Protocol for Vyjuvek® (beremagene geperpavec-svdt)

Approved October 2023

Background: *Dystrophic epidermolysis bullosa (DEB) is one of the major types of EB, a rare hereditary group of trauma-induced blistering skin disorders. DEB is caused by inherited pathogenic variants in the COL7A1 gene, which encodes type VII collagen, the major component of anchoring fibrils which maintain adhesion between the outer epidermis and underlying dermis.*

Vyjuvek *is a herpes-simplex virus type 1 (HSV-1) vector-based gene therapy indicated for the treatment of wounds in patients 6 months of age and older with dystrophic epidermolysis bullosa with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene.*

Criteria for approval:

1. Patient is 6 months or older

2. Patient has a diagnosis of DEB with documentation of mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene. Diagnosis is confirmed by ONE of the following:
   a. Skin biopsy for immunofluorescence mapping
   b. Transmission electron microscopy
   c. Genetic testing

3. Patient has at least one open wound that is not infected

4. Patient does not have current evidence or history of squamous cell carcinoma (SCC) in the area to be treated

5. Medication is prescribed by or in consultation with a dermatologist.

6. Medication will be applied by a healthcare professional

7. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Continuation of therapy:

1. Patient had a positive clinical response to therapy (e.g., decrease in wound size, increase in granulation tissue, complete wound closure)

2. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

References:

1. Vyjuvek [prescribing information]. Krystal Biotech Inc. Pittsburgh, PA 15203 May 2023
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2020. Updated periodically
3. Hou P et al. Innovations in the Treatment of Dystrophic Epidermolysis Bullosa (DEB): Current Landscape and Prospects. Therapeutics and Clinical Risk Management 2023:19
4. Has C, Liu L, Bolling MC, et al. Clinical practice guidelines for laboratory diagnosis of epidermolysis bullosa. Br J Dermatol 2020; 182:574