Catabasis Pharmaceuticals Announces Collaboration with the University of Texas Southwestern to Explore the Potential Benefits of Edasalonexent on Cardiac Function in Duchenne and Becker Muscular Dystrophies

November 13, 2018

-- One-Year Preclinical Study of Edasalonexent, a Novel Inhibitor of NF-kB, in Animal Models of Duchenne to Investigate Potential Improved Cardiac Function --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 13, 2018-- Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced a collaboration with Pradeep Mammen, MD, FACC, FAHA, founder and Medical Director of the Neuromuscular Cardiomyopathy Clinic at the University of Texas Southwestern (UT Southwestern) Medical Center as well as Co-Director of the National Institute of Health Sponsored UT Southwestern Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center. The mission of this center is to rapidly translate discoveries at the bench into therapies for Duchenne muscular dystrophy (DMD) in the clinic. The collaboration is designed to explore the potential of edasalonexent, a novel oral NF-kB inhibitor, to improve cardiac function in Duchenne and Becker muscular dystrophies.

Cardiomyopathy is the leading cause of mortality in Duchenne and Becker muscular dystrophies. Young boys with Duchenne typically have an elevated heart rate that exceeds the normal resting rate for age, which is the first cardiac manifestation in boys with DMD. Preclinical and clinical biomarker data support the potential for cardiac benefits with edasalonexent in Duchenne and Becker muscular dystrophies.

The one-year collaboration between Catabasis and Dr. Mammen will utilize the muscular dystrophy *mdx* mouse model with reduced utrophin as these animals display an early and prominent cardiomyopathy. The study will evaluate both functional and echocardiographic assessments of the heart as well as histological, biochemical and molecular assessments to study the potential benefits of edasalonexent treatment. Results are expected in the second half of 2019.

“Inhibiting NF-kB with edasalonexent offers a unique mechanism with the advantage of potentially impacting both skeletal and cardiac muscle disease in those living with Duchenne and Becker muscular dystrophies,” said Andrew Nichols, Chief Scientific Officer at Catabasis Pharmaceuticals. “With the promising preclinical and clinical biomarker data that we have seen to-date, we are excited to collaborate with Dr. Mammen to learn from his extensive experience and further explore the potential beneficial heart effects of edasalonexent in these muscular dystrophies.”

Dr. Mammen is an expert in advanced heart failure, with a focus on neuromuscular cardiomyopathy, and is board certified in advanced heart failure/VAD/transplant cardiology. As a treating physician at the UT Southwestern Medical Center, Dr. Mammen cares for Duchenne and Becker muscular dystrophy patients in his role as the Medical Director of the UT Southwestern’s dedicated Neuromuscular Cardiomyopathy Clinic, which he founded in 2010. In addition, Dr. Mammen is also the Director of Translational Research for the Advanced Heart Failure Program and investigates the molecular mechanisms underlying heart failure with more than 50 published journal articles. He holds the Alfred W. Harris, M.D. Professorship in Cardiology at UT Southwestern.

“There is significant unmet medical need for therapies that could treat both the skeletal and cardiac muscle disease in Duchenne and Becker muscular dystrophies,” said Pradeep Mammen, M.D., Medical Director of the Neuromuscular Cardiomyopathy Clinic at UT Southwestern. “I have dedicated my career to improving the lives of patients with heart failure, and I look forward to helping advance the understanding of edasalonexent and how it could benefit patients in the future.”

The collaboration builds upon preclinical and clinical biomarker data supporting the potential for cardiac benefits with edasalonexent. In the Catabasis Phase 2 MoveDMD clinical trial and open-label extension, significantly decreased heart rate towards age-normative values was observed in boys with DMD. Preclinical data in *mdx* mice and GRMD dogs, animal models of Duchenne, have shown substantially decreased cardiac fibrosis with NF-kB inhibition.

Edasalonexent is currently being studied as a potential treatment for Duchenne muscular dystrophy in the Phase 3 PolarisDMD clinical trial, a one-year, randomized, double-blind, placebo-controlled trial. Catabasis plans to enroll approximately 125 patients in the trial ages 4 to 7 (up to 8th birthday) regardless of mutation type who have not been on steroids for at least 6 months. In the clinic, edasalonexent has been shown to preserve muscle function and substantially slow DMD disease progression across all four assessments of muscle function (the North Star Ambulatory Assessment, time to stand, 4-stair climb, and 10-meter walk/run) compared to control. Edasalonexent has been well tolerated through more than 50 patient-years of treatment with no safety signals observed.

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-kB, which is a key link between loss of dystrophin and disease progression in DMD. NF-kB has a fundamental role in 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. In the clinic, 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 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-kB 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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