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Proteostasis Therapeutics receives FDA approval for Cystic Fibrosis drug

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The FDA Office of Orphan Products Development grants Orphan Drug Designation to novel drugs or biologics that are intended for the treatment of rare diseases or conditions affecting fewer than 200,000 patients in the United States. The designation allows the sponsor of the drug to be eligible for various incentives, including a seven-year period of U.S. marketing exclusivity upon regulatory approval of the drug, as well as tax credits for clinical research costs, annual grant funding, clinical trial design assistance, and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

Proteostasis Therapeutics, Inc. , a clinical stage biopharmaceutical company dedicated to the discovery and development of groundbreaking therapies to treat cystic fibrosis (CF) and other diseases caused by dysfunctional protein processing, has announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for PTI-428, the Company's cystic fibrosis transmembrane conductance regulator (CFTR) amplifier drug candidate.

"This is the second important regulatory designation PTI-428 has been granted from the FDA this week, highlighting our amplifier's potential to provide clinical benefit in the treatment of CF," said Meenu Chhabra, president and chief executive officer of Proteostasis Therapeutics."

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About PTI-428

PTI-428 is an investigational CFTR amplifier in development for the treatment of CF in patients who are homozygous for the F508del mutation in the CFTR gene as an add-on therapy to approved CFTR modulators or as part of PTI's proprietary triple combination regimen that includes PTI-808, a potentiator, and PTI-801, a corrector. PTI-428 has been shown to work early during CFTR biogenesis to increase levels of newly synthesized CFTR protein, suggesting potential therapeutic benefits in combination with CFTR correctors and potentiators. In addition to Orphan Drug Designation, PTI-428 has been granted Breakthrough Therapy Designation as well as Fast Track Designation from the FDA.

In December, Proteostasis announced the results from a Phase 2, randomized, placebo controlled study of PTI-428, in 24 CF subjects on background Orkambi therapy which showed that treatment with PTI-428 led to mean absolute improvement in percent predicted forced expiratory volume in 1 second (ppFEV1) of 5.2 percentage points from baseline through Day 28 compared to placebo ($p<0.05$).

Proteostasis plans to initiate a triple combination CF Study of PTI-428 with PTI-801, its third generation corrector, and PTI-808, its potentiator, in the second quarter of 2018, with preliminary results anticipated in the second half of 2018. The Company is also planning an additional study of PTI-428 in CF Subjects on Symdeko™, with initial data anticipated in early 2019.