



Catabasis Pharmaceuticals Completes Target Enrollment for Part B of the MoveDMD[®] Trial, a Phase 2 Trial of Edasalonexent (CAT-1004) for the Potential Treatment of Duchenne Muscular Dystrophy

- Trial Evaluates MRI T2 as Primary End Point in Boys Affected by DMD Regardless of Mutation Type; Top-line Results Expected in the First Half of Q1 2017 -

CAMBRIDGE, MA, October 4, 2016 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ: CATB), a clinical-stage pharmaceutical company, today announced that target enrollment of 30 patients has been reached for Part B of the MoveDMD trial, a 12-week trial to assess the safety and efficacy of edasalonexent (CAT-1004) in Duchenne muscular dystrophy (DMD). Edasalonexent is an oral small molecule that the Company believes has the potential to be a disease-modifying therapy for DMD patients, regardless of their underlying dystrophin mutation. Catabasis has previously reported positive safety, tolerability, pharmacokinetics and biomarker results from Part A of the trial. Based on scheduling of the enrolled patients as of September 30, 2016, the Company expects to report top-line safety and efficacy results from this Phase 2 trial in the first half of Q1 2017.

“Completing target enrollment in Part B of our MoveDMD trial is another important milestone in the development of edasalonexent. We appreciate the exceptional support that we have received from the DMD community that has resulted in enrolling Part B of the trial,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer and Senior Vice President, Clinical Development at Catabasis. “We are grateful to the participants and their families as well as the clinical trial site staff involved.”

“It is encouraging to see the progress of this potential therapy given the profound unmet medical need in Duchenne and our desire to have therapies with the potential to benefit all of those affected regardless of the underlying mutation type,” said Pat Furlong, Founding President and Chief Executive Officer of Parent Project Muscular Dystrophy (PPMD).

Edasalonexent is an inhibitor of NF- κ B, a protein that is activated in DMD as well as multiple other diseases. Inhibition of NF- κ B has the potential to slow muscle degeneration and enhance muscle regeneration.

In the first portion of the MoveDMD trial (Part A), 17 ambulatory boys between ages 4 and 7 with a genetically confirmed diagnosis of DMD across a range of dystrophin mutations received edasalonexent. The boys were steroid naive or had not used steroids for at least six months prior to the trial. This portion of the trial was conducted at three sites in the U.S., and assessed the safety, tolerability and pharmacokinetics of edasalonexent in patients at three dosing levels (33 mg/kg/day, 67 mg/kg/day and 100 mg/kg/day) during seven days of dosing. Sixteen of the boys who participated in the first part of the MoveDMD trial (Part A) are participating in Part B. Phase 2 of the MoveDMD trial (Part B) is a randomized, double-blind, placebo-controlled trial of approximately 30 boys to evaluate the safety and efficacy of edasalonexent in DMD over a 12-week period at five clinical trial sites in the U.S. at two dosing levels, 67 mg/kg/day and 100 mg/kg/day.

The open-label extension (Part C) was initiated in July and includes dosing with edasalonexent for 36 weeks beyond the 12-week placebo-controlled portion of the trial and will evaluate longer term safety and efficacy with the same clinical end points. PPMD and the Muscular Dystrophy Association are providing 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.

About Edasalonexent (CAT-1004)

Edasalonexent (CAT-1004) is an oral small molecule that has the potential to be a disease-modifying therapy for all patients affected by Duchenne muscular dystrophy (DMD or Duchenne), regardless of their underlying mutation. Edasalonexent inhibits NF- κ 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- κ B activity in Phase 1 trials in adults. We are currently conducting the MoveDMD[®] trial of edasalonexent in 4-7 year-old boys affected by Duchenne. From Part A of the MoveDMD trial, we have reported that edasalonexent was generally well tolerated with no safety signals observed and we observed NF- κ B target engagement. Pharmacokinetic results demonstrated edasalonexent plasma exposure levels consistent with those previously observed in adults, at which inhibition of NF- κ B was observed.

About MoveDMD[®]

The MoveDMD trial is 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 MoveDMD trial evaluated the safety, tolerability and pharmacokinetics of, and NF- κ B target engagement with, edasalonexent and showed positive results. Part B of the trial is a Phase 2 trial to evaluate the safety and efficacy of edasalonexent in DMD over a 12-week period in approximately 30 boys. The primary end point is change in MRI of the lower leg muscles, and the secondary end points are age-appropriate timed function tests: 10-meter walk/run, 4-stair climb and time to stand. Additional assessments include muscle strength, the North Star Ambulatory Assessment and the pediatric outcomes data collection instrument (PODCI). Part C is an open-label extension that includes dosing with edasalonexent for 36 weeks beyond the 12-week placebo-controlled portion of the trial (Part B) and will evaluate longer term safety and efficacy with the same clinical end points as Part B.

About MRI

Magnetic resonance imaging (MRI) is a non-invasive imaging technique that can assess muscle structure and composition and measure disease status in children with DMD. Two MRI measures used in Duchenne to indicate muscle degeneration are T2 and fat fraction. MRI is sensitive to changes in muscle structure and composition induced by disease processes such as the inflammation, edema, muscle damage and fat infiltration that occur in Duchenne. Changes in T2 may be seen in less than 12 weeks while changes in fat fraction may take longer. Changes in these MRI measures have been correlated with longer-term changes in clinically meaningful measures of functional activity. Changes in MRI can show the effects of an investigational therapy on disease progression in Duchenne in an objective and quantifiable manner.

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.

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, including any impact of scheduling and potential rescheduling of patient assessments on the timing of the release of data from 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 June 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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