Zevaskyn™ (prademagene zamikeracel) – New orphan drug approval

- On April 29, 2025, Abeona Therapeutics announced the FDA approval of Zevaskyn (prademagene zamikeracel), for the **treatment of wounds in adult and pediatric patients with recessive dystrophic epidermolysis bullosa (RDEB)**.

- RDEB is a rare dermatologic condition with mutations in both copies of the *COL7A1* gene that expresses Type VII collagen. People with RDEB have extremely fragile skin characterized by extensive blistering and severe wounds that often cover more than 30% of a patient's body surface, and in some cases up to 80%.

- Zevaskyn consists of a patient's own skin cells that have been genetically modified, to produce functional Type VII collagen. Zevaskyn sheets are surgically applied to the patient's wounded areas.

- The efficacy of Zevaskyn was established in a randomized, intrapatient-controlled study (VIITAL). The study compared the application of Zevaskyn to the standard of care treatment in patients with wounds associated with RDEB. A total of 86 wounds in 11 patients were enrolled and treated with Zevaskyn or standard of care. The co-primary efficacy outcome measures were 1) proportion of randomized wound pairs with at least 50% healing at month 6 with confirmation of wound healing two weeks later, and 2) pain reduction as assessed by the mean differences in patient-reported pain scores using the Wong-Baker FACES scale between randomized wound pairs at month 6.

    - The proportion of randomized wound pairs healed ≥ 50% from baseline was 81% and 16% with Zevaskyn and control, respectively ($p < 0.0001$).
    - The mean pain reduction from baseline was -3.07 and -0.90 with Zevaskyn and control, respectively ($p = 0.0002$).

- Warnings and precautions for Zevaskyn include hypersensitivity reaction, retroviral vector-mediated insertional oncogenesis, and transmission of infectious agents.

- The most common adverse reactions (≥ 5%) with Zevaskyn use were procedural pain and pruritus.

- The recommended dose of Zevaskyn is based on the surface area of the wound(s). One sheet of Zevaskyn covers an area of 41.25 cm^2. Up to twelve Zevaskyn sheets may be manufactured from the patient biopsies and supplied for potential use.

    - Zevaskyn is to be prepared by the manufacturer in an appropriate healthcare setting for surgical application by a qualified healthcare provider.

- **The list price for Zevaskyn is $3.1 million**.

- Abeona Therapeutics plans to launch Zevaskyn in the third quarter of 2025