



NEWS RELEASE

bridgebio pharma affiliate phoenix tissue repair announces positive results from phase 2 trial of ptr-01, a protein replacement therapy for the treatment of recessive dystrophic epidermolysis bullosa (rdeb)

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- PTR-01 was well-tolerated over a four-month treatment period in RDEB patients
- Treatment with PTR-01 led to rapid, consistent, and durable wound healing as observed in reduction of wound surface area and clinician-reported assessments
- All patients that completed the study reported a decrease in pain over the course of treatment with PTR-01

PALO ALTO, Calif., May 20, 2022 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) and affiliate company Phoenix Tissue Repair, which is focused on advancing a novel systemic treatment for recessive dystrophic epidermolysis bullosa (RDEB), announced data from the Phase 2 trial of PTR-01, an intravenously-administered recombinant collagen 7 (rC7) protein replacement therapy, in patients with recessive dystrophic epidermolysis bullosa (RDEB). The data are being shared in a poster at the Society for Investigative Dermatology (SID) Annual Meeting 2022 between May 18 – 21, 2022 in Portland, Oregon.

RDEB is a rare genetic disorder caused by mutations in the gene encoding collagen type VII (C7) and is one of the most severe forms of epidermolysis bullosa, characterized by severe and painful skin blistering, as well as extreme fragility and scarring of mucous membranes throughout the body. There is currently no known cure or effective treatment available for patients suffering from this disease.



“In patients with recessive dystrophic epidermolysis bullosa (RDEB) even minor friction or trauma can cause debilitating blistering, tearing and scarring of the skin, along with severe pain and itching. Our data shows that treatment with PTR-01 led to rapid, consistent, and durable wound healing,” said Sanuj K. Ravindran, M.D., executive chairman of Phoenix Tissue Repair. “We are hopeful that by addressing the root cause of this rare disease, we will be able to provide a treatment beyond daily wound care and pain management for patients in need.”

The Phase 2, open-label study was designed to examine the effect of PTR-01 on wound healing as well as other endpoints, and to evaluate the long-term safety and tolerability of the drug candidate. The data shared at the 2022 SID Annual Meeting demonstrated that PTR-01 was well tolerated when given once per week for 4 weeks and then every other week for 14 weeks.

In addition, treatment with PTR-01 led to rapid, consistent, and durable wound healing as observed in reduction of wound surface area and clinician-reported assessments. Specifically, over 80% of target wounds (21/26) demonstrated a 50% or greater reduction in wound surface area at the end of treatment (day 120) compared to baseline. Notably, this response was observed in a breadth of wound types: recurrent (86%) and chronic (75%); and a range of wound sizes: large (89%) and small (77%), as determined by at least 50% reduction in wound surface area compared to baseline. Clinician assessment of the same target lesions scored on a 7-pt scale compared to baseline demonstrated similar levels of efficacy and wound improvement. By day 15 after initiating treatment, 15 of 26 wounds (58%) met the response criteria of ≥ 2 -point increase on the wound-specific scale, and at day 120, 18/26 wounds (69%) met the response criteria. Based on these criteria, four out of six patients who enrolled (67%) were responders since they had ≥ 2 -point increase on this scale in $\geq 50\%$ of their wounds at day 120.

Patient-reported outcomes measuring pain, essential function, mood, activities of daily living and disease impact also showed marked mean and median reductions comparing end of study to baseline. Notably, all patients that completed the study (N=5) reported a decrease in pain over the course of treatment with PTR-01. There was a 36% mean reduction in total pain from end of study compared to baseline as measured on the Instrument for Scoring Clinical Outcomes of Research for Epidermolysis Bullosa (iscorEB) patient reported subdomain scale.

Finally, systemic administration of PTR-01 resulted in rapid deposition of rC7 at the DEJ during the loading phase (first 28 days of treatment) and remained present up to 3 months after treatment.

Phoenix Tissue Repair has initiated a Phase 2 extension study.

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, mutilating 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 an affiliate company of BridgeBio and 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 (FDA) and the European Medicines Agency.

About BridgeBio Pharma, Inc.

BridgeBio Pharma, Inc. (BridgeBio) is a commercial-stage biopharmaceutical company founded to discover, create, test and deliver transformative medicines to treat patients who suffer from genetic diseases and cancers with clear genetic drivers. BridgeBio's pipeline of development programs ranges from early science to advanced clinical trials. BridgeBio was founded in 2015 and its team of experienced drug discoverers, developers and innovators are committed to applying advances in genetic medicine to help patients as quickly as possible. For more information visit [bridgebio.com](https://www.bridgebio.com) and follow us on [LinkedIn](#) and [Twitter](#).

BridgeBio Pharma Forward-Looking Statements

This press release contains 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 the ability of BridgeBio and Phoenix Tissue Repair to take advantage of expedited FDA review for PTR-01. Statements in this press release that are not statements of historical fact are considered forward-looking statements within the meaning of Section 27A of the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), which are usually identified by the use of words such as

“anticipates,” “believes,” “continues,” “could,” “estimates,” “expects,” “intends,” “may,” “plans,” “potential,” “predicts,” “projects,” “seeks,” “should,” “will,” and variations of such words or similar expressions. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements are neither forecasts, promises nor guarantees, and are based on the current beliefs of BridgeBio’s management and Phoenix Tissue Repair’s management as well as assumptions made by and information currently available to BridgeBio and Phoenix Tissue Repair. Such statements reflect the current views of BridgeBio and Phoenix Tissue Repair with respect to future events and are subject to known and unknown 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, the results from prior clinical trials and nonclinical studies of PTR-01 not being indicative of the results from future clinical trials and nonclinical studies of PTR-01, and the nature of BridgeBio and Phoenix Tissue Repair’s interactions with regulatory authorities. Moreover, BridgeBio and Phoenix Tissue Repair operate in a very competitive and rapidly changing environment in which new risks emerge from time to time. Although BridgeBio and Phoenix Tissue Repair believe that BridgeBio’s and Phoenix Tissue Repair’s plans, intentions, expectations, strategies and prospects as reflected in or suggested by these forward-looking statements are reasonable, neither BridgeBio nor Phoenix Tissue Repair can give any assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a number of risks, uncertainties and assumptions, including, without limitation, those risks and uncertainties described under the heading “Risk Factors” in BridgeBio’s most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K filed with the SEC and in subsequent filings made by BridgeBio with the SEC, which are available on the SEC’s website at www.sec.gov. Moreover, BridgeBio and Phoenix Tissue Repair operate in very competitive and rapidly changing environments in which new risks emerge from time to time. These forward-looking statements are based upon the current expectations and beliefs of BridgeBio’s management and Phoenix Tissue Repair’s management as of the date of this press 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. We anticipate that subsequent events and developments will cause our views to change. Except as required by law, each of BridgeBio and Phoenix Tissue Repair disclaims any intention or responsibility for updating or revising any forward-looking statements contained in this press release in the event of new information, future developments or otherwise. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this press release.

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