Catabasis Pharmaceuticals Research on CAT-5571, a Novel Activator of Autophagy and Potential Oral Treatment for Cystic Fibrosis, Published in Journal of Medicinal Chemistry

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-- Preclinical Data Demonstrate that Fatty Acid Cysteamine Conjugates Are Autophagy Activators that Enhance the Correction of Misfolded F508del-CFTR --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 5, 2017-- Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced the publication of preclinical data on CAT-5571, a novel activator of autophagy and potential oral treatment for cystic fibrosis (CF). The preclinical data demonstrate an increase in CF transmembrane conductance regulator (CFTR) activity and trafficking which are detailed in an article titled “Fatty Acid Cysteamine Conjugates as Novel and Potent Autophagy Activators that Enhance the Correction of Misfolded F508del-CFTR” in the Journal of Medicinal Chemistry (J Med Chem. 2016 Dec 23. doi: 10.1021/acs.jmedchem.6b01539).

The publication describes the synthesis and biology of the CAT-5000 series of molecules developed using the proprietary Catabasis SMART linker drug discovery platform. The data demonstrate that CAT-5571 is a novel autophagy activator which, in combination with the current standard of care therapy, increased the cell surface expression and function of CFTR in bronchial epithelial cells isolated from multiple CF patients with the F508del mutation. When CAT-5571 was used in combination with lumacaftor and ivacaftor, it significantly enhanced the effects on the cells of the standard of care combination, both in the amount of the more mature C-band form of the CFTR protein with complex glycosylation, and in the amount of the CFTR protein reaching the cell surface. Importantly, CAT-5571 significantly enhanced the lumacaftor/ivacaftor-mediated chloride current increase in cultured primary homozygous F508del human bronchial epithelial cells.

“We are excited about these preclinical results and the potential of autophagy activation by CAT-5571 as a treatment for CF that is able to improve CFTR trafficking and function. We look forward to progressing CAT-5571 in preclinical development and toward clinical development,” said Andrew Nichols, Ph.D., Chief Scientific Officer of Catabasis. “This program further builds our rare disease pipeline and shows the capability of the proprietary Catabasis SMART linker drug discovery platform.”

Catabasis expects to initiate a Phase 1 clinical trial with CAT-5571 for the potential treatment of CF in Q4 2017 or Q1 2018. CAT-5571 is a novel molecule comprising cysteamine covalently conjugated to docosahexaenoic acid (DHA) using the company’s SMART linker drug discovery platform to enhance the intracellular activity of the bioactive components. CAT-5571 allows sustained intracellular delivery of the two bioactive components leading to activation of autophagy through two different pathways. Autophagy is a process that maintains cellular homeostasis and host defense mechanisms, and is known to be impaired in CF. We have found that the level of autophagy activation achieved with CAT-5571 cannot be replicated by administering the bioactive components either individually or in combination, even at much higher concentrations.

About CAT-5571
Catabasis is developing CAT-5571 as a potential oral treatment for cystic fibrosis (CF) with potential effects on both the cystic fibrosis transmembrane conductance regulator (CFTR) and on the clearance of *Pseudomonas aeruginosa*. CAT-5571 is a small molecule that activates autophagy, a process that maintains cellular homeostasis and host defense mechanisms, and is known to be impaired in CF. Catabasis has shown in preclinical studies that CAT-5571, in combination with lumacaftor/ivacaftor, enhances cell-surface trafficking and function of CFTR with the F508del mutation. Catabasis has also shown that CAT-5571 enhances the clearance of *P. aeruginosa* infection in preclinical models of CF, regardless of CFTR mutation status.

About Cystic Fibrosis
Cystic fibrosis (CF) is a rare, chronic, genetic, life-shortening disease that affects over 70,000 patients worldwide, predominantly in the Caucasian population. In CF, a malfunctioning cystic fibrosis transmembrane conductance regulator (CFTR) ion channel impairs chloride secretion, with deleterious effects on multiple organs, and particularly devastating effects on pulmonary, intestinal and pancreatic function. Patients affected with CF are also predisposed to respiratory failure caused by persistent lung infections that are difficult to treat with standard antibiotics. Advancements in research and treatments have extended the life expectancy for those living with CF, however there is currently no cure.

About Catabasis
At Catabasis Pharmaceuticals, our mission is to bring hope and life-changing therapies to patients and their families. Our SMART (Safely Metabolized And Rationally Targeted) linker drug discovery platform enables us to engineer molecules that simultaneously modulate multiple targets in a disease. We are applying our SMART linker platform to build an internal pipeline of product candidates for rare diseases and plan to pursue partnerships to develop additional product candidates. For more information on the Company's drug discovery platform and pipeline of drug candidates, please visit www.catabasis.com.

Forward Looking Statements
Any statements in this press release about future expectations, plans and prospects for the Company, including statements about future clinical trial plans and other statements containing the words “believes,” “anticipates,” “plans,” “expects,” “may” and similar expressions, constitute forward-looking

statements within the meaning of the Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development of the Company's product candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products; availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; other matters that could affect the availability or commercial potential of the Company's product candidates; and general economic and market conditions and other factors discussed in the "Risk Factors" section of the Company's Quarterly Report on Form 10-Q for the period ended September 30, 2016, which is on file with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. In addition, the forward-looking statements included in this press release represent the Company's views as of the date of this press release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this release.

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