



## Catabasis Pharmaceuticals Announces Top-Line Results for Part B of the MoveDMD® Trial for Edasalonexent (CAT-1004) in Duchenne Muscular Dystrophy

January 31, 2017

-- Conference Call Today at 4:30pm ET --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 31, 2017-- [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced top-line safety and efficacy results for Part B of the MoveDMD® trial of edasalonexent (CAT-1004) for the treatment of Duchenne muscular dystrophy (DMD). The objective of Part B of the MoveDMD trial was to evaluate the effects of edasalonexent using magnetic resonance imaging (MRI) T2 as a biomarker at 12 weeks. The primary efficacy end point of average change from baseline to week 12 in the MRI T2 composite measure of lower leg muscles for the pooled edasalonexent treatment groups of 67 mg/kg/day and 100 mg/kg/day compared to placebo was not met (0.37 milliseconds for the pooled edasalonexent treatment groups versus 0.47 milliseconds for placebo, a smaller increase in MRI T2 correlating with less muscle inflammation). The edasalonexent 100 mg/kg/day treatment group consistently showed numerical improvement versus placebo across multiple measures although 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. Catabasis plans to complete a full analysis of the data from Part B of the MoveDMD trial and to submit the data for presentation at an upcoming scientific conference. The open-label extension portion (Part C) of the MoveDMD trial is ongoing. Catabasis intends to report the results from Part C in 2017, with an interim update in Q2.

Thirty-one boys enrolled in Part B and all completed the trial. Both dose levels of edasalonexent evaluated were well tolerated with no safety signals observed. The majority of adverse events were mild in nature and the most common treatment-related adverse events were gastrointestinal, primarily mild diarrhea and vomiting. There were no treatment-related serious adverse events, no drug discontinuations and no dose reductions. Edasalonexent plasma exposure in Part B of the MoveDMD trial was consistent with that observed in Part A.

Additional efficacy end points included three age-appropriate timed function tests (10-meter walk/run, 4-stair climb and time to stand), the North Star Ambulatory Assessment (NSAA) and additional MRI measures. The trial was not powered to detect statistically significant changes in the timed function tests or NSAA and no significant changes were detected in these measures for those dosed with edasalonexent versus placebo. For all three timed function tests, the change in speed of performance from baseline to week 12 was numerically better for the 100 mg/kg/day treatment group compared to placebo. The change in NSAA was also numerically better for the 100 mg/kg/day treatment group compared to placebo. In addition to the lower leg, MRI T2 was also measured for the upper leg. The results in MRI T2 score for change from baseline to week 12 for the vastus lateralis (a large component of the quadriceps) were not statistically significant but showed a numerical improvement in the 100 mg/kg/day treatment group compared to placebo. Compared to the placebo group, patients in the edasalonexent 100 mg/kg/day group had characteristics of more advanced disease at baseline. Patients in this treatment group had been diagnosed at a younger age and, at baseline, did not perform as well on function tests.

"Although we did not meet the MRI T2 composite end point, the continued safety, tolerability and plasma exposure data in Part B of the MoveDMD trial are reassuring. We observed potential treatment-associated effects at 12 weeks in the 100 mg/kg/day treatment group, which we believe warrant further evaluation to see if the signals strengthen in the longer-term data from the ongoing open-label extension. Following additional data analysis from the open-label extension, we will determine the next steps for edasalonexent in DMD," said Jill C. Milne, Chief Executive Officer of Catabasis. "We are enormously grateful to the boys and the families involved in the MoveDMD trial as well as the clinical trial site staff and patient groups who are making this trial possible."

"The top-line data results from Part B of the MoveDMD trial provide us with an early snapshot of the effects of edasalonexent in boys with DMD over a 12-week period. Continuing the open-label extension of the MoveDMD trial will allow us to further evaluate the potential for edasalonexent to provide benefit in DMD," said Richard Finkel, M.D., Division Chief, Division of Neurology, Department of Pediatrics at Nemours Children's Health System and a Principal Investigator for the study. "The unmet medical need in Duchenne is profound and potential therapies that benefit all patients are needed."

"We in the DMD community continue to learn from each completed clinical trial and appreciate the effort of Catabasis in studying a potential therapy that could be applicable for all of those affected by DMD regardless of the underlying mutation type," said Pat Furlong, Founding President and Chief Executive Officer of Parent Project Muscular Dystrophy (PPMD).

### About Part B of the MoveDMD Trial

Part B of the MoveDMD trial was a randomized, double-blind, placebo-controlled trial with 31 ambulatory boys between ages 4 and 7 with a genetically confirmed diagnosis of DMD across a range of dystrophin mutations. The boys were steroid naive. This portion of the trial was conducted at five sites in the U.S., and assessed the safety and efficacy of edasalonexent in patients at two dosing levels (67 mg/kg/day and 100 mg/kg/day) or placebo before and after 12 weeks of dosing. The 67 mg/kg/day treatment group was dosed 33 mg/kg twice per day and the 100 mg/kg/day treatment group was dosed 33 mg/kg three times per day. Sixteen of the 17 boys who participated in the first part of the MoveDMD trial (Part A) participated in Part B. The primary efficacy end point in Part B for which the trial was powered was average change from baseline to week 12 in MRI T2 measure for a

composite of five lower leg muscles for the pooled edasalonexent treatment groups, 67 mg/kg/day and 100 mg/kg/day, compared to placebo. MRI T2 is a measure of the composition of muscle and an indicator of muscle inflammation. Safety and tolerability were also evaluated. Additional assessments were measured; however, the trial was not powered for statistical significance for these assessments. The additional assessments include timed function tests (10-meter walk/run, 4-stair climb and time to stand), the North Star Ambulatory Assessment (NSAA), muscle strength measures and the pediatric outcomes data collection instrument (PODCI).

The open-label extension (Part C) was initiated in July and includes dosing with edasalonexent for 36 weeks beyond Part B and will evaluate longer term safety and efficacy with the same clinical end points as in Part B. PPMD and the Muscular Dystrophy Association have provided funding to support participant travel for the MoveDMD trial.

More information about the MoveDMD trial can be found on the [clinical trials page](#) of the Catabasis website and on [ClinicalTrials.gov](#) under trial identifier NCT02439216.

Catabasis today also announced that Rick Modi, Chief Business Officer, has submitted his resignation to pursue a new opportunity.

#### **Conference Call Dial-In Information:**

Catabasis will host a conference call and webcast today, January 31, 2017, at 4:30pm ET to discuss the top-line results for Part B of the MoveDMD trial.

Participant Toll-Free Dial-In Number: (877) 388-2733  
Participant International Dial-In Number: (541) 797-2984  
Pass Code: 62428972

Please specify to the operator that you would like to join the "Catabasis MoveDMD Part B Results Call."

Interested parties may access a live audio webcast of the conference call via the investor section of the Catabasis website, [www.catabasis.com](http://www.catabasis.com). Please connect to the Catabasis website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. The webcast will be archived for 90 days.

#### **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- $\kappa$ B, 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- $\kappa$ B activity in Phase 1 trials in adults. We are currently conducting the MoveDMD<sup>®</sup> 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- $\kappa$ B 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), muscle strength and the pediatric outcomes data collection instrument (PODCI). 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 full analyses of data from Part B of the trial and from the ongoing open-label extension are pending.

#### **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](http://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 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; availability and timing of results from preclinical studies and clinical trials; 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 period ended September 30, 2016, 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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