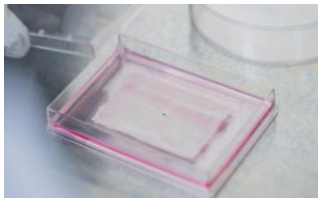
RDEB is a genetic disorder with devastating cutaneous complications leading to morbidity & early mortality

- ✓ RDEB results from insufficient keratinocyte production of functional type 7 collagen (COL7) anchoring fibrils
- ✓ RDEB leads to widespread blistering leading to life-threatening complications such as infections and squamous cell carcinoma
- ✓ Patients have chronic and recurrent wounds, of which, the majority are large (>20cm2)
- ✓ Current costs for supportive care can exceed $150,000 (US) per year

EB-101 is a novel, gene-corrected cell therapy for RDEB



- ✓ EB-101 is an autologous (derived from patients' tissues) cell therapy which corrects the COL7 genetic defect directly in a patient's keratinocytes
- ✓ Keratinocytes and keratinocyte stem cells are isolated from two 8mm punch biopsies and developed into multiple gene corrected sheets which are transplanted back on the patient
- ✓ EB-101 sheets can treat large RDEB wounds from 20cm2 to 240cm2 in a single administration of six gene-corrected sheets

NOW ENROLLING: The pivotal Phase 3 VIITAL™ study

VIITAL™ PHASE 3 STUDY

In January 2020, investigators from Stanford University Medical Center opened enrollment of the VIITAL™ study. The multi-center, randomized study will assess EB-101 in up to 15 patients age 6 or older, with approximately 30 chronic wound sites treated in total. More information about the study can be found at Abeona Therapeutics.com/ClinicalTrials and at Clinical Trials.gov by searching 'NCT 04227106.'

EB-101 is uniquely positioned to treat large & painful RDEB wounds, which are debilitating & afflict a majority of patients

JCI insight

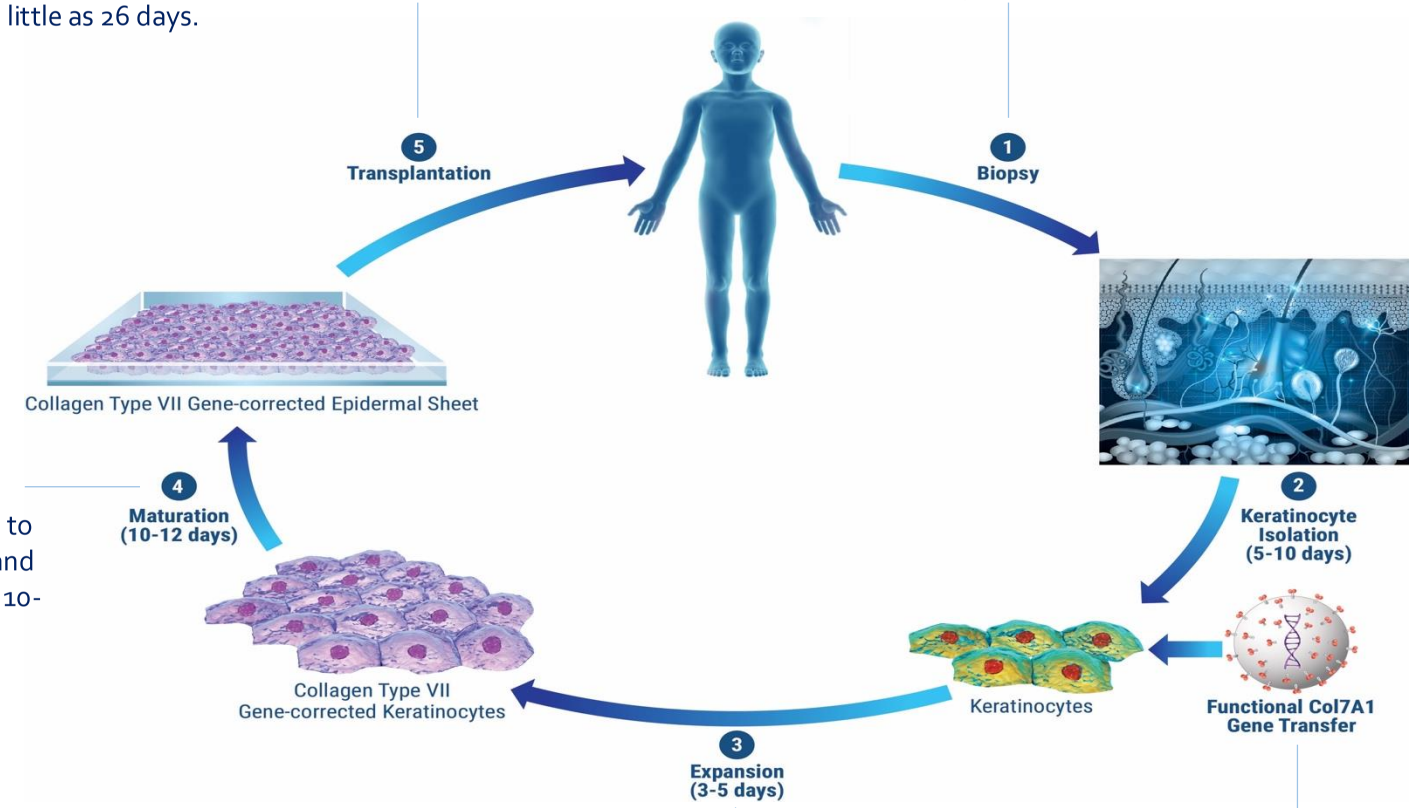
EB-101 was evaluated in a Phase 1/2a trial and treated 42 wounds in 7 RDEB patients. Long-term data from the study published in JCI Insight suggest EB-101 may provide durable healing in even the largest, most challenging RDEB wounds, including some that had remained open for up to 20 years.

- In Phase 1/2a, EB-101-treated wounds durably healed: with 80% of wounds achieving 50% healing and 70% achieving greater than or equal to 75% healing. These healing rates corresponded to an unparalleled area of healed skin ranging from 130-150+ cm^2 for most study participants.
- Patients realized reduced pain and itching in EB-101 treated wounds: >50% healing was associated with improvement in patient-reported pain, itch, and wound healing durability.
- EB-101 was associated with long-term expression of collagen 7: indicating durability for our gene-corrected cell therapy.
- No treatment-related adverse events reported to date: no replication competent virus was detected at any time point.

EB-101: Gene Therapy for RDEB

Two 8mm skin biopsies are taken from a patient and shipped to Abeona. These biopsies can be produced into 6-8 sheets of EB-101 that are ~40 cm^2.

After being shipped to the treatment center, EB-101 is transplanted onto patients' wounds in as little as 26 days.



Abeona scientists expand and prepare the keratinocyte cells in the biopsies for the next step in the process.

The cells continue to mature and grow for 10-12 days.

Functional type 7 collagen is transduced into the keratinocyte cells. As the cells mature, they express gene-corrected type 7 collagen. This is why EB-101 is a gene-corrected cell therapy.

After transduction, keratinocyte cells are expanded and harvested as 5.5x7.5cm sheets.