



**FOR IMMEDIATE RELEASE**

**Catabasis Pharmaceuticals Initiates MoveDMD, a Phase 1/2 Trial of CAT-1004 for the Treatment of Duchenne Muscular Dystrophy**

**CAMBRIDGE, MA, June 17, 2015 –** [Catabasis Pharmaceuticals, Inc.](#), a clinical-stage drug development company built on a pathway pharmacology technology platform, today announced that dosing of the first patient has been initiated in 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, by inhibiting activated NF- $\kappa$ B. NF- $\kappa$ B is a protein that plays an important role in regulating muscle health, and chronic activation of NF- $\kappa$ B has been reported in multiple skeletal muscle disorders, such as DMD. Preclinical studies have demonstrated that CAT-1004 can not only reduce muscle inflammation and degeneration, but also promote 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 MoveDMD trial will enroll approximately 18 ambulatory boys between ages 4 and 7 with a genetically confirmed diagnosis of DMD, regardless of the specific dystrophin mutation. The enrolled boys will be steroid naive or have not used steroids for at least six months prior to the trial. The trial will be conducted at three sites in the United States in two sequential parts, Part A and Part B. Part A of the study will assess the safety, tolerability and pharmacokinetics of CAT-1004 in patients at three dosing levels following seven days of dosing. The primary endpoint for Part A is safety. Part B will be a randomized, double-blind, placebo-controlled trial. The primary efficacy endpoint in Part B will be change in muscle inflammation as measured by magnetic resonance imaging (MRI) of leg muscles. Additional measures of physical function and muscle strength will be collected.

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- $\kappa$ 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- $\kappa$ B drives muscle degeneration and suppresses muscle regeneration. In animal models of DMD, CAT-1004 inhibited activated NF- $\kappa$ B, reduced muscle inflammation and degeneration and increased muscle regeneration. In Phase 1 clinical trials, CAT-1004 inhibited activated NF- $\kappa$ B and was well-tolerated with no observed safety concerns. The FDA has granted CAT-1004 orphan drug designation for the treatment of DMD.

**About MoveDMD**

MoveDMD is a Phase 1/2 clinical trial of CAT-1004 in boys ages 4 to 7 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](http://www.catabasis.com).

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