



Catabasis Pharmaceuticals Announces Publication of Phase 1 Clinical Results of Edasalonexent (CAT-1004) in Duchenne Muscular Dystrophy

-- MoveDMD Trial Data Showed Edasalonexent Was Well-Tolerated with No Safety Signals and Confirmed NF-kB Target Engagement --

CAMBRIDGE, Mass., November 27, 2018 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced that data from the Phase 1 MoveDMD clinical trial of edasalonexent were [published](#) in the Journal of Neuromuscular Diseases, “Phase 1 Study of Edasalonexent (CAT-1004), an Oral NF-kB Inhibitor, in Pediatric Patients with Duchenne Muscular Dystrophy”. Edasalonexent was well tolerated in pediatric patients and the data demonstrated that edasalonexent inhibited NF-kB in pediatric patients with Duchenne muscular dystrophy (DMD). NF-kB is a key link between loss of dystrophin and disease progression in DMD. MoveDMD is a multi-part trial including Phase 1, Phase 2 and an on-going open-label extension. Catabasis is currently enrolling the Phase 3 PolarisDMD trial for edasalonexent.

“The data from the Phase 1 MoveDMD trial reinforce the good tolerability and safety profile of edasalonexent that we have now also observed in the Phase 2 trial and open-label extension,” said Erika Finanger, M.D., Associate Professor of Pediatrics, Division of Neurology, School of Medicine at Oregon Health & Science University and principal investigator for both the MoveDMD and PolarisDMD trials. “I am pleased to continue to evaluate edasalonexent as a potential novel therapy for those affected by Duchenne, and I am excited to participate in the Phase 3 PolarisDMD study.”

The Phase 1 MoveDMD data demonstrate that:

- Edasalonexent was well tolerated, with all patients completing the 1-week study without serious adverse events or dose reductions and with no safety signals
- Edasalonexent was rapidly absorbed, with peak levels observed 2-6 hours after dosing and exposure was dose-dependent
- After 7 days of treatment, NF-kB-regulated genes were significantly decreased in a dose-dependent manner, confirming that edasalonexent inhibited NF-kB in boys with DMD

“These data support the potential of edasalonexent as a safe and effective foundational therapy for boys affected by Duchenne. Phase 1 informed dosing for the subsequent MoveDMD trial phases and the cumulative MoveDMD trial data provided key information to design our currently enrolling Phase 3 PolarisDMD trial,” said Joanne Donovan, Chief Medical Officer at Catabasis Pharmaceuticals. “Thank you to the boys, their families and the clinical trial staff in our MoveDMD trial. We deeply appreciate your partnership and support of our shared mission to improve the lives of all affected by Duchenne.”

The primary objective of the Phase 1 portion of the MoveDMD trial was to assess the safety and tolerability of edasalonexent, with the secondary objective to assess the PK and target engagement of edasalonexent in pediatric patients with DMD to provide guidance in the Phase 2 portion of the study. The one week, open-label, multiple-dose study had three ascending doses

of edasalonexent (33 mg/kg/day, 67 mg/kg/day and 100 mg/kg/day) administered to males diagnosed with DMD between the ages of 4 and 8 years old.

The results from the MoveDMD trial informed the Phase 3 PolarisDMD trial, which is underway and currently recruiting patients. The PolarisDMD clinical trial is a randomized, double-blind, placebo-controlled trial, with 2 boys receiving edasalonexent for each boy receiving placebo. The PolarisDMD trial is enrolling approximately 125 boys ages 4 to 7 (up to 8th birthday) regardless of mutation type who have not been on steroids for at least 6 months. The MoveDMD and PolarisDMD trials have many important aspects in common including the age range, patient population and key endpoints.

About Edasalonexent (CAT-1004)

Edasalonexent (CAT-1004) is an investigational oral small molecule that is being developed as a potential new standard of care for all patients affected by DMD, regardless of their underlying mutation. Edasalonexent inhibits NF- κ B, which is a key link between loss of dystrophin and disease progression in DMD. NF- κ B has a fundamental role in skeletal and cardiac muscle disease in DMD. We are currently enrolling our global Phase 3 PolarisDMD trial to evaluate the efficacy and safety of edasalonexent for registration purposes. 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. Edasalonexent continues to be dosed in the open-label extension of the MoveDMD trial. 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- κ B inhibitor in development for the treatment of Duchenne muscular dystrophy. Our global Phase 3 PolarisDMD trial is currently enrolling boys affected by Duchenne. For more information on edasalonexent and our Phase 3 PolarisDMD trial, please visit www.catabasis.com or www.twitter.com/catabasispharma.

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 Company's global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2018, 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