US FDA approves first cell-based gene therapy for rare genetic skin disorder

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Bengaluru: The U.S. Food and Drug Administration approved Abeona Therapeutics' gene therapy for a rare skin disorder on Tuesday.

Shares of the Cleveland-based drugmaker rose 13 per cent in premarket trading.

The gene therapy, called Zevaskyn, is approved to treat adult and pediatric patients with recessive dystrophic epidermolysis bullosa.

Patients with the disorder have extremely fragile skin, with blisters and tears forming large wounds all over the body that are difficult to heal and can remain open for years.

The disorder results from a defect in the COL7A1 gene, which prevents the production of a crucial type of collagen responsible for anchoring the upper and lower layers of the skin.

The current standard of care treatment is daily wound care and protective bandaging.

The treatment is expected to be available in the third quarter of 2025 at specific treatment sites, Abeona said.

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The FDA's approval was based on data from early-to-mid-stage and late-stage studies, in which the therapy showed significant healing of wounds and pain reduction after treatment.

Current treatment options include Krystal Biotech's Vyjuvek, approved in 2023, which is used to treat smaller-sized wounds. Last year, Vyjuvek brought in sales of $290.5 million.

Patients being able to use both Vyjuvek and pz-cell at the same time will help treat the disorder more effectively and "hopefully make this disease chronic but livable," said Brett Kopelan, executive director at debra of America, a nonprofit patient support organization.

Jefferies analyst Maury Raycroft estimates peak sales for the therapy, after royalty payments, to be at $427 million by 2034 and expects the treatment to be priced at $1.75 million per round of treatment.

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