Catabasis Pharmaceuticals Announces Plans for Edasalonexent Phase 3 POLARIS DMD Trial in Duchenne Muscular Dystrophy

July 9, 2018

-- Global Phase 3 POLARIS DMD Trial Expected to Initiate in the Second Half of 2018 and Enroll Approximately 125 Patients --

-- Phase 2 MoveDMD® Trial and Open-Label Extension Showed Substantially Slowed Duchenne Disease Progression in Patients Treated with Edasalonexent --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 9, 2018-- Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced plans for the Phase 3 POLARIS DMD trial with edasalonexent in patients with Duchenne muscular dystrophy (DMD). Catabasis plans to initiate the global POLARIS DMD trial in the second half of 2018 with top-line results expected in the second quarter of 2020.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20180709005089/en/

The POLARIS DMD 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 trial design was informed by discussions with the U.S. Food and Drug Administration (FDA) as well as input from treating physicians and families of boys affected by Duchenne.

The randomized, double-blind, placebo-controlled POLARIS DMD trial has many key elements in common with the Phase 2 MoveDMD® trial, including the patient population and functional endpoints. Catabasis anticipates enrolling approximately 125 patients between the ages of 4 and 7 regardless of mutation type who have not been on steroids for at least 6 months. Boys on a stable dose of eteplirsen may be eligible to enroll. 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 are planned to 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 planned to be included. Two boys will receive edasalonexent for every boy that receives placebo and after 12 months, all boys are expected to receive edasalonexent in an open-label extension.

“We have designed a robust study with POLARIS DMD to evaluate edasalonexent as a potential new treatment for Duchenne. We have benefited from input from many people that are part of the Duchenne community and we are well underway with our preparations to begin the trial,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “We are very excited to advance edasalonexent through this potentially last phase of clinical development with the hope of providing a new treatment option to all boys affected by this disease. We believe that edasalonexent has great potential as a therapy to be taken on its own as well as in combination with other treatments.”

Edasalonexent is a potential oral foundational therapy that is being developed for all patients affected by DMD. Edasalonexent is being developed for use as monotherapy and in possible combination with dystrophin upregulation therapies. Edasalonexent has been shown to preserve muscle function and substantially slow Duchenne disease progression in the MoveDMD Phase 2 trial and open-label extension. Preclinical data and clinical biomarker data from the MoveDMD Phase 2 trial suggest that edasalonexent could have potential benefits in skeletal muscle, diaphragm and heart. Edasalonexent has been safe and well tolerated through more than 45 patient-years of treatment.

About Edasalonexent (CAT-1004)
Edasalonexent (CAT-1004) is an investigational oral small molecule that is being developed as a potential disease-modifying therapy for all patients affected by DMD, regardless of their underlying mutation. Edasalonexent inhibits NF-kB, a protein that is activated in DMD and drives inflammation, fibrosis and muscle degeneration and suppresses muscle regeneration. Edasalonexent continues to be dosed in an open-label extension of the MoveDMD Phase 2 clinical trial, and Catabasis is preparing to initiate a single global Phase 3 trial in the second half of 2018 to evaluate the efficacy and safety of edasalonexent for registration purposes. 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 reported to-date, 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. Edasalonexent was designed using our SMART (Safely Metabolized And Rationally Targeted) Linker drug discovery platform that enables us to engineer molecules that simultaneously modulate multiple targets in a disease. For more information on edasalonexent or our drug discovery platform, 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 plans to commence a single global Phase 3 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; the Company’s ability to obtain financing on acceptable terms and in a timely manner to fund the Company’s planned Phase 3 trial of edasalonexent in DMD for registration purposes; 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 March 31, 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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