



## **Catabasis Pharmaceuticals Presents New Data for CAT-5571 as a Novel Potential Oral Treatment for Cystic Fibrosis at the 40<sup>th</sup> European Cystic Fibrosis Society Conference**

*-- CAT-5571 Restores Autophagy, a Host Defense Mechanism, Which Is Known to be Impaired in Cystic Fibrosis --*

*-- Data Demonstrate Improved Intracellular Clearance of Bacteria of Importance in Patients with Cystic Fibrosis --*

**CAMBRIDGE, MA, June 8, 2017** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, announces positive preclinical data on CAT-5571, supporting its potential as an oral treatment for cystic fibrosis (CF). These data are being presented today at the 40<sup>th</sup> European Cystic Fibrosis Society (ECFS) Conference in Seville, Spain.

Patients with CF suffer from persistent lung infections with opportunistic pathogens such as *Pseudomonas aeruginosa* and *Burkholderia cenocepacia* causing chronic infections that are difficult to eradicate from lung tissue. Approximately 50% of CF patients are known to suffer from chronic infection of *P. aeruginosa*. *B. cenocepacia* is often resistant to all available antibiotics and can be fatal for CF patients. These intracellular pathogens are typically restricted via autophagy in people not affected by CF. CAT-5571 activates autophagy, a host defense mechanism, and the data presented demonstrate that CAT-5571 improves bacterial clearance of the chronic intracellular pathogens *P. aeruginosa* and *B. cenocepacia*.

“We are excited about the potential of CAT-5571 to address impaired autophagy, a host defense mechanism, in CF patients, strengthening their ability to clear persistent serious lung infections. These data demonstrating CAT-5571’s ability to significantly reduce the intracellular bacterial load of *P. aeruginosa* and *B. cenocepacia* suggest that CAT-5571 could play an important role in improving clinical outcomes in combination with current CF therapies,” said Andrew Nichols, Ph.D., Chief Scientific Officer of Catabasis. “We look forward to progressing CAT-5571 in preclinical development. This program further builds our rare disease pipeline which also includes our lead program edasalonexent for the potential treatment of Duchenne muscular dystrophy and CAT-4001 for the potential treatment of neurodegenerative diseases.”

The data presented demonstrate that CAT-5571 activates autophagy, a host defense mechanism, in a cystic fibrosis animal model. In *cftr F508del/F508del* mice, treatment with CAT-5571 restored the depressed autophagy markers Beclin-1 and LC3B, which are critical components of the host defense system. The data presented also demonstrate an impact on the intracellular clearance of two different types of bacteria. *In vitro* studies using normal or homozygous F508del human bronchial epithelial cells infected with *P. aeruginosa* showed that CAT-5571 treatment caused a

significant reduction in the intracellular bacterial load. Similarly, in macrophages isolated from *cftr F508del/F508del* mice, *in vitro* treatment with CAT-5571 reduced the intracellular bacterial load of *B. cenocepacia*.

CAT-5571 is a novel molecule comprising cysteamine covalently conjugated to docosahexaenoic acid (DHA) that was engineered 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. Catabasis is conducting IND-enabling activities for CAT-5571 and expects to initiate a Phase 1 clinical trial in 2018.

### **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.

### **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](http://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 March 31, 2017, 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.

###

### Investor and Media Contact

Andrea Matthews

Catabasis Pharmaceuticals, Inc.

T: (617) 349-1971

[amatthews@catabasis.com](mailto:amatthews@catabasis.com)