## 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<sup>™</sup> study

In January 2020, investigators from Stanford University Medical Center

opened enrollment of the VIITAL<sup>™</sup> 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

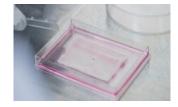
EB-101 was evaluated in a Phase 1/2a trial and treated 42 wounds in 7 RDEB patients. Long-term data from the study published in JCI Insight suggest EB-101 may provide durable healing in even the largest, most challenging RDEB wounds, including some that had remained open for up to 20 years.

- 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<sup>2</sup> 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.

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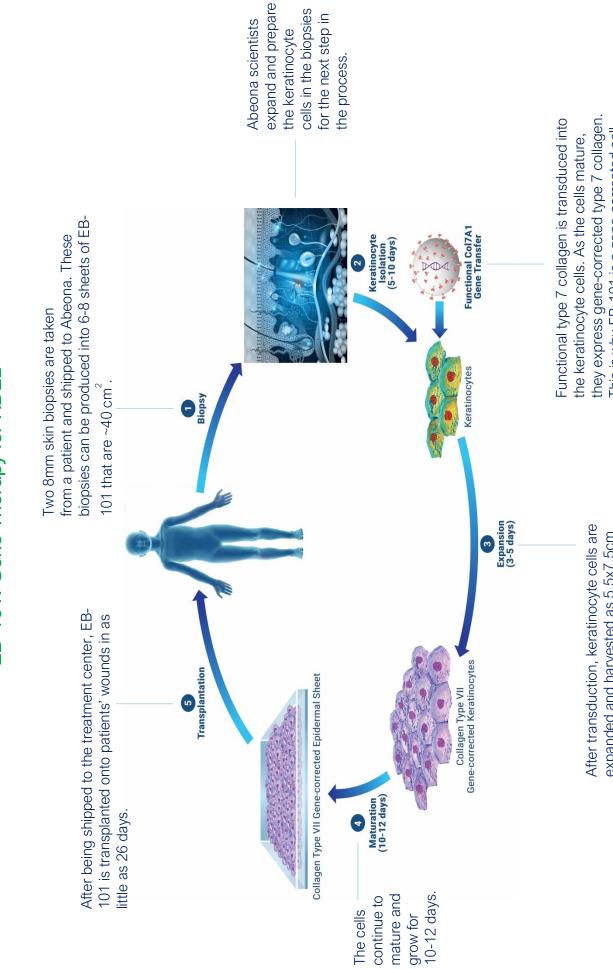




PHASE 3 STUDY

insight





EB-101: Gene Therapy for RDEB

expanded and harvested as 5.5x7.5cm sheets.

This is why EB-101 is a gene-corrected cell therapy.