Filsuvez® (birch triterpenes) – New orphan drug approval

- On December 19, 2023, Chiesi Global Rare Diseases announced the FDA approval of Filsuvez (birch triterpenes), for the treatment of wounds associated with dystrophic and junctional epidermolysis bullosa (EB) in adult and pediatric patients 6 months of age and older.

- Patients with severe forms of EB suffer from severe, chronic blistering, ulceration, and scarring of the skin, mutilating scarring of the hands and feet, joint contractures, strictures of the esophagus and mucous membranes, a high risk of developing aggressive squamous cell carcinomas, infections, and risk of premature death.

- Filsuvez contains a dry extract from two species of birch bark consisting of naturally occurring substances known as triterpenes.

- The efficacy of Filsuvez was established in EASE, a randomized, double-blind, placebo-controlled study in 223 adults and pediatric patients 6 months of age and older with EB. Patients were randomized to receive Filsuvez or placebo and instructed to apply approximately 1 mm of the investigational product to all their wounds at each dressing change (every 1 to 4 days) for 90 days. At randomization, 1 wound was selected by the investigator as the target wound for the evaluation of the primary endpoint. The primary endpoint was the proportion of patients with first complete closure of the target wound by day 45 of the 90-day double-blind phase of the study.

    - The proportion of patients with first complete closure of target wound within 45 days was 41.3% and 28.9% with Filsuvez and placebo, respectively (95% CI for the treatment difference: 0.8, 25.6).
    - The proportion of patients with first complete closure of target wound within 90 days was 50.5% and 43.9% with Filsuvez and placebo, respectively (95% CI for the treatment difference: -6.2, 20.0).

- A warning and precaution for Filsuvez is hypersensitivity reactions.

- The most common adverse reaction (≥ 2%) with Filsuvez use was application site reactions.

- Filsuvez is applied as a 1 mm layer to the affected wound surface only. The wound should be covered with a sterile non-adhesive wound dressing. Alternatively, Filsuvez can be applied directly to the dressing so that the topical gel is in direct contact with the wound.

    - Filsuvez should be applied to cleansed wounds with wound dressing changes until the wound is healed.
    - If a Filsuvez-treated wound becomes infected, treatment should be discontinued to that wound until the infection has resolved.

- Chiesi's launch plans for Filsuvez are pending. Filsuvez topical gel will be available as 10% birch triterpenes w/w supplied in 25 mL sterile tubes.