



Catabasis Pharmaceuticals Presents Edasalonexent, a Potential Foundational Treatment for Duchenne Muscular Dystrophy

-- Differentiating Safety and Tolerability Profile of Edasalonexent Through More Than 55 Patient Years of Exposure --

CAMBRIDGE, MA, October 5, 2019 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today presented the findings from the MoveDMD trial of edasalonexent. In the Phase 2 MoveDMD trial and open-label extension, edasalonexent slowed disease progression compared to the off-treatment control period and was well tolerated through more than 55 cumulative patient years of exposure in boys affected by Duchenne muscular dystrophy (DMD). These data were presented by Dr. Richard Finkel, M.D., Chief, Division of Neurology, Department of Pediatrics at Nemours Children’s Health System, and Principal Investigator for the Phase 2 MoveDMD and Phase 3 PolarisDMD studies of edasalonexent in DMD at the 24th International Annual Congress of the World Muscle Society.

“Our goal is to provide a therapy for DMD that slows disease progression, has a compelling safety profile and can be used in boys regardless of mutation,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “The safety and tolerability data from the MoveDMD trial support the potential of edasalonexent to become a foundational therapy for those with Duchenne, from the time of diagnosis onwards. Edasalonexent has broad potential for benefit and can be used as a monotherapy as well as potentially with other therapies. Our hope is to improve the quality of life for those affected by Duchenne.”

In the MoveDMD trial and open-label extension, edasalonexent preserved muscle function and substantially slowed disease progression compared to rates of change in the off-treatment control period, significantly improved biomarkers of muscle health and inflammation and was safe and well-tolerated. In more than 55 cumulative patient years of exposure, the majority of adverse events were mild in nature, and the most common treatment-related adverse event was diarrhea, generally mild and transient. There were no serious adverse events observed on treatment, and no adverse trends in chemistry, hematology, or measures of adrenal function. Edasalonexent is not a steroid and has not shown the known side effects of corticosteroids.

Edasalonexent is an investigational oral small molecule designed to inhibit NF-κB. In DMD the loss of dystrophin leads to chronic activation of NF-κB, which is a key driver of skeletal and cardiac muscle disease progression. Edasalonexent is currently being studied in the Phase 3 PolarisDMD trial, which has fully enrolled 130 boys with DMD, ages 4 to 7 (up to 8th birthday) with any mutation type and who had not been on steroids for the past 6 months. After the completion of 52 weeks of treatment, all boys and their eligible siblings are expected to have the option to enroll in GalaxyDMD, an open-label extension study designed to assess the long-term safety of

edasalonexent. Top-line results from the Phase 3 PolarisDMD trial are expected in the fourth quarter of 2020, and the trial is anticipated to support an NDA filing in 2021.

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 open-label extension trial GalaxyDMD. 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 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 Company's global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, including the anticipated timing for top-line results, potential timing for the filing of an NDA, 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 preclinical or 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 quarterly period ended June 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.

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