Catabasis Pharmaceuticals Announces Favorable Results for Functional Assessments in the MoveDMD® Trial for Edasalonexent in Duchenne Muscular Dystrophy at the American Academy of Neurology 69th Annual Meeting

April 25, 2017

-- Prespecified Analysis of Part B Data Shows Improvement in Rates of Change Across Five Functional Assessments --

-- Part C Interim Results to be Announced in Q3 2017 --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 25, 2017-- Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced additional favorable results across multiple functional assessments in the MoveDMD trial at the American Academy of Neurology 69th Annual Meeting. In Part B of the MoveDMD trial, designed to evaluate the potential of edasalonexent in the treatment of Duchenne muscular dystrophy (DMD), numerical improvements were seen in prespecified rate change analyses across five functional assessments. These results are in addition to and consistent with the numerical improvements in the same functional assessments with edasalonexent compared to placebo after 12 weeks of edasalonexent treatment.

“Following our completed analysis of the rate of change data from Part B of the MoveDMD trial, we are encouraged by the consistency of the possible treatment effects across the range of functional assessments after only 12 weeks of dosing as well as the numerical improvements in functional assessments compared to placebo. These functional assessments are meaningful to boys affected by Duchenne and are known to correlate with loss of milestones and disease progression,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “Coupled with the reassuring safety, tolerability and plasma exposure data in patients affected by Duchenne, we are optimistic about edasalonexent’s potential and look forward to continuing to evaluate it as a novel treatment for this devastating disease.”

In the MoveDMD trial, functional assessments were performed at baseline of Part A, at baseline of Part B, which was on average 8 months later, and at the endpoint of Part B, which was following 12 weeks of treatment. This design enabled the comparison of changes in functional ability between an extended off-treatment period and 12 weeks of treatment with edasalonexent. The MoveDMD trial was not powered for functional assessments and these analyses were generally not statistically significant.

- During the off-treatment period there were declines in average speeds of timed function tests (10-meter walk/run, 4-stair climb and time to stand) as well as the North Star Ambulatory Assessment (NSAA) and Pediatric Outcomes Data Collection Instrument (PODCI) assessment
- During edasalonexent treatment, positive numerical changes were observed across the five functional assessments compared with the off-treatment period:
    - Rate of decline slowed by 50% for 10-meter walk/run
    - Rate of decline slowed by 45% for time to stand
    - Rate of decline slowed by more than 50% for 4-stair climb with no decline in function
    - Rate of decline in score slowed by more than 50% for NSAA with positive improvement seen
    - Rate of decline in score slowed by more than 50% for PODCI (p=0.01) with positive improvement seen

The edasalonexent 100 mg/kg/day treatment group also showed numerical improvement versus placebo across these five functional assessments in Part B of the trial. Functional assessments included in this trial have precedence as endpoints for pivotal trials in DMD.

Catabasis’ MoveDMD trial is a three-part clinical trial investigating the safety and efficacy of edasalonexent in boys ages 4 – 7 affected with DMD (any confirmed mutation). The planned prespecified analyses from Part B of the MoveDMD trial included a cross-over comparison to evaluate the rate of change for the functional assessments while the patients were largely off-treatment (Part A baseline to Part B baseline) to the rate of change while on edasalonexent treatment for 12 weeks in Part B. This comparison included the twelve boys that participated in Part A and then crossed over to edasalonexent treatment in Part B. In January 2017, Catabasis reported that the primary efficacy endpoint of MRI T2 was not met and numerical improvements were observed for the functional assessments with the placebo-controlled comparisons in Part B of the trial.

From Part A of the MoveDMD trial, the Company reported in January 2016 that edasalonexent was generally well tolerated with no safety signals observed and NF-kB target engagement was observed. Consistent with Part A, there were no safety signals and edasalonexent was well tolerated in Part B of the trial. There were no treatment-related serious adverse events, no drug discontinuations and no dose reductions.

Catabasis intends to report results from Part C, the open-label extension part of the MoveDMD trial, in 2017. To allow for all the boys participating in Part C to complete 24 weeks of dosing with edasalonexent, an interim update on Part C results is now planned for Q3 2017. Following additional data analysis on functional assessments from Part C, the Company will determine the next steps for edasalonexent in DMD.

About Edasalonexent (CAT-1004)
Edasalonexent (CAT-1004) is an investigational oral small molecule that is being developed as a potential disease-modifying therapy for all patients affected by DMD, regardless of their underlying mutation. Edasalonexent inhibits NF-kB, a protein that is activated in DMD and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. We are currently conducting the MoveDMD trial, a three-part clinical trial investigating the safety and efficacy of edasalonexent in boys ages 4 – 7 affected with DMD (any confirmed mutation). The third part of the trial, an open-label extension with edasalonexent, is ongoing. 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. For a summary of clinical results reported to-date, please visit www.catabasis.com.

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 Annual Report on Form 10-K for the year ended December 31, 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.

View source version on businesswire.com: http://www.businesswire.com/news/home/20170425005573/en/

Source: Catabasis Pharmaceuticals, Inc.

Investor and Media Contact:
Catabasis Pharmaceuticals, Inc.
Andrea Matthews, 617-349-1971
amatthews@catabasis.com