



Catabasis Pharmaceuticals Completes Enrollment for Part A of the MoveDMDSM Trial, a Phase 1/2 Trial of CAT-1004 for the Treatment of Duchenne Muscular Dystrophy

CAMBRIDGE, MA, December 18, 2015 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ: CATB), a clinical-stage drug development company built on a pathway pharmacology technology platform, today announced that enrollment is complete in Part A of the MoveDMD trial, a Phase 1/2 trial of CAT-1004 for the treatment of Duchenne muscular dystrophy (DMD). CAT-1004 is an oral small-molecule that the Company believes has the potential to be a disease-modifying therapy for DMD patients, regardless of the underlying dystrophin mutation. CAT-1004 is an inhibitor of NF- κ B, a protein that is activated in DMD as well as multiple other skeletal muscle disorders. In animal models of DMD, CAT-1004 inhibited activated NF- κ B, reduced muscle inflammation and degeneration and increased muscle regeneration. DMD is a rare disease that involves progressive muscle degeneration that eventually leads to death and for which there are no approved therapies in the United States.

“The completion of enrollment in Part A of our MoveDMD trial is an important milestone in our development program for CAT-1004 and we look forward to reporting the safety, tolerability and pharmacokinetics results from this study in early Q1 2016,” said Joanne Donovan, M.D., Ph.D., chief medical officer at Catabasis. “We are grateful to the participants and their families as well as the clinical trial site staff who have made this possible and appreciate the enthusiasm and support that we have received from the DMD community.”

The MoveDMD trial enrolled ambulatory boys between ages 4 and 7 with a genetically confirmed diagnosis of DMD across a range of dystrophin mutations. The enrolled boys are steroid naive or have not used steroids for at least six months prior to the trial. The trial is being conducted at three sites in the United States in two sequential parts, Part A and Part B. Part A of the study assessed the safety, tolerability and pharmacokinetics of CAT-1004 in patients at three dosing levels following seven days of dosing. Part B will be a randomized, double-blind, placebo-controlled trial to evaluate the safety and efficacy of CAT-1004 in DMD over a 12-week period. The boys in the first part of the trial will be asked to participate, if eligible, in the second part of the trial, and additional participants will also be enrolled. We are currently identifying additional patients who are interested in participating in Part B of the trial. Entry criteria are expected to be similar to those in Part A.

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 inhibits activated NF- κ B, a protein that coordinates cellular response to muscular damage, stress and inflammation and plays an important role in muscle health. In skeletal muscle, activated NF- κ B drives muscle degeneration and suppresses muscle regeneration. In animal models of DMD, CAT-1004 inhibited activated NF- κ B, reduced muscle inflammation and degeneration and increased muscle regeneration. In Phase 1 clinical trials, CAT-1004 inhibited activated NF- κ B and was well tolerated with no observed safety concerns. The FDA has granted CAT-1004 orphan drug, fast track and rare pediatric disease designations for the treatment of DMD. The European Commission has granted CAT-1004 orphan medicinal product designation for DMD. Catabasis is currently conducting the MoveDMD Phase 1 / 2 trial of CAT-1004 in 4-7 year-old boys with DMD.

About MoveDMD

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 will be a two-part clinical trial investigating the safety and efficacy of CAT-1004 in DMD. The first part of the MoveDMD trial will include 7 days of treatment with CAT-1004 with the goal of evaluating the safety, tolerability and pharmacokinetics of CAT-1004. In addition, the Company will collect data at baseline on the muscles of the lower and upper legs using MRI, physical function (including timed function tests), and muscle strength. The boys in the first part of the trial will be asked to participate, if eligible, in the second part of the trial. The second part of the trial will be planned to evaluate the safety and efficacy of CAT-1004 in DMD over a 12-week period. Additional details of the second part of the trial will be available once the first part is complete and the protocol is finalized.

About Catabasis

Catabasis Pharmaceuticals is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics using its proprietary Safely Metabolized And Rationally Targeted, or SMART, linker technology platform. The Company's SMART linker technology platform is based on the concept of treating diseases by simultaneously modulating multiple targets in one or more related disease pathways. The Company engineers bi-functional product candidates that are conjugates of two molecules, or bioactives, each with known pharmacological activity, joined by one of its proprietary SMART linkers. The SMART linker conjugates are designed for enhanced efficacy and improved safety and tolerability. The Company's focus is on treatments for rare diseases. The Company is also developing other product candidates for the treatment of serious lipid disorders. For more information on the Company's technology 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," 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 three months ended September 30, 2015, 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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