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Proteostasis Therapeutics, Inc. Presents Preclinical Data for Its CFTR Amplifier Program at the 29th Annual North American Cystic Fibrosis Conference

CAMBRIDGE, MA--(Marketwired - Oct 8, 2015) - Proteostasis Therapeutics, Inc. (PTI), a company developing novel therapeutics to treat diseases caused by defects in protein processing, today announced the presentation of data on the Company's CFTR amplifier program and its potential for use in combination therapies for cystic fibrosis (CF) at the 29th Annual North American Cystic Fibrosis Conference in Phoenix, Arizona.

In the Company's oral presentation titled "CFTR Amplifiers Are a New Class of CFTR Modulators", PTI reported an observed increase in cystic fibrosis transmembrane conductance regulator (CFTR) modulating activity in human bronchial epithelial (hBE) cells, when its compounds were used as stand-alone treatment, and evidence that these compounds can further enhance CFTR mediated current when used in combination with existing clinical-stage correctors.

Because mutations in the CFTR gene cause CF, CFTR amplifiers can represent a new class of CFTR modulating agents with potential therapeutic use in the treatment of this severe and incurable condition. CFTR amplifiers enhance the effect of existing CFTR modulators, such as potentiators and correctors. The CFTR amplifiers have demonstrated potential to be effective across CFTR mutation classes in preclinical studies and such results form the basis for PTI's strategy to develop a broad-acting combination therapy able to serve CF patients.

"We are very pleased with the clinical potential of the CFTR amplifier compounds that double the activity of the most effective combination of clinical-stage correctors in the gold-standard HBE cell assay, not only for the most common mutation, F508del/F508del, but in other mutations found in the cystic fibrosis population as well," said Meenu Chhabra, President and Chief Executive Officer of PTI. "We are confident that we will continue to build on our promising preclinical results to advance our products toward clinical trials."

The Company is developing PTI-428 as a new CFTR amplifier for the treatment of CF. PTI-428 has demonstrated pharmacologic properties amenable to oral dosing. A twenty-eight day, non-GLP preclinical toxicology study testing multiple dose groups of PTI-428 in non-human primates demonstrated a favorable safety and tolerability profile for clinical development. PTI will advance PTI-428 as its lead clinical development candidate for the treatment of CF and expects to file an IND with the FDA by the end of 2015.

Additionally, PTI has initiated a new preclinical program in chronic obstructive pulmonary disease (COPD) with another one of its CFTR amplifiers, PTI-130. COPD is characterized by shortness of breath, coughing and increased mucus formation, which can be a significant contributor to morbidity. PTI-130

may represent a novel treatment approach due to its ability to increase CFTR-mediated ion transport in non-CF hBE cells. Targeting CFTR function may potentially improve hydration and restore mucus formation to normal physiological levels in the airway.

PTI's CFTR amplifiers are wholly-owned by PTI and were internally discovered through the company's proprietary Disease-Relevant Translation, or DRT™ platform. PTI-428 and PTI-130 are results of medicinal chemistry optimization of internally discovered active compounds. In tests using HBE cell Ussing functional assays, both drug candidates increased CFTR function and nearly doubled the efficacy of CFTR modulating agents such as correctors and potentiators. These data provide a basis for the clinical exploration of the use of PTI-428 as an add-on therapy to the emerging standard of care (corrector/potentiator combination) to deliver greater benefit to CF patients, and the use of PTI-130 to enhance non-mutant CFTR function in COPD.

About Cystic Fibrosis
Cystic fibrosis is a genetic disorder affecting approximately 70,000 to 100,000 people worldwide. Improvement in disease management protocols and approval of new drugs to treat the symptoms have extended the life expectancy for CF patients. However, CF remains an incurable disease that leads to death.

About Chronic Obstructive Pulmonary Disease
Chronic obstructive pulmonary disease, or COPD, is a type of chronic progressive lung disease characterized by poor airflow. COPD is a leading cause of death worldwide. The main symptoms include shortness of breath, cough and sputum production. There is no known cure for COPD. The current standard of care is therapeutic intervention to alleviate symptoms and delay progression of the disease.

About Proteostasis Therapeutics
Proteostasis Therapeutics, Inc. (PTI) is developing disease-modifying therapeutics for diseases of protein processing. By combining the DRT™ platform, a phenotypic screening approach based on the use of functionally pertinent cellular assays, with state of the art medicinal chemistry tools, PTI generates highly selective drug candidates that modulate the proteostasis imbalance in the cell. In addition to its multiple wholly-owned programs in CF and COPD, PTI has formed collaborations with Biogen 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.

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