NEWS RELEASE

phoenix tissue repair doses first patient in phase 1/2 clinical trial of ptr-01 (bbp-589) for treatment of recessive dystrophic epidermolysis bullosa (rdeb)

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BOSTON, Feb. 22, 2019 /PRNewswire/ — Phoenix Tissue Repair, Inc., a biotechnology company focused on developing transformational disease-modifying treatments for dystrophic epidermolysis bullosa (DEB), today announced that the first patient has been dosed in the Phase 1/2, first in-human trial of PTR-01 (BridgeBio Pharma designation BBP-589), a protein replacement therapy for recessive DEB (RDEB). DEB and RDEB are rare genetic multisystem disorders associated with severe skin blistering, and treatment is currently limited to palliative symptom management.

“Dosing the first patient in the clinic is an important milestone for the development of PTR-01 as a potential treatment for RDEB,” said Dr. Neil Kirby, President and Chief Executive Officer of Phoenix Tissue Repair. “The community of RDEB patients, families and physicians urgently need effective treatments for this devastating disease. With an approach that aims to target the root cause of the disease, we are optimistic that PTR-01 could provide hope to patients and their families.”

DEB is a rare genetic disorder symptomatic from birth that is caused by mutations in the gene encoding collagen type VII protein (C7). Symptoms include extreme skin and mucosal fragility and can also include erosions and scarring of other mucous membranes throughout the body. Severe comorbidities are common, and patients require intensive and constant care. DEB, and RDEB in particular, is associated with a considerable reduction in quality-of-life and life span. There are currently no approved disease-modifying therapies for any form of DEB.

The PTR-01-001 Phase 1/2 trial will enroll 14 patients who will each be dosed over a 10-week period. The primary objective of the trial is to evaluate the safety, tolerability and pharmacokinetics of PTR-01 in RDEB patients. The trial will also assess various secondary endpoints. To learn more about the PTR-01 Phase 1/2 clinical trial, please visit **www.clinicaltrials.gov** and search the identifier **NCT03752905**.

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). C7 is essential for the formation of anchoring fibrils, structures which connect the uppermost two layers of the skin, the epidermis and dermis. Patients with RDEB tend to have particularly severe symptoms due to severe insufficiency of functional C7. Symptoms include extreme skin and mucosal fragility that presents as recurrent, painful blistering and scarring of the skin; ulcerations of the mouth and 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 PTR-01

Phoenix Tissue Repair is advancing an investigational therapy known as PTR-01, a protein replacement therapy which uses a recombinant collagen type VII (rC7) for the potential 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 distributes to the basement membrane of the skin, and was observed to restore anchoring fibrils, promote healing and improve survival.

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

About the Phase 1/2 Clinical Trial

The PTR-01-001 trial is a saline-controlled, single-blind, multiple ascending dose, dose-escalation, multi-center study. Patients will be enrolled into one of three cohorts. Cohorts 1, 2 and 3 will consist of two, four and eight patients, respectively. During the Treatment Period a total of three doses of PTR-01 and three doses of saline control will be administered to all patients for a total of six doses in a cross-over design over a 10-week period. The primary objective of the trial is to evaluate the safety, tolerability and pharmacokinetics of PTR-01 in RDEB patients. Additionally, the trial will assess the proof of biologic activity through skin biopsy evaluation of C7 and the

presence of anchoring fibrils. Wound healing and clinically meaningful patient reported outcomes will also be evaluated. To learn more about the PTR-01 Phase 1/2 clinical trial, please visit **www.clinicaltrials.gov** and search the identifier **NCT03752905**.

About Phoenix Tissue Repair and BridgeBio

Phoenix Tissue Repair is a Boston-based company that is part of the BridgeBio Pharma LLC ("BridgeBio") family. BridgeBio is a clinical-stage biopharmaceutical company working to create life-altering medicines that target well-characterized genetic diseases at their source. The BridgeBio approach combines a traditional focus on drug development with a corporate model that is designed to allow rapid translation of early stage science into medicines that treat disease at its source. Founded in 2015 by a team of industry veterans, BridgeBio has built a pipeline of 15 development programs, each housed in its own subsidiary, ranging from pre-clinical to late stage development in multiple therapeutic areas including dermatology, cardiology, oncology and endocrinology. BridgeBio's focus on scientific excellence and rapid execution aims to translate today's discoveries into tomorrow's medicines.

Investor Contact:
LeYi Wang, PhD
Vice President, Business Development
(650) 391-9740
info@phoenixtissuerepair.com

Media Relations Contact:
Carolyn Hawley
Canale Communications
(619) 849-5382
carolyn@canalecomm.com

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