



NEWS RELEASE

bridgebio pharma's phoenix tissue repair provides updates to its recessive dystrophic epidermolysis bullosa (rdeb) program and announces new leadership appointments

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BOSTON, May 13, 2020 (GLOBE NEWSWIRE) -- Phoenix Tissue Repair, Inc. (PTR), an affiliate company of BridgeBio Pharma, Inc. (Nasdaq: BBIO) today announced updates to its ongoing Phase 1/2 study of PTR-01 (BridgeBio designation BBP-589), an intravenously-administered recombinant collagen 7 protein replacement therapy for patients with recessive dystrophic epidermolysis bullosa (RDEB). Cohorts 1 – 3 have completed treatment at escalating dose levels, and an additional fourth cohort to evaluate higher dosing of PTR-01 has begun enrolling patients.

Based on an interim review of data from the first three cohorts of trial participants, PTR-01 has been well tolerated in all nine patients, and there have been no treatment-related serious adverse events at any dose. In addition, as assessed by our investigators, there has been a dose-dependent increase in collagen 7 skin deposition after just three infusions over 28 days.

RDEB is a rare genetic disorder characterized by severe blistering and scarring of the skin caused by even minor friction or trauma, as well as systemic manifestations including esophageal strictures and dysphagia, corneal abrasions, anemia and nutritional deficiencies.

"RDEB is a debilitating disease, and current treatment options are limited to palliative skin care involving daily wound maintenance, protective bandaging, pain and itch management, and treatment of secondary complications

such as anemia,” said Anna L. Bruckner, M.D., MSCS, associate professor of dermatology and pediatrics at University of Colorado School of Medicine. “Intravenous collagen 7 replacement therapy with PTR-01 is the only treatment in development designed to provide broad disease-modifying effects for this multisystem disease. In addition to benefiting the skin, this treatment has the potential to improve wounds that affect the eye or internal mucosa surfaces such as the esophagus.”

The ongoing clinical trial is a randomized, saline-controlled, double-blind, repeat dose, dose-escalation, multi-center study. Patients enrolled in the fourth cohort will each be dosed over a 10-week period, with three 3.0 mg/kg doses of PTR-01 and three doses of saline control. The primary objective of the trial is to evaluate the safety and tolerability of PTR-01 in adults with RDEB. Additional endpoints include assessment of biologic activity through skin biopsy evaluation of collagen 7 deposition and clinical assessments including wound healing, pain, itch and quality of life.

After completion of this trial, the company is planning to follow it up with a six-month open-label clinical trial to inform the design of a pivotal clinical trial. To learn more about the PTR-01 Phase 1/2 clinical trial, please visit www.clinicaltrials.gov and search the identifier [NCT03752905](https://clinicaltrials.gov/ct2/show/study/NCT03752905).

The company also announced that Sanuj K. Ravindran, M.D. has been appointed to lead PTR as executive chairman, and Hal Landy, M.D., and Deborah Ramsdell have joined PTR as chief medical officer and chief operating officer, respectively. Dr. Ravindran is currently CEO of PellePharm Inc., another BridgeBio company focused on rare genetic skin diseases. Both Dr. Landy and Ms. Ramsdell were involved in PTR-01’s early development at Lotus Tissue Repair, which was acquired by Shire prior to PTR securing rights to the PTR-01 program.

“With safety as a top priority, we are committed to creating the first-ever systemic treatment for RDEB, which targets the genetic root of the disease by replacing the collagen protein that normally helps keep the epidermis from separating from the dermis,” said Dr. Ravindran. “As the program advances, we look forward to working closely with the epidermolysis bullosa patient community to help raise awareness of this debilitating disease and ensure that we develop our therapy to best address the unmet needs that patients currently face.”

About Dystrophic Epidermolysis Bullosa (DEB)

DEB is a rare genetic disorder symptomatic from birth that is caused by mutations in the gene for a protein called collagen type VII (C7). The C7 protein is essential for the formation of anchoring fibrils, structures which connect the epidermis and dermis—the uppermost two layers of the skin. Patients with the recessive form of DEB (RDEB) tend to have particularly severe symptoms due to severe insufficiency of functional C7. Symptoms include extreme skin and mucosal fragility that present as recurrent, painful blistering and scarring of the skin, as well as ulcerations of the mouth, tongue and dental caries. In addition to the cutaneous and oral symptoms, severe forms are associated with erosions and scarring of mucous membranes of the eye, esophagus, genitals and anus. Joint contractures,

mutilitating deformities of hands and feet, malnutrition, growth retardation, recurrent infections and a significantly increased risk for squamous cell carcinoma are also common. There are currently no approved disease-modifying therapies for any form of DEB, and the standard of care focuses on wound and pain management.

About Phoenix Tissue Repair and PTR-01

Phoenix Tissue Repair is a Boston-based company that is an affiliate of BridgeBio Pharma, and is focused on advancing a novel systemic treatment for recessive dystrophic epidermolysis bullosa (RDEB).

PTR-01 is an investigational protein replacement therapy which uses a recombinant collagen type VII (rC7) for the treatment of RDEB. PTR-01 is designed to be systemically available through intravenous delivery. Phoenix Tissue Repair acquired worldwide rights to PTR-01 in 2017. Preclinical studies of PTR-01 have demonstrated C7 staining in basement membranes with de novo anchoring fibril formation and improved survival in models of RDEB.

PTR-01 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration and the European Medicines Agency.

About BridgeBio Pharma, Inc.

BridgeBio is a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. BridgeBio was founded in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio's pipeline of over 20 development programs includes product candidates ranging from early discovery to late-stage development. For more information, visit bridgebio.com.

Forward-Looking Statements

This press release contains forward-looking statements. All statements contained herein other than statements of historical fact constitute forward-looking statements, including statements relating to expectations, plans, and prospects regarding Phoenix Tissue Repair's clinical development plan, clinical trial results, timing and completion of clinical trials, and ability to take advantage of expedited FDA review for PTR-01. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to, Phoenix Tissue Repair's ability to advance PTR-01 in clinical development in accordance with its plans, the results from any clinical trials and nonclinical studies of PTR-01, and the nature of Phoenix Tissue Repair's interactions with regulatory authorities. Moreover, Phoenix Tissue Repair operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. These forward-looking statements are based upon the current expectations and beliefs of Phoenix Tissue Repair's management as of the date of this release and are subject to certain risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. All forward-looking statements in this press release are based on information

available to Phoenix Tissue Repair as of the date hereof, and Phoenix Tissue Repair disclaims any obligation to update these forward-looking statements except as required by law.

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