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Catabasis Pharmaceuticals Initiates Part B of the MoveDMDSM Trial of CAT-1004 for the Treatment of Duchenne Muscular Dystrophy

- Positive NF-kB Biomarker Data from Part A of the MoveDMD Trial in Patients -

CAMBRIDGE, MA, April 12, 2016 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced that dosing of the first patient has been initiated in Part B of the MoveDMD trial, a 12-week trial to assess the efficacy of CAT-1004 in Duchenne muscular dystrophy (DMD). Catabasis also announced positive biomarker results from Part A of the MoveDMD trial, demonstrating successful NF-kB target engagement. The biomarker assay piloted in boys affected by DMD showed a statistically significant decrease in NF-kB gene expression markers compared to baseline at the 67 mg/kg and 100 mg/kg per day doses as well as a statistically significant dose response in the gene expression data measured from whole blood samples. Catabasis has previously reported positive safety, tolerability and pharmacokinetics results from Part A of the trial.

“We are very glad to advance CAT-1004 into Phase 2 with the initiation of Part B of the MoveDMD trial in boys affected by DMD,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “We are pleased with the positive NF-kB biomarker results in Part A of the trial demonstrating target engagement as well as the 5 - 10 fold higher CAT-1004 concentration that we have seen in muscle compared to plasma in pre-clinical models.”

“I am glad to see the advancement of this novel potential therapy in boys affected by Duchenne,” said H. Lee Sweeney, Ph.D., Professor, Director, Myology Institute at the University of Florida. “Therapies that have the potential to make a meaningful difference are needed to address the profound unmet medical need in DMD.”

CAT-1004 is an oral small-molecule that the Company believes has the potential to be a disease-modifying therapy for the treatment of DMD, regardless of the underlying dystrophin mutation. CAT-1004 is an inhibitor of NF-kB, a protein that is chronically activated in DMD as well as multiple other skeletal muscle disorders and rare diseases. In animal models of DMD, CAT-1004 inhibited NF-kB, reduced muscle degeneration and increased muscle regeneration.

The MoveDMD trial is being conducted in two sequential parts, Part A and Part B. In Part A of the MoveDMD trial, 17 ambulatory boys between ages 4 and 7 with a genetically confirmed diagnosis of DMD across a range of dystrophin mutations received CAT-1004. The boys were steroid naive or had not used steroids for at least six months prior to the trial. Part A of the trial was conducted at three sites in the U.S., and assessed the safety, tolerability and pharmacokinetics of CAT-1004 in patients at three dosing levels (33 mg/kg/day, 67 mg/kg/day and 100 mg/kg/day) during seven days of dosing. Part B is a randomized, double-blind, placebo-controlled trial to evaluate the

safety and efficacy of CAT-1004 in DMD over a 12-week period at 5 clinical trial sites in the U.S. at two dosing levels, 67 mg/kg/day and 100 mg/kg/day. The boys that participated in Part A of the MoveDMD trial are asked to participate in Part B and additional participants are expected to be enrolled for a total of approximately 30 boys. We are currently identifying additional patients who are interested in participating in Part B of the trial. Entry criteria are similar to those in Part A. The Parent Project Muscular Dystrophy 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 CAT-1004

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 the underlying mutation. CAT-1004 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, CAT-1004 inhibited NF- κ B, reduced muscle degeneration and improved muscle regeneration and function, and beneficial effects were observed in skeletal, diaphragm and cardiac muscle. The FDA has granted orphan drug, fast track and rare pediatric disease designations and the European Commission has granted orphan medicinal product designation to CAT-1004 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 MoveDMDSM trial of CAT-1004 in 4-7 year-old boys affected by Duchenne. From Part A of the MoveDMD trial, we have reported that CAT-1004 was generally well tolerated with no safety signals observed and successful NF- κ B target engagement. Pharmacokinetic results demonstrated CAT-1004 average plasma exposure levels consistent with those previously observed in adults at which inhibition of NF- κ B was observed.

About MoveDMDSM

MoveDMD is a Phase 1 / 2 clinical trial of CAT-1004 in boys ages 4-7 affected with DMD (any confirmed mutation). The MoveDMD trial is a two-part clinical trial investigating the safety and efficacy of CAT-1004 in DMD. Part A of the MoveDMD trial evaluated the safety, tolerability and pharmacokinetics of CAT-1004 with positive results. The boys in Part A of the trial will be asked to participate, if eligible, in Part B of the trial. Part B of the trial will be planned to evaluate the safety and efficacy of CAT-1004 in DMD over a 12-week treatment period and will enroll approximately 30 boys. The primary end point is changes in MRI of the leg muscles, and the secondary end point is age-appropriate timed function tests. Additional assessments include muscle strength, the North Star Ambulatory Assessment and the pediatric outcomes data collection tool (PODCI).

About MRI

Magnetic resonance imaging (MRI) is a non-invasive imaging technique that can visualize 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. We have product candidates in both rare diseases and serious lipid disorders. 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. 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 Annual Report on Form 10-K for the year ended December 31, 2015, and in other filings that the Company may make with the Securities and Exchange Commission in the future. The forward-looking statements contained in this presentation reflect our current views with respect to future events, and we do not undertake, and specifically disclaim, any obligation to update any forward-looking statements.

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