BIOPHARMADIVE

DIVE BRIEF

FDA approves Krystal gene therapy for rare wound disorder

Published May 22, 2023

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Food and Drug Administration headquarters in White Oak, Maryland. *Sarah Silbiger/Getty Images via Getty Images*

Dive Brief:

- The Food and Drug Administration on Friday approved the first topical gene therapy, clearing a treatment developed by Krystal Biotech for a rare genetic skin disease that causes severe skin blistering and has no cure.
- The redosable treatment, which will be sold by Krystal as Vyjuvek, is for dystrophic epidermolysis bullosa. Its approval was supported by Phase 3 trial data published in The New England Journal of Medicine last December, several months after the company submitted its treatment to the FDA.
- Krystal said it will begin promoting its new treatment ahead of its expected availability beginning in the third quarter. The drug will have a list price of $24,250 per vial, which translates to an estimated steady state cost of $631,000 per patient per year, the company said.

Dive Insight:

Dystrophic epidermolysis bullosa is one of four main types of the disorder. Depending on the inheritance pattern, the condition can be severe and disabling, causing blisters anywhere on the skin from minor trauma and friction.

The form Vyjuvek is cleared to treat is caused by mutations in a gene responsible for encoding collagen, a necessary protein that helps strengthen and stabilize the outer layers of skin.

Vyjuvek is designed to deliver functional copies of the gene, called COL7A1, directly into the wound via a modified herpes simplex virus type 1. The functional gene is meant to produce a type of collagen, thereby promoting wound healing.

Treatment for the disease has been limited to pain and wound management. Vyjyuvek, which is approved for people 6 months of age or older, is mixed with a gel for topical application by a healthcare provider.

“Until now, doctors and nurses had no way to stop blisters and wounds from developing on dystrophic EB patient skin and all we could do was to give them bandages and helplessly watch as new blisters formed,” said Peter Marinkovich, and investigator in Krystal’s trial and director of the Blistering Disease Clinic at Stanford Health Care, in a statement provided by the company.

Other biotechnology companies have also targeted epidermolysis bullosa, or EB. One, Amicus Therapeutics, discontinued its research for a drug for dystrophic EB after negative results in a Phase 3 study five years ago. Another, Abeona Therapeutics, is working toward submitting its cell therapy for the disease to the FDA this year.

Earlier in development, a new company called Telaria is working on its own gene therapy using herpes simplex viral vectors.

Krystal's treatment is the sixth gene therapy approved in the U.S. to treat an inherited disease. The drug follows Zolgensma, Luxturna, Zynteglo, Skysona and Hemgenix onto the market.

Krystal's shares rose by more than 15% through noon Monday, trading above $110 apiece.