



## **Constant Pharmaceuticals to Initiate Clinical Development of TXA127 for Epidermolysis Bullosa (EB)**

### **Funding to be Provided by Consortium of EB Charities**

Boston, MA (November 27, 2018). Constant Pharmaceuticals today announced that it plans to begin a development program for its compound, TXA127, for the treatment of the rare pediatric disease, epidermolysis bullosa in collaboration with four EB focused charities - DEBRA (Austria), Cure-EB, the Epidermolysis Bullosa Research Partnership (EBRP), and the Epidermolysis Bullosa Medical Research Foundation (EBMRF). The plan is for this program to first complete the development of an oral formulation of the TXA127 peptide and then conduct a Phase 2 clinical trial in Europe and the US with that formulation.

In work done at the University of Freiburg under the direction of Professor Bruckner-Tuderman, TXA127 showed significant clinical benefit in the animal model corresponding to the most severe form of EB, Recessive Dystrophic Epidermolysis Bullosa (RDEB), and showed positive effects in related in-vitro models. TXA127 demonstrated substantial anti-fibrotic effects and showed a reduction in the fusion of digits, a symptom in RDEB mice that is analogous to mitten deformity common in patients with severe RDEB.

“DEBRA Austria is delighted to be able to provide further support towards the clinical development of TXA127. This will build on the high-quality preclinical research in Freiburg that we have funded to date, that indicates the promise of TXA127 as a candidate to tackle the disabling effects of fibrosis. As a father of an EB patient, it is great to see advances in the development of drugs with possible major beneficial impact on the life of people who live with RDEB,” said Dr. Rainer Riedl, CEO of DEBRA Austria.

“We are very pleased to have had the support of DEBRA (Austria) in sponsoring the preclinical investigations of TXA127 for the treatment of RDEB, and thankful to each of the charities for their interest in this important program going forward. There are currently no approved therapies for EB, and TXA127 may represent the first systemic therapy capable of targeting the multi-organ symptoms of this severe disease.” said Dr. Richard Franklin, CEO of Constant Pharmaceuticals.

In the first part of the new program, Constant will complete the development of an oral formulation of TXA127. TXA127 is currently delivered as a daily subcutaneous injection, but this is not suitable for patients with EB.

“EB Research Partnership’s venture philanthropy model is focused on working together with companies like Constant Pharmaceuticals to accelerate life-changing treatments for people with EB. We look forward to partnering with Constant on the development of TXA127 and are proud to share with them a commitment to innovation and urgency to deliver results to patients”, said Michael Hund, Executive Director of EBRP.

The second component of the program will be a Phase 2 trial done in Europe and the US. That trial will examine a number of endpoints that are clinically important in EB, including systemic and cutaneous fibrosis.

"Cure EB is pleased to assist in this collaboration that will accelerate development of TXA127 for RDEB patients. The development of ‘mitten deformities’ is an extremely severe consequence of RDEB and we thank Constant Pharmaceuticals for the great opportunity to move towards a therapy for RDEB alongside its programs for other conditions" said Sharmila Collins Founder and Trustee, CureEB.

#### About EB and RDEB

Epidermolysis bullosa is a group of genetic conditions, often involving a mutation in the collagen gene, COL7A1, that cause the skin to be very fragile and to blister easily. Blisters and skin erosions form in response to minor injury or friction, such as rubbing or scratching. Dystrophic epidermolysis bullosa (DEB) is one of the major forms of epidermolysis bullosa. The signs and symptoms of this condition vary widely among affected individuals. In mild cases, blistering may primarily affect the hands, feet, knees, and elbows. Severe cases of this condition, including RDEB, involve widespread blistering that can lead to vision loss, disfigurement, and other serious medical problems.

#### About TXA127

TXA127 is a pharmaceutical grade formulation of the naturally occurring peptide Angiotensin (1-7), which Constant is developing for the treatment of a number of orphan and genetic diseases, as well as stroke recovery. TXA127 has Orphan Drug Designation and Pediatric Rare Disease Designation and is expected to enter Phase II in EB in 2020, following formal GMP development of an oral formulation, which is required for systemic therapy in EB. TXA127 has received orphan drug designation for the treatment of EB in Europe and the US.

#### About Constant

Constant Pharmaceuticals LLC (formerly Tarix Orphan LLC) is a private biopharmaceutical company focused on the treatment of stroke recovery and rare neuromuscular disorders and connective tissue diseases. The company’s lead drug candidate is TXA127, a pharmaceutical formulation of the naturally occurring peptide Angiotensin (1-7). TX127 has shown therapeutic activity in animal models of RDEB, Duchenne muscular dystrophy (DMD), Limb-girdle muscular dystrophy (LGMD), congenital muscular dystrophy (MDC1A) and stroke recovery, where the drug is effective with treatment started weeks after the stroke. Constant has broad IP protection for TXA127, and Orphan Drug Designations (ODDs) have been granted for DMD,

LGMD, and RDEB in the United States, and for DMD and EB in Europe. For more information on Constant Pharmaceuticals, please visit our website at [www.constantpharma.com](http://www.constantpharma.com).

#### About DEBRA Austria

DEBRA Austria is a member of DEBRA International, the worldwide alliance of national DEBRA Epidermolysis Bullosa (EB) patient support groups, working in over 50 countries and growing. Our aims are to support people with EB and their organizations to work together to maximize quality of life, and to promote the development of effective treatments for the condition as quickly as possible through research. <http://www.debra-international.org>.

#### About Cure-EB

Cure EB (formerly Sohana Research Fund) exists to fund medical research to find effective treatments and eventually a cure for epidermolysis bullosa, an inherited skin condition that leads to blistering and skin loss. Our focus is on translating research to develop a combination of treatments to combat the multi-system disease condition and leading to a cure. It is a UK based charity founded by the parents of a young sufferer [www.cure-eb.org](http://www.cure-eb.org).

#### About EBRP

EB Research Partnership is the largest nonprofit dedicated to funding research aimed at treating and ultimately curing Epidermolysis Bullosa, a group of devastating and life-threatening skin disorders that affect children from birth. EBRP works to treat and cure EB as quickly and efficiently as possible, fulfilling our mission through partnerships with non-profit and for-profit organizations, foundations, individual donors, and the EB and research communities. [www.ebresearch.org](http://www.ebresearch.org). EB Research Partnership has raised \$25 million to fund innovative research aimed at finding treatments and cures for EB. EB is a genetic disease, so many of the

#### About EBMRF

The Epidermolysis Bullosa Medical Research Foundation was established in 1991 by Gary & Lynn Fechser Anderson at the request of Dr. Eugene Bauer, then Professor and Chairman of the Department of Dermatology at The Stanford University School of Medicine. The EBMRF is unique in that the Foundation pays no salaries. All work, including executive, development and administrative, is done on a volunteer basis. The Foundation prides itself on its efficiency, keeping operating costs at less than 1% of incoming donations so that a full 99% of contributions can go directly to its research programs. [www.ebmrf.org](http://www.ebmrf.org).

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