



Catabasis Pharmaceuticals to Present at the 2017 Muscular Dystrophy Association Scientific Conference

March 13, 2017

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 13, 2017-- [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced that it will present data from Part B of the MoveDMD trial of edasalonexent (CAT-1004) for the treatment of Duchenne muscular dystrophy (DMD) at the 2017 Muscular Dystrophy Association Scientific Conference to be held March 19 – 22, 2017, in Arlington, VA, at the Hyatt Regency Crystal City.

Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis, will deliver oral and poster presentations titled “MoveDMD: Phase 1/2 trial of Edasalonexent, an NF-κB Inhibitor, in 4- to 7-Year-Old Patients with Duchenne Muscular Dystrophy.” The oral presentation will take place on Wednesday, March 22, 2017 from 11:15am – 11:45am ET. The poster presentations will take place during the poster sessions on Monday, March 20, 2017 from 6:45pm – 8:45pm and Tuesday, March 21, 2017 from 6:30pm – 8:30pm ET.

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 Duchenne muscular dystrophy (DMD or Duchenne), regardless of their underlying mutation. Edasalonexent inhibits NF-κB, a protein that is activated in Duchenne and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. In animal models of DMD, edasalonexent produced beneficial effects in skeletal, diaphragm and cardiac muscle and improved function. 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. We have previously reported safety, tolerability and reduction in NF-κB activity in Phase 1 trials in adults. We are currently conducting the MoveDMD® trial, a three-part clinical trial investigating the safety and efficacy of edasalonexent in boys ages 4 – 7 affected with DMD (any confirmed mutation). Part A of the trial evaluated the safety, tolerability and pharmacokinetics of, and NF-κB target engagement with, edasalonexent in 17 boys with DMD. Part B of the trial was a double-blind, placebo-controlled evaluation of the safety and efficacy of edasalonexent over a 12-week period in 31 boys. The primary efficacy end point for Part B was average change from baseline to week 12 in MRI T2 measures in boys given edasalonexent compared to placebo. Additional efficacy end points included age-appropriate timed function tests (10-meter walk/run, 4-stair climb and time to stand), North Star Ambulatory Assessment (NSAA), the pediatric outcomes data collection instrument (PODCI) and muscle strength. Part C is an open-label extension with edasalonexent for 36 weeks beyond Part B and will evaluate longer term safety and efficacy with the same clinical end points as Part B. From the MoveDMD trial, we have reported that edasalonexent was well tolerated with no safety signals. We reported top-line data for Part B that the primary efficacy end point was not met. The edasalonexent 100 mg/kg/day treatment group consistently showed numerical improvement versus placebo across multiple timed function tests and the NSAA, although as expected the changes were not statistically significant. The 67 mg/kg/day treatment group had mixed results compared with both the 100 mg/kg/day treatment group and placebo, which in each case were not statistically significant. Part C of the trial is ongoing.

About Catabasis

At Catabasis Pharmaceuticals, our mission is to bring hope and life-changing therapies to patients and their families. Our SMART (Safely Metabolized And Rationally Targeted) linker drug discovery platform enables us to engineer molecules that simultaneously modulate multiple targets in a disease. We are applying our SMART linker platform to build an internal pipeline of product candidates for rare diseases and plan to pursue partnerships to develop additional product candidates. For more information on the Company's drug discovery platform and pipeline of drug candidates, please visit www.catabasis.com.

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