Real-World Use of the Topical Gene Therapy B-VEC in Dystrophic Epidermolysis Bullosa Patients Under 6 Months of Age

Brittani Agostini[1]; Torien Ledbetter[1]; Kolleen Fitzgerald[1]; Dominic Marchese[1]; Suma M. Krishnan[1]

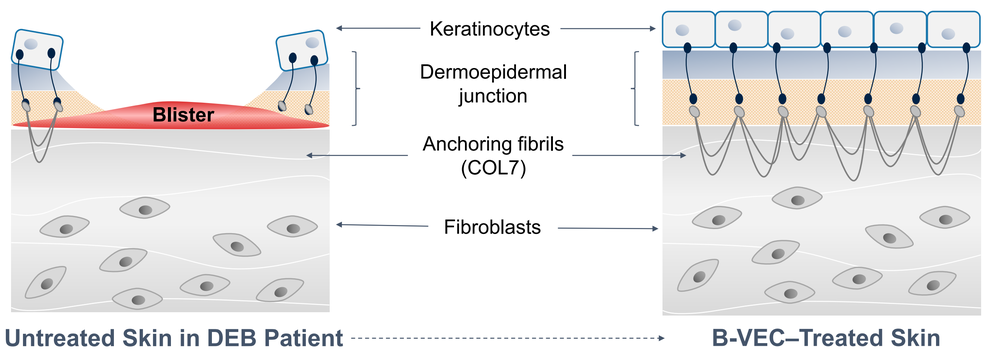
[1]Krystal Biotech, Inc., Pittsburgh, Pennsylvania

PeDRA 2025
13th Annual Meeting

B-VEC: Topical Gene Therapy for the Treatment of DEB

- Dystrophic epidermolysis bullosa (DEB) is a rare genetic condition caused by pathogenic variants in the gene encoding type VII collagen (COL7).[1]
- COL7 is critical for attaching the epidermis to the dermis; its loss leads to fragile skin, resulting in blistering, wounding, and subsequent scarring after minor trauma.
- DEB may be inherited in a dominant (DDEB) or recessive (RDEB) manner and symptoms can present as early as birth, leading to a lifelong burden in these patients.
- Beremagene geperpavec-svdt (B-VEC; tradename Vyjuvek®) is a replication defective, non-integrating herpes simplex virus type 1 (HSV-1)-based gene therapy vector designed to deliver the gene encoding COL7 directly to DEB wounds via topical application to promote durable wound healing (**Fig. 1**).[2-4]

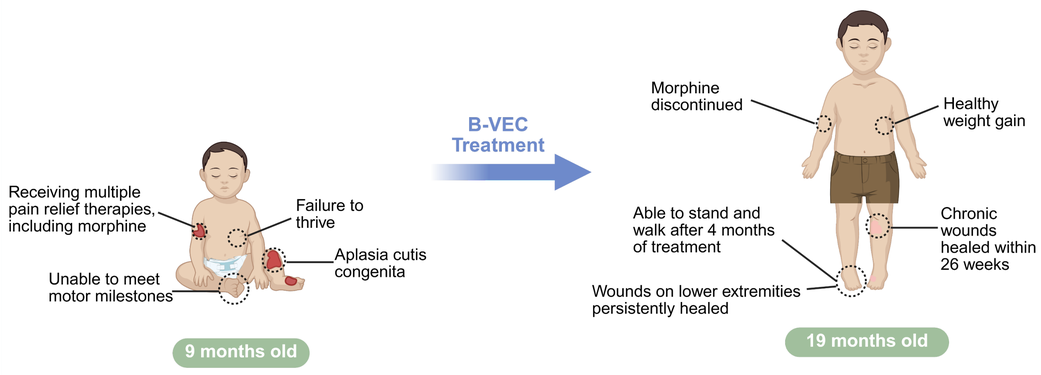
Figure 1. B-VEC Mechanism of Action



The Benefit of Early Intervention

- B-VEC was first approved in May 2023 for use in DEB patients ≥6 months old and was approved for the treatment of patients from birth in September 2025.
- A recent case report detailing treatment of a 9-month-old RDEB patient during the early access program in Germany revealed not only profound wound healing from B-VEC treatment in infants (**Fig. 2**), but also the overall benefit to general childhood development.[5]

Figure 2. 9-month-old RDEB patient before and after B-VEC Treatment



To better elucidate the safety and efficacy of B-VEC therapy in infants, here we present the data of all patients <6 months of age who enrolled for B-VEC treatment as of July 2025.

Patient Demographics

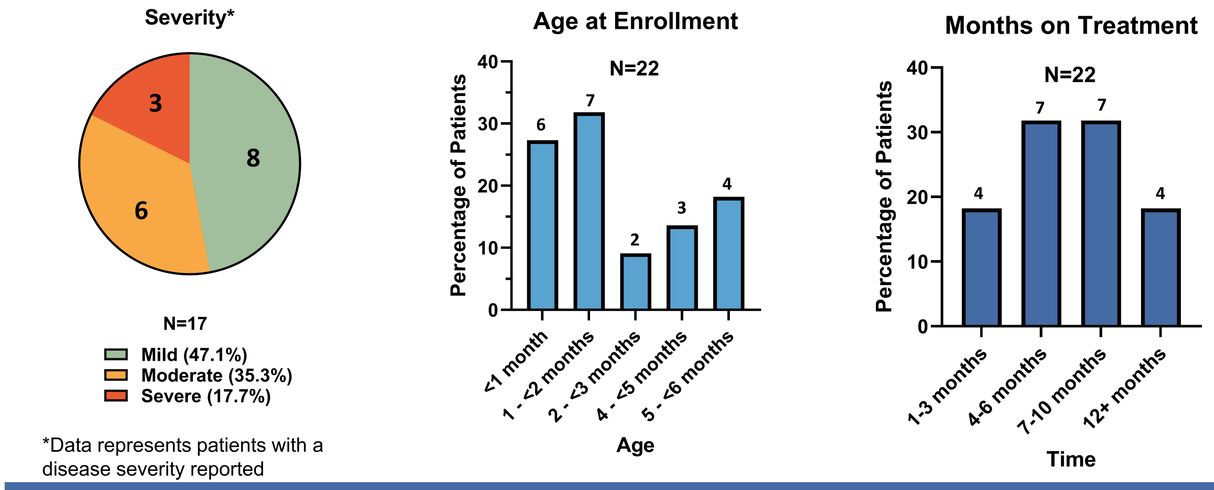
- From the period of May 2023 to July 2025, 22 patients <6 months old were enrolled for B-VEC treatment.
  - Average time between enrollment and first dose was 51.5 days.
- Of 22 patients, 2 discontinued therapy for non-drug-related reasons.

Table 1. Demographics and clinical characteristics

	Total Patients (n=22)
Age at Enrollment, days	
Mean (SD)	73.7 (60.6)
Median	48
Range	3 – 179
Sex, n (%)	
Female	14 (63.6)
Male	8 (36.4)
Genotype, n (%)	
DDEB	7 (31.8)
RDEB	15 (68.2)
Treatment Status, n (%)	
On Therapy	20 (90.9)
Stopped Therapy	2 (9.1)

Demographics and Clinical Characteristics

Figure 3. Demographics and clinical characterization of B-VEC patients <6-months-old



*Data represents patients with a disease severity reported

Safety

- There were a total of seven adverse events (AEs) reported in five patients. No AEs led to death.
- One AE, drug ineffective, was considered possibly related to B-VEC treatment.
- Two patients experienced a total of 3 serious AEs, none related to treatment: *Staphylococcal* infection (1 patient, 2 events), and poor weight gain (1 event).
- Two of the seven AEs occurred when the patient was under 6 months old (*Staphylococcal* infection and somnolence, in the same patient)

Table 2. Safety summary

Patient	Preferred Term	Age at Onset	Age at Enrollment	Serious (Yes/No)	Relationship
1	*Staphylococcal* infection	3 weeks	3 days	Yes	Not related
1	Somnolence	1 month	3 days	No	Unlikely related
1	*Staphylococcal* infection	10 months	3 days	Yes	Unlikely related
2	Weight gain poor	7 months	36 days	Yes	Unlikely related
3	*Staphylococcal* infection	1 year	52 days	No	Unlikely related
4	Drug ineffective*	11 months	121 days	No	Possibly related
5	Pain	1 year	139 days	No	Unlikely related

*Patient received 7 months of treatment with B-VEC; after data cut, formation of new wounds during growth prompted the caregiver to elect to postpone treatment until patient is older.

Example of Wound Healing in a DEB Infant

Figure 4. Wound healing in an infant with RDEB after 6 weeks of B-VEC treatment

Birth

12 weeks old (6 weeks of B-VEC treatment)

7 weeks old (after 1 B-VEC treatment)

12 weeks old (6 weeks of B-VEC treatment)

This patient presented with aplasia cutis (congenital absence of skin) of the hand and foot at birth and began B-VEC treatment at 6 weeks of age. Following 6 weeks of B-VEC treatment, the wounds on the hand and foot demonstrated marked improvement with growth of skin, as shown above. This patient did not report any AEs and is continuing weekly application of B-VEC to open wounds.

Conclusions

The positive safety profile of B-VEC in this population, paired with ongoing treatment in >90% of these young patients, highlights the potential for significant positive impact of this therapy starting from birth.

Acknowledgments and Disclosures

We would like the acknowledge the patient and their parent who provided images and medical information for use in this poster. All authors are current employees of Krystal Biotech, Inc. We would like to acknowledge Dr. Jada George (Krystal Biotech) for drafting this poster.

References

1. Has C, *et al*. Br J Dermatol. 2020;182(3):574-592.
2. Gurevich I, *et al*. Nat Med. 2022;28(4):780-788.
3. Guide SV, *et al*. N Engl J Med. 2022;387(24):2211-2219.
4. Marinkovich MP, *et al*. Am J Clin Dermatol. 2025;26(4):623-635.
5. Karakioulaki M, *et al*. J Eur Acad Dermatol. 2025; online ahead of print.