



Catabasis Pharmaceuticals Provides Edasalonexent and Rare Disease Pipeline Updates at Investor Day

-- Upcoming Phase 2 Results for Edasalonexent, a Potential Disease-Modifying Treatment for Duchenne Muscular Dystrophy (DMD) --

-- Rare Disease Pipeline Now Includes CAT-5571, a Potential Treatment for Cystic Fibrosis --

CAMBRIDGE, MA, November 17, 2016 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today is holding its first Investor Day and will provide an in-depth review of the Company's strategy and pipeline in rare diseases, including edasalonexent (CAT-1004) and other programs. Guest speakers will include Craig McDonald, M.D., UC Davis NeuroNEXT Program Director, University of California, and H. Lee Sweeney, Ph.D., Myology Institute Director, University of Florida.

"At Catabasis, we have been executing relentlessly on our strategic plan and mission to bring hope and life-changing therapies to patients and their families suffering from rare diseases," said Jill C. Milne, Chief Executive Officer of Catabasis. "We expect to report top-line safety and efficacy results from the Phase 2 portion of the MoveDMD® clinical trial in the first half of Q1 2017 after the JP Morgan conference. Assuming positive Phase 2 results, we anticipate initiating two additional clinical trials in DMD next year, including a pivotal Phase 3 trial. In addition to our work in DMD, we are also progressing the Catabasis rare disease pipeline. We look forward to initiating clinical work in an additional rare disease for edasalonexent where NF-kB plays an important role. Further, Catabasis recently expanded its rare disease pipeline by adding CAT-5571, an activator of autophagy, as a potential treatment of cystic fibrosis."

Program Updates and Highlights:

Edasalonexent (CAT-1004) for the Potential Treatment of DMD

- Top-line safety and efficacy results from the placebo-controlled portion of the MoveDMD Phase 2 trial are expected in the first half of Q1 2017.
- MoveDMD Phase 2 trial design: The primary efficacy end point is change in magnetic resonance imaging (MRI) T2 for the composite of five lower leg muscles for the pooled edasalonexent dose groups compared to placebo. Safety and tolerability will also be evaluated. Additional assessments are being measured, however the trial is not powered for statistical significance for these assessments. The additional assessments include timed function tests (10-meter walk/run, 4-stair climb and time to stand), muscle strength measures, the North Star Ambulatory Assessment (NSAA) and the pediatric outcomes data collection instrument (PODCI).

- The open-label extension for the MoveDMD trial, in which patients will continue on open-label edasalonexent for 36 weeks following completion of the 12-week, placebo-controlled portion of the trial, is ongoing. During the open-label extension, safety will be monitored and assessments including MRI, timed function tests, muscle strength measures, the NSAA and the PODCI, will be performed. Catabasis expects to report periodic results from the MoveDMD open-label extension in 2017.
- Assuming positive results from the Phase 2 MoveDMD clinical trial and supportive discussions with regulatory authorities, Catabasis intends to initiate a Phase 3 placebo-controlled pivotal clinical trial of edasalonexent in ambulatory boys with DMD aged 4 to 7 in H2 2017. The primary end point is expected to be one of the age-appropriate timed function tests included in the Phase 2 trial. The final design of the Phase 3 trial is expected to be informed by the results of the Phase 2 MoveDMD trial as well as the open-label extension data available prior to the initiation of the Phase 3 trial.
- Assuming positive results from the Phase 2 MoveDMD clinical trial, Catabasis also intends to initiate a clinical trial in non-ambulatory patients with DMD in H2 2017.
- There are additional diseases in which inhibiting NF- κ B with edasalonexent may be beneficial. Catabasis expects to initiate a Phase 2 trial for an additional rare disease indication for edasalonexent in Q4 2017 or Q1 2018.

Additional Rare Disease Programs

- Preclinical research with CAT-4001 has continued in diseases such as amyotrophic lateral sclerosis (ALS) and Friedreich's ataxia. Ongoing preclinical research for CAT-4001 is expected in 2017.
- CAT-5571 is a potential therapy to treat cystic fibrosis (CF). CAT-5571, an activator of autophagy, in combination with lumacaftor/ivacaftor, enhanced cell-surface trafficking and function of cystic fibrosis transmembrane conductance regulator (CFTR) in bronchial epithelial cells from CF patients with the F508del mutation. Catabasis also demonstrated that CAT-5571 enhanced the clearance of *Pseudomonas aeruginosa* infection in preclinical models of CF, irrespective of CFTR mutation status. Catabasis expects to initiate a Phase 1 trial with CAT-5571 for the potential treatment of CF in Q4 2017 or Q1 2018.

A live webcast of the presentations is available via the investor section of the Catabasis website, www.catabasis.com. Following the live webcast, an archived version will be available for 90 days.

About Edasalonexent (CAT-1004)

Edasalonexent (CAT-1004) is an oral small molecule that has the potential to be a disease-modifying therapy for all patients affected by Duchenne muscular dystrophy (DMD or Duchenne), regardless of their underlying mutation. Edasalonexent inhibits NF- κ B, a protein that is activated in Duchenne and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. In animal models of DMD, edasalonexent produced beneficial effects in skeletal, diaphragm and cardiac muscle and improved function. The FDA has granted orphan drug, fast track and rare pediatric disease designations and the European Commission has granted orphan

medicinal product designation to edasalonexent for the treatment of DMD. We have previously reported safety, tolerability and reduction in NF-κB activity in Phase 1 trials in adults. We are currently conducting the MoveDMD® trial of edasalonexent in 4-7 year-old boys affected by Duchenne. From Part A of the MoveDMD trial, we have reported that edasalonexent was generally well tolerated with no safety signals observed and we observed NF-κB target engagement. Pharmacokinetic results demonstrated edasalonexent plasma exposure levels consistent with those previously observed in adults, at which inhibition of NF-κB was observed.

About CAT-4001

Catabasis is developing CAT-4001 as a potential treatment for neurodegenerative diseases such as Friedreich's ataxia (FA) and amyotrophic lateral sclerosis (ALS). CAT-4001 is a small molecule that activates Nrf2 and inhibits NF-κB, two pathways that have been implicated in FA and ALS. Catabasis has shown that CAT-4001 modulates the Nrf2 and NF-κB pathways in both cellular assays and animal models.

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, irrespective of CFTR mutation status.

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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