



Catabasis Pharmaceuticals Presents Data Supporting MRI T2 as a Potential Marker of Clinical Outcome in Duchenne Muscular Dystrophy at the World Muscle Society Congress

-- Data from Large Natural History Study Show Strong Correlation of Lower Leg Composite MRI T2 with DMD Disease Progression, Supporting Positive Edasalonexent Effects in the MoveDMD® Trial --

-- Positive MoveDMD Clinical Results Through 72 Weeks of Edasalonexent Treatment and Phase 3 PolarisDMD Trial Design Also Presented --

CAMBRIDGE, MA, October 3, 2018 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported new data supporting magnetic resonance imaging (MRI) T2 as a potential marker of clinical outcome in boys with Duchenne Muscular Dystrophy (DMD). Data from ImagingDMD, the largest natural history database of MRI assessments in boys with DMD, demonstrate a strong correlation between a composite of lower leg MRI T2 and boys' functional abilities. These results highlight the clinical importance of the significant improvement observed in the MoveDMD trial with edasalonexent treatment for lower leg MRI T2 compared to the off-treatment control period. These data were presented today in the late breaking session at the 23rd International Congress of the World Muscle Society in Mendoza, Argentina.

The utility of MRI T2 as a marker to predict disease progression in DMD was assessed in the ImagingDMD natural history database of more than 150 boys. The lower leg composite MRI T2 was highly correlated with functional abilities in boys with DMD. The lower leg composite MRI T2 correlated with ability to complete assessments of muscle function, with 2 milliseconds (MRI T2 measurement units) corresponding to clinically relevant functional changes. The MoveDMD edasalonexent trial also assessed MRI T2 and saw a statistically significant improvement in the lower leg composite MRI T2 rate of change following 12, 24, 36 and 48 weeks of edasalonexent treatment compared to the off-treatment control period, supporting the potential clinical benefit of edasalonexent. These improvements in MRI T2 are consistent with the improvements observed in all assessments of muscle function with edasalonexent treatment compared to the off-treatment control period.

“It is extremely exciting to see the MRI composite correlate with disease progression in boys with Duchenne in the natural history data as we have been interested in MRI measures as a potential marker of clinical outcome in Duchenne to make drug development more efficient,” said H. Lee Sweeney, Ph.D., Myology Institute Director, University of Florida and ImagingDMD co-Principal Investigator. “Current assessments of Duchenne rely on physical tests and invasive procedures. MRI represents an objective, sensitive and non-invasive way to assess disease progression in

Duchenne and will serve as an incredibly valuable tool as we look to evaluate and quickly make available new treatment options for the disease.”

“The potential of MRI T2 to predict disease progression and potential treatment benefit in Duchenne will greatly enhance the ability of investigators to assess clinical effects of investigational agents and hopefully lead to improved clinical management of boys living with the disease,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “This represents a promising step forward to better understand MRI endpoints and their utility in Duchenne to support our work to bring hope and life changing therapies to those affected by Duchenne. We are excited to have recently initiated our Phase 3 PolarisDMD trial for edasalonexent based on the positive results observed in the MoveDMD trial.”

Catabasis also presented new MoveDMD trial data and the Phase 3 PolarisDMD trial design at the International Congress of the World Muscle Society:

MoveDMD Trial Data through 72 Weeks of Edasalonexent Treatment

- New positive efficacy and safety results through 72 weeks of edasalonexent treatment were presented, showing preservation of muscle function and sustained disease-modifying effects in all assessments of muscle function in boys with DMD in the MoveDMD trial and open-label extension compared to the off-treatment control period.
- Significant decreases in muscle enzymes through 72 weeks were also seen in boys treated with edasalonexent, supporting the durability of edasalonexent treatment effects.
- Significantly decreased heart rate towards age-normative values was observed and supports the potential beneficial cardiac effects of edasalonexent. Boys with DMD in this age range typically have resting tachycardia, a heart rate that exceeds the normal resting rate, and is the first cardiac manifestation in boys with DMD. Cardiomyopathy is the leading cause of mortality in DMD.
- Edasalonexent continued to be well tolerated with no safety signals observed in the trial. Boys treated with edasalonexent continue to follow age-appropriate growth curves with age-appropriate increases in weight and height and overall BMI has trended down to age-normative values.

PolarisDMD Phase 3 Trial Design for Edasalonexent

- The recently initiated Phase 3 PolarisDMD trial will evaluate the efficacy and safety of edasalonexent in patients with DMD and is intended to support an application for commercial registration of edasalonexent.
- The PolarisDMD trial is expected to include approximately 40 clinical trial sites globally.
- The Phase 3 PolarisDMD trial is a one-year, randomized, double-blind, placebo-controlled trial. Catabasis plans to enroll approximately 125 patients ages 4 to 7 (up to 8th birthday) regardless of mutation type who have not been on steroids for at least 6 months.
- The primary efficacy endpoint will be change in the North Star Ambulatory Assessment score after 12 months of treatment with edasalonexent compared to placebo. Key

secondary endpoints include the age-appropriate timed function tests time to stand, 4-stair climb and 10-meter walk/run. Assessments of growth, cardiac and bone health are also included.

- Top-line results from the Phase 3 PolarisDMD trial are expected in the second quarter of 2020.

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 the key link between loss of dystrophin and disease pathology and plays a fundamental role in the initiation and progression of skeletal and cardiac muscle disease in DMD. Catabasis is currently enrolling the single global Phase 3 PolarisDMD trial to evaluate the efficacy and safety of edasalonexent for registration purposes. Edasalonexent continues to be dosed in an open-label extension of the MoveDMD Phase 2 clinical 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. The 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 June 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.

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