
System Organ Class Preferred Term Wound Type	Patients n (%)
Injury, Poisoning and Procedural Complications	7 (38.9)
Procedural Pain	7 (38.9)
Treated Study Wound	1 (5.6)
Control Study Wound	1 (5.6)
Non-Study Wound	6 (33.3)
General Disorders and Administration Site Conditions	3 (16.7)
Local Reaction	3 (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

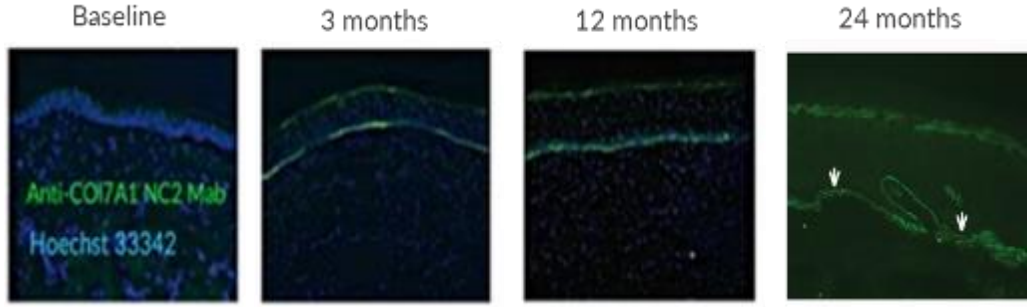
- Across Phase 1/2a and VIITAL Phase 3 studies, 99 total wounds were treated in 18 patients; aggregated data from these studies and the long-term follow-up are shown
- 10 patients (55.6%) had adverse reactions related to pz-cel
- 2 TEAEs with a fatal outcome, 1 event each of sepsis and failure to thrive, 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

TEAE = treatment-emergent adverse events; TEWAE = treatment-emergent wound adverse events
Sources: So et al. Orphanet J Rare Dis. 2022 Oct 17;17(1):377.; Data on file (Phase 3 VIITAL study results)

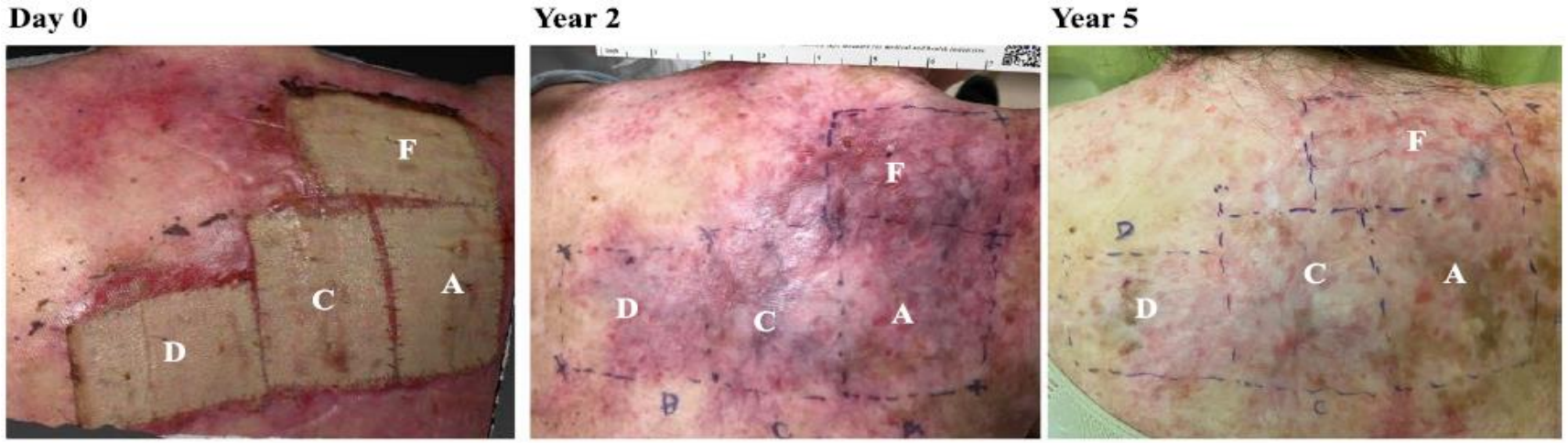
Long-term follow-up: Evidence for collagen expression and durable wound healing (up to 8 years) following one-time surgical application with pz-cel[1]



Long term *COL7A1* expression at treated site detected by immunofluorescence



Example from pz-cel Phase 1/2a Study



[1] So et al. Orphanet J Rare Dis. 2022 Oct 17;17(1):377.

Documentation

- What diagnoses are associated with RDEB?
  - Q81.2 Epidermolysis bullosa dystrophica; Q81.8 Other epidermolysis bullosa; Q81.9 Epidermolysis bullosa, unspecified
- Where would prademagene zamikeracel be documented in the medical record for individuals?
  - Documentation would be found in an Operative Report or Procedure Note since it will be surgically applied in a surgical suite under general anesthesia
- What are the different naming conventions for the drug/device/technology/service or procedure?
  - prademagene zamikeracel gene-corrected autologous cell therapy, pz-cel, pz-cel cell therapy, pz-cel, EB-101, EB-101 gene-corrected autologous cell therapy, EB-101 gene-corrected keratinocyte sheets, EB-101 LZRSE-COL7A1 engineered autologous epidermal sheets

Summary

- Biologics License Application (BLA) under Priority Review by the FDA with Prescription Drug User Fee Act (PDUFA) date of **May 25, 2024**
- Pz-cel is a genetically engineered autologous cell therapy that contains a functional *COL7A1* transgene delivered by a retrovirus vector (RVV) for the treatment of wounds associated with RDEB
- Pz-cel uses a patient's own skin cells (biopsy) as starting material and introduces the missing *COL7A1* transgene into keratinocyte cells *ex vivo* to express collagen protein from mature epidermal sheets
- Genetically engineered epidermal sheets are then surgically applied onto a patient's wound bed under general anesthesia, followed by a 5 to 7 days of wound immobilization – all of which requires extensive healthcare resource utilization
- If approved for use, we anticipate pz-cel will be available at 5 to 7 medical centers in the US
- 15-year patient follow-up will be conducted for pharmacovigilance like other cell & gene therapies