Confidential

CC-CHOC Pediatric Point Person Project (P4)

Dystrophic Epidermolysis Bullosa (DEB) Information

If you have patients with Dystrophic Epidermolysis Bullosa, please follow this link to report the number and type of DEB: https://redcap.ctsacentral.org/surveys/?s=ntcpA3

Submitted 06-19-2012

Participant ID 15 DEB

Sponsor Industry: Lotus Tissue Repair

Target Population Patients and families affected by Dystrophic Epidermolysis Bullosa (DEB)

Information Lotus Tissue Repair is developing recombinant collagen VII (rC7) as a protein replacement therapy for dystrophic epidermolysis bullosa (DEB), a devastating skin blistering disease. DEB patients are deficient in collagen VII or produce a dysfunctional form. Collagen VII is the major component of anchoring fibrils that anchor the epidermis to the dermis.
Patients with the recessive form of DEB have severe morbidity with pseudosyndactyly (mitten deformity), esophageal strictures, extensive skin blistering and scarring, growth retardation, and early mortality from metastatic squamous cell carcinoma and infections.
Newborn children are usually blistered or have missing skin at birth and spend 30 or more days in the NICU. They are then usually referred to dermatologists.
Lotus Tissue Repair has promising results with protein replacement therapy in preclinical animal models of DEB and they will be starting clinical trials with rC7 protein replacement therapy in 2013.

Lotus and DEBRA of America (the DEB patient organization) recently launched a patient registry to better understand the prevalence and burden of illness of DEB in the USA and other countries.

Goals

- Increase awareness of Dystrophic Epidermolysis Bullosa
- Provide patients and families access to the resources of DEBRA (The Dystrophic Epidermolysis Bullosa Research Association of America); this includes DEBRA's *"New Family Advocate Program"* with an on-call nurse to help families with newly diagnosed EB patients.
- Increase participation in EBCare.org ; the patient-reported registry for Epidermolysis Bullosa
- Gather information on the number and location of patients with Dystrophic Epidermolysis Bullosa

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Any questions? Email ctsa_childhealth@ctsaC4.org