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Real world case series: Filsuvez efficacy in 2 patients with Junctional Epidermolysis Bullosa

Junctional Epidermolysis Bullosa (JEB) is a rare and severe genetic skin disorder characterized by fragile skin that forms blisters in the lamina lucida with minimal trauma. Filsuvez (birch triterpenes gel), is FDA-approved for JEB and dystrophic EB (DEB), and it is the only FDA-approved option for JEB. Data on Filsuvez efficacy in JEB are limited compared to DEB because only 26/223 subjects in the pivotal trial had JEB. JEB subgroup analysis showed no significant difference in proportion of patients achieving first complete wound closure with 45 days or 90 days for Filsuvez versus placebo, showing the challenge of interpreting subgroup data with small sample sizes.

We report two female pediatric patients under 21-years-old with refractory JEB and LAMB3 gene mutations treated with Filsuvez. They showed significant improvements in wound healing, pain reduction, and overall quality of life. Notably, Filsuvez allowed for less frequent dressing changes and analgesic dosing, reducing the overall treatment burden for patients and caregivers. The application of Filsuvez was well-tolerated, with no serious adverse effects observed. One patient even used the medication during an active purulent cellulitis of a small chronic wound with no reported complications, despite FDA-label directions to discontinue Filsuvez until a wound infection resolves.

These cases demonstrate real-world efficacy and safety of Filsuvez in JEB. Further studies are warranted to confirm these findings, explore the long-term efficacy and safety of Filsuvez in managing JEB, and work with insurance providers to ensure access to this treatment which remains the only approved therapy.