Proteostasis Therapeutics Receives FDA Fast Track Designation for PTI-428 in Patients with Cystic Fibrosis

February 01, 2016

Cambridge, Mass., February 1, 2016 — Proteostasis Therapeutics, Inc., a company developing novel therapeutics that regulate protein homeostasis to improve outcomes for patients with orphan and protein processing diseases, today announced that it has received Fast Track designation from the U.S. Food and Drug Administration (FDA) for PTI-428, an investigational oral treatment for cystic fibrosis (CF). The FDA's Fast Track programs are designed to facilitate the development and expedite the review of new drugs that are intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs. A drug that receives Fast Track program designation is eligible for more frequent communications between the FDA and the company relating to the development plan and clinical trial design, and may be eligible for priority review if certain criteria are met.

PTI-428 is a novel and orally bioavailable Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) modulator belonging to the amplifier class. Amplifiers are CFTR modulators that selectively increase the amount of an immature form of CFTR protein, thereby providing additional substrate for other CFTR modulators, such as correctors or potentiators, to act upon.

"We are very pleased to receive this Fast Track designation from the FDA for PTI-428 for the treatment of CF" stated Meenu Chhabra, President and Chief Executive Officer of Proteostasis Therapeutics. "PTI-428 is a unique CFTR amplifier that when used in combination with existing treatments and therapies presently in clinical development, has shown a consistent positive effect on CFTR protein activity in vitro, nearly doubling activity in patient derived human bronchial epithelial cells not only for the most common gene mutation found in CF, but across multiple gene mutations. We have also demonstrated that a novel combination of PTI-428, together with a proprietary corrector and a proprietary potentiator, can restore in vitro CFTR

protein activity to approximately 97% of normal in patient-derived HBE cells homozygous for F508del."

CF is a progressive disease caused by mutations in the gene that encodes CFTR resulting in disrupted ion flow which leads to the buildup of thick mucus in several organs including the lungs, and the vast majority of patients die of respiratory failure.

The Company recently received authorization from the FDA to start first-in-human studies in CF patients which will be initiated in the first quarter of 2016. The goal of the Phase 1 clinical trial is to study safety, pharmacokinetics and preliminary pharmacodynamics and the final report is expected by the end of 2016.

About Proteostasis Therapeutics

Proteostasis Therapeutics, Inc. (PTI) is developing disease-modifying therapeutics for diseases of protein processing. Using the DRT™ platform, a phenotypic screening approach based on the use of functionally pertinent cellular assays and disease relevant models, PTI identifies highly selective drug candidates that modulate the proteostasis imbalance in the cell. In addition to its multiple wholly-owned programs in cystic fibrosis, PTI has formed collaborations with Biogen New Ventures Inc. to research and identify therapeutic candidates for neurodegenerative disease and with Astellas Pharma Inc. to research and identify therapies targeting the Unfolded Protein Response (UPR) pathway. For more information visit www.proteostasis.com. http://www.proteostasis.com/

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