

Sustained Wound Healing and Long-Term Safety of Prademagene Zamikeracel (Pz-cel) in Recessive Dystrophic Epidermolysis Bullosa (RDEB): Five-Year Results from the VIITAL™ Phase 3 Trial

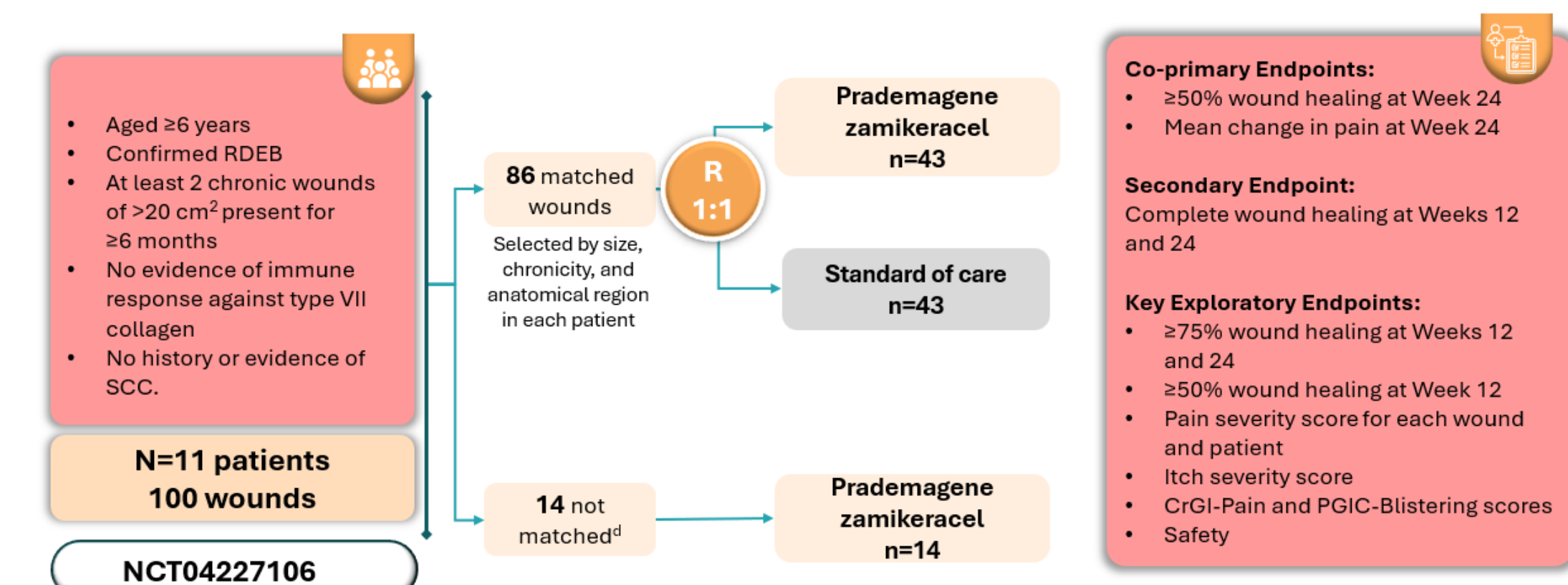
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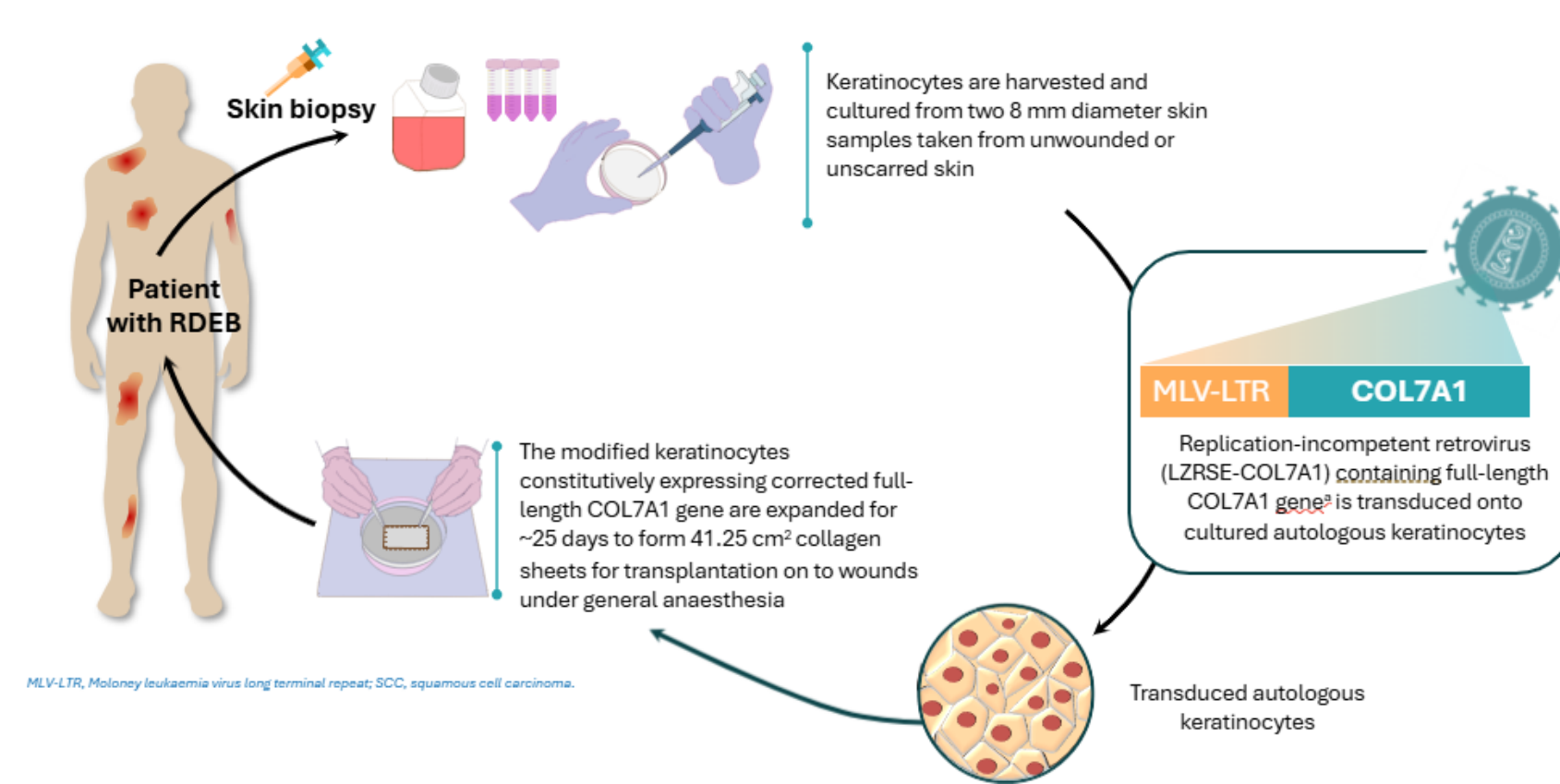
Background

Recessive dystrophic epidermolysis bullosa (RDEB) is a rare genetic skin disease caused by mutations in the *COL7A1* gene, encoding type VII collagen, the major component of anchoring fibrils that tether the epidermis to the dermis. Individuals with RDEB have fragile skin and most develop large, chronic wounds. Prademagene zamikeracel (pz-cel) is an autologous *COL7A1* gene-modified cellular sheet sutured onto large, chronic RDEB wounds. The aim of the VIITAL study was to evaluate the efficacy and safety of a one-time surgical application of pz-cel in wound healing and pain reduction. On the back of robust phase 3 results, pz-cel (Zevaskyn®) was FDA-approved for the treatment of RDEB wounds on April 28, 2025; however, durability of response and long-term safety beyond the 24-week cutoff have not been elucidated.

VIITAL – A Phase 3 Study in Patients with RDEB Study Design



Pz-cel Mechanism of Action



Results of five-year follow-up

Cohort Characteristics at Baseline and Post Treatment Follow Up

	1	2	3	4	5	6	7	8	9	10	11
Age	17	18	23	30	20	32	40	21	16	24	6
Sex	M	M	F	F	F	M	F	M	F	F	F
Race/Ethnicity	White/Not Hispanic or Latino	White/Not Hispanic or Latino	Unknown/Not Hispanic or Latino	White/Not Hispanic or Latino	White/Not Hispanic or Latino	White/Not Hispanic or Latino	White/Not Hispanic or Latino	White/NR	White/Not Hispanic or Latino	White/Not Hispanic or Latino	White/Hispanic or Latino
Grafting Year	2020	2020	2020	2021	2021	2021	2021	2022	2022	2022	2022
Follow up duration (as of 3/31/2026 (months))	71.8	61	60.2	60.2	56.6	48.7	34.6	48	48.1	15	49.5
Serum replication competent retrovirus (RCR)	No	No	No	No	No	No	No	No	No	No	No
Development of SCC at graft site	No	No	No	No	No	No	No	No	No	No	No

Wound Healing (Co-Primary Endpoint)

- Between January 2020 and March 2022, 15 subjects were screened and 11 were enrolled (43 pz-cel treated wounds).
- As of **March 31, 2026**, the **median exposure was 49.5 months (range 15.0-71.8 months)**.
- 4/11 subjects** had at **least 60 months (5 years) of post treatment follow up**.
- At months 12, 18, 24, 30 and up to Month 60 post-treatment, a great proportion of treated wounds demonstrated sustained efficacy following a one-time surgical application of pz-cel.

Subject	Anatomic Site	Month 6	Year 1	Year 2	Year 3	Year 4	Year 5
1	Left anterior shoulder						
	Right upper arm						
	Left superior central chest						
2	Right posterior shoulder						
	Right medial scapula						
	Upper right mid back						
3	Anterior right lower leg						
	Anterior left lower leg						
	Lateral left thigh						
4	Right upper back						
	Right mid back						
	Right lower back						
5	Mid chest						
	Right flank						
	Left flank						
6	Left lateral chest						
	Left medial chest						
	Right medial chest						
7	Right lateral chest						
	Left lateral upper back						
	Left lateral upper back						
8	Right midline						
	Left upper hip						
	Left upper hip						
9	Midline upper back						
	Left lateral mid back						
	Right upper shoulder						
10	Right lateral shoulder						
	Right medial shoulder						
	Upper mid back						
11	Upper mid back						
	Right hip						
	Right lateral thigh						
11	Lower abdomen						
	Upper right arm						
	Lower right arm						
11	Right Leg						
	Right lateral thigh						
	Lower left arm						

Color legend = Dark green: complete wound healing compared to baseline; Green: 75-99% healing compared to baseline; Yellow: 50-74% healing compared to baseline; Red: less than 50% healing compared to baseline; Gray box: wound healing evaluation not available

Pain Reduction (Co-Primary Endpoint)

- Pain reduction assessed by the investigator using the validated Wong-Baker FACES Scale.
- In the phase 3 trial, the mean change from baseline to week 24 in wound pain was -3.07 with pz-cel and -0.90 in controls ($p=0.0002$).
- Sustained reduction in pain was observed at year 5 for evaluable wounds.

Pain Reduction in Pz-cel Wounds (N=43)

Month 12	21/34 (61.8)
Month 24	22/33 (66.7)
Month 48	22/30 (73.3)
Month 60	10/15 (66.7)

Proportion of Wounds with an Improvement in Pain Severity, n1/n2 (%), where n1 = Number of wounds with an improvement in pain severity (FACES) score from baseline; n2 = number of wounds with non-missing pain reduction score.

Safety

Adverse Events	Patients (N=11)	Adverse events reported in at least 10% of patients (2)	Patients (N=11)
Total Number of Adverse Events (AEs)	228	Wound infection	8 (72.7)
Patients with at least one adverse event [1]	11 (100)	Procedural pain	7 (63.6)
Mild	10 (90.9)	Skin infection	7 (63.6)
Moderate	11 (100.0)	Constipation	4 (36.4)
Severe	8 (72.7)	Upper respiratory tract infection	4 (36.4)
Life-threatening or debilitating	0	Abdominal pain	3 (27.3)
Fatal	0	Pruritus	3 (27.3)
Related to prademagene zamikeracel	4 (36.4)	Pyrexia	3 (27.3)
Leading to discontinuation of prademagene zamikeracel	0	Anemia	2 (18.2)
		Anxiety	2 (18.2)
		Back pain	2 (18.2)
		Blister	2 (18.2)
		COVID-19	2 (18.2)
		Nausea	2 (18.2)
		Oropharyngeal pain	2 (18.2)
		Seasonal allergy	2 (18.2)

- As of March 2026, there were no serious AEs related to pz-cel, instances of positive replication-competent retrovirus (RCR) or squamous cell carcinoma (SCC) in pz-cel treated wounds (11-year follow up).
- 4 events of SCC were observed in 3 subjects at non-treated sites and were deemed to be unrelated to pz-cel.

Examples of Wound Healing with Pz-cel Over Five Years

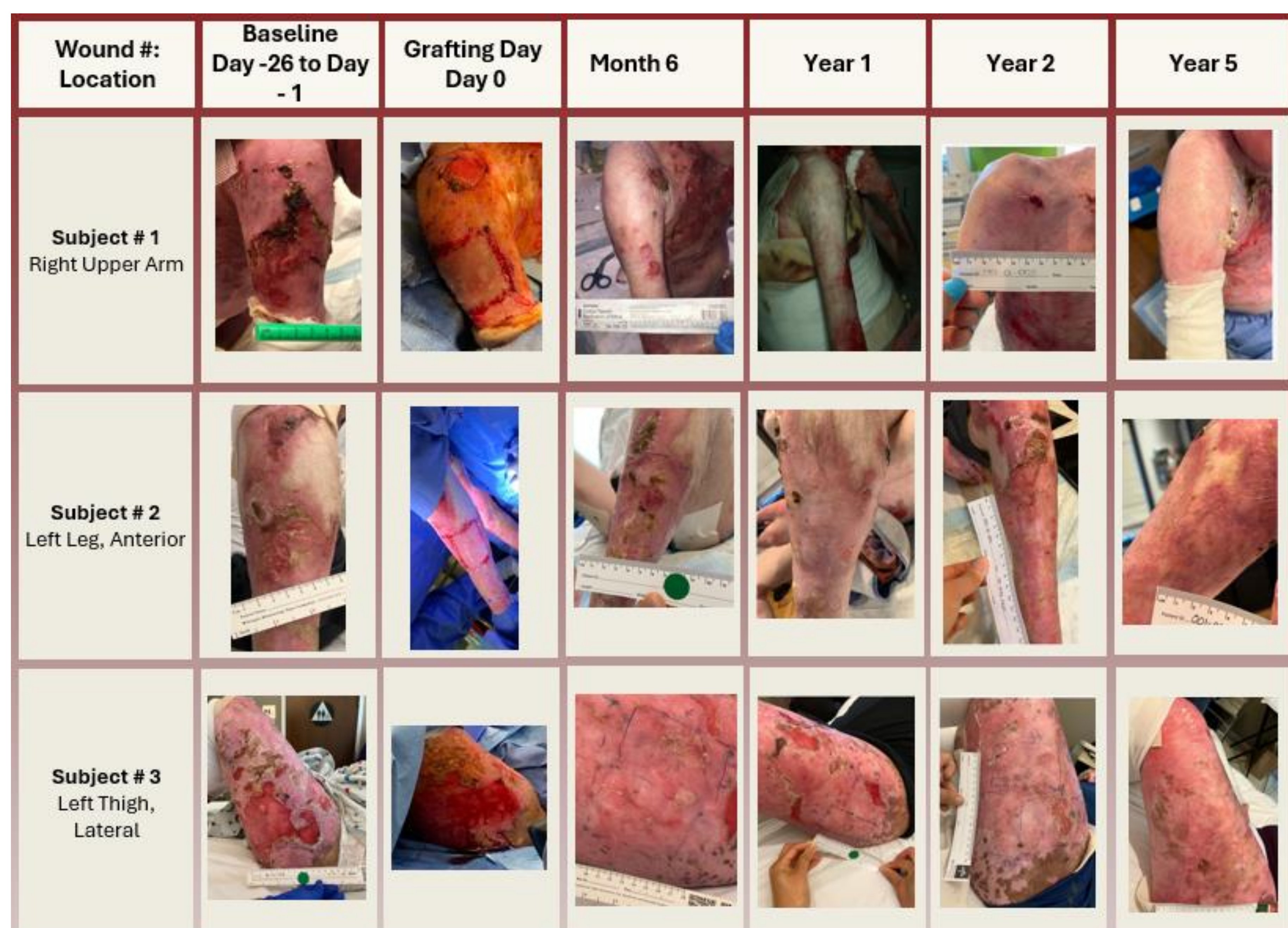


Figure 2. Representative example of treated wounds follow-up from baseline to five-year mark

Discussion

Long-term follow-up through five years demonstrated durability of response and an overall safety profile for pz-cel that was consistent with observations from the primary study phase, with no new safety signals identified.

References

- Tang JY, Marinkovich MP, Wiss K, McCarthy D, Truesdale A, Chiou AS, Eid E, McIntyre JK, Bailey I, Furukawa LK, Gorell ES, Harris N, Khosla RK, Peter Lorenz H, Lu Y, Nazarov J, Grachev ID, Moore AJ. Prademagene zamikeracel for recessive dystrophic epidermolysis bullosa wounds (VIITAL): a two-centre, randomised, open-label, intrapatient-controlled phase 3 trial. *Lancet*. 2025 Jul 12;406(10499):163-173. doi: 10.1016/S0140-6736(25)00778-0. Epub 2025 Jun 23. PMID: 40570869.
- ZEVASKYN Full Prescribing Information. Abeona Therapeutics Inc. Cleveland, OH. 2025.