



Catabasis Pharmaceuticals and Duchenne UK Announce Partnership to Evaluate Edasalonexent in a Phase 2 Non-Ambulatory Duchenne Muscular Dystrophy Trial

-- Duchenne UK Grants Over \$600,000 in Funding to Support Patient and Clinical Trial Site Costs --

BOSTON, Mass., January 8, 2020 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, and Duchenne UK, a charity that seeks to fund and accelerate treatments and a cure for Duchenne muscular dystrophy (DMD), have entered into a partnership for a Phase 2 trial of edasalonexent, a novel NF- κ B inhibitor, in non-ambulatory DMD patients. This exploratory Phase 2 trial, which is subject to the receipt of adequate funding, is designed to assess safety, pharmacokinetics and exploratory measures of function including cardiac, skeletal muscle and pulmonary function in non-ambulatory DMD patients.

“We are thrilled to announce plans to expand our knowledge of edasalonexent to non-ambulatory boys and men affected by Duchenne. We recognize the urgent need for a well-tolerated treatment like edasalonexent with the potential to slow disease progression and preserve muscle function by benefitting both skeletal muscle as well as cardiac function,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “We are incredibly fortunate to have the opportunity to partner with Duchenne UK for this important work and appreciate their deep commitment as we work together to bring treatment options to all patients.”

“We first approached Catabasis last year to ask if we could encourage them to advance a trial to look at the non-ambulant patient population and we are delighted to be able to announce this collaboration today,” said Emily Crossley and Alex Johnson, Duchenne UK cofounders. “Duchenne UK is committed to developing medicines for all boys and men with DMD, regardless of their physical stage, mutation or age. This trial will represent an important step in that direction.”

The Phase 2 trial is designed to be a one-year, randomized, double-blind, placebo-controlled trial evaluating safety, pharmacokinetics and exploratory measures of function with edasalonexent in non-ambulatory boys and men affected by DMD. The trial expects to enroll approximately 16 non-ambulatory patients ages 10 and older regardless of mutation type who have not been on steroids for at least 6 months at clinical trial sites in the United Kingdom. The exploratory functional endpoints are anticipated to include assessments of cardiac function, upper limb skeletal muscle function and pulmonary function. In addition, the trial is also expected to explore patient reported outcomes. The intention is that upon completing this trial, patients will have the option to transition to the GalaxyDMD open-label extension trial and receive edasalonexent.

About Edasalonexent (CAT-1004)

Edasalonexent (CAT-1004) is an investigational oral small molecule designed to inhibit NF-kB that is being developed as a potential foundational therapy for all patients affected by DMD, regardless of their underlying mutation. In DMD the loss of dystrophin leads to chronic activation of NF-kB, which is a key driver of skeletal and cardiac muscle disease progression. Our ongoing global Phase 3 PolarisDMD trial is evaluating the efficacy and safety of edasalonexent for registration purposes. Edasalonexent is also being dosed in the GalaxyDMD open-label extension trial. In our MoveDMD Phase 2 trial and open-label extension, we observed that edasalonexent preserved muscle function and substantially slowed disease progression compared to rates of change in a control period, and significantly improved biomarkers of muscle health and inflammation. 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, 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 lead program is edasalonexent, an NF-kB inhibitor in Phase 3 development for the treatment of Duchenne muscular dystrophy. For more information on edasalonexent and our Phase 3 PolarisDMD trial, 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 including, among other things, statements about the potential commencement of the Company's planned Phase 2 trial in non-ambulatory patients, the Company's global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes and the GalaxyDMD open-label extension trial, including the anticipated timing for top-line results, the potential timing for the filing of an NDA, the Company's cash expectations, the Company's planned transition to a commercial-stage organization 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; 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-Q for the year ended September 30, 2019, 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