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### **Muscular Dystrophy Association to Collaborate with Catabasis Pharmaceuticals on Development of CAT-1004, a Potential Novel Oral Disease-Modifying Treatment for Duchenne Muscular Dystrophy**

**CAMBRIDGE, MA, and CHICAGO, IL, February 5, 2016** – [Catabasis Pharmaceuticals, Inc.](http://www.catabasis.com) (NASDAQ:CATB), a clinical-stage drug development company built on a pathway pharmacology technology platform, and the Muscular Dystrophy Association (MDA), today announced a collaboration to support the Part B portion of the MoveDMD clinical trial of CAT-1004, a novel product candidate for the treatment of patients with Duchenne muscular dystrophy (DMD). Under the collaboration, MDA will provide funding for transportation to participants who need to travel to take part in the study. Part A of the MoveDMD trial has completed and Catabasis recently reported positive top-line results for safety, tolerability and pharmacokinetics. Part B of the trial is expected to initiate in the first half of 2016. The boys in Part A of the trial will be asked to participate in Part B and additional participants will also be enrolled. Catabasis is currently identifying additional patients who are interested in participating in Part B of the trial. Enrollment criteria for Part B are expected to be similar to those for Part A.

The incidence of DMD in the U.S. population is estimated to be between one in every 3,500 to one in 6,000 male births. The loss of a key muscle protein called dystrophin causes muscle wasting and weakness, eventually leading to the loss of ambulation, difficulty breathing, and heart failure. Life expectancy is currently the mid- to late 20s.

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

“Catabasis and the Muscular Dystrophy Association share the mission to provide all boys suffering from DMD with the hope of a new and effective treatment,” said Joanne Donovan, M.D., Ph.D., Catabasis Chief Medical Officer. “We are grateful for the support from MDA for this study and look forward to success in this collaboration for our patients, their families and all of those who are impacted by this disease.”

“The DMD community desperately needs and deserves treatment options that will help families by allowing for everyday freedoms that many take for granted such as fastening a button without assistance, moving into a more comfortable position independently, or just being able to hug the people you love,” said MDA Executive Vice President and Chief Medical and Scientific Officer

Valerie Cwik, M.D. “We are proud to partner with Catabasis and look forward to what is sure to be an exciting partnership that will work toward bringing urgently needed treatments to our families.”

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

### **About CAT-1004**

CAT-1004 is an oral small molecule that inhibits activated NF-κB, 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-κB drives muscle degeneration and suppresses muscle regeneration. In animal models of DMD, CAT-1004 inhibited activated NF-κB, reduced muscle inflammation and degeneration and increased muscle regeneration. In Phase 1 clinical trials, CAT-1004 inhibited activated NF-κB and was well tolerated with no observed safety concerns in adults. 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 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. In addition, the Company collected 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 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 period. Additional details of the second part of the trial will be available once the protocol is finalized.

### **About the Muscular Dystrophy Association**

MDA is leading the fight to free individuals — and the families who love them — from the harm of muscular dystrophy, ALS and related muscle-debilitating diseases that take away physical strength, independence and life. We use our collective strength to help kids and adults live longer and grow stronger by finding research breakthroughs across diseases; caring for individuals from day one; and empowering families with services and support in hometowns across America. Learn how you can fund cures, find care and champion the cause at [mda.org](https://mda.org).

## 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](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," 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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