



**Catabasis Pharmaceuticals Announces Positive Top-Line Results from Part A of the MoveDMD<sup>SM</sup> Trial, a Phase 1 / 2 Trial of CAT-1004 for the Treatment of Duchenne Muscular Dystrophy**

- Trial Demonstrated Favorable Safety, Tolerability and Pharmacokinetics in Patients -
- Results Support Initiation of Part B of Trial, Expected in First Half of 2016 -

**CAMBRIDGE, MA, January 25, 2016** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ: CATB), a clinical-stage drug development company built on a pathway pharmacology technology platform, today announced positive top-line results from Part A of the MoveDMD trial, a Phase 1 / 2 trial of CAT-1004 for the treatment of Duchenne muscular dystrophy (DMD or Duchenne). All three doses of CAT-1004 tested were generally well tolerated with no safety signals observed. The majority of adverse events were mild in nature, and the most common adverse events were gastrointestinal, primarily diarrhea. There were no serious adverse events and no drug discontinuations. Pharmacokinetics results demonstrated CAT-1004 average plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-kB was observed. Based on these results, Catabasis plans to initiate Part B of the MoveDMD trial in the first half of 2016. Catabasis plans to submit the data from Part A of the trial for presentation at an upcoming medical meeting.

CAT-1004 is an oral small-molecule that the Company 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-kB, 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-kB, reduced muscle degeneration and increased muscle regeneration.

“We are pleased with these results and believe that they support the initiation of Part B of the MoveDMD trial in the first half of 2016” said Jill Milne, Ph.D., Chief Executive Officer of Catabasis. “We sincerely thank the boys, their families, the investigators and the study site staff who participated in the trial as well as the advocacy organizations for their support of the CAT-1004 program.”

“The unmet medical need in Duchenne is profound and potential therapies that could make a meaningful difference are needed” said Richard Finkel, M.D., Division Chief, Division of Neurology, Department of Pediatrics at Nemours Children’s Health System. “Showing positive safety, tolerability and pharmacokinetics results is an important milestone in the development of CAT-1004. I look forward to the advancement of this novel potential therapy.”

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 have 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 100mg/kg/day) during 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 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- $\kappa$ 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 in adults, CAT-1004 inhibited activated NF- $\kappa$ 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 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 treatment period. Additional details of Part B of the trial will be available once 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).

### **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.

###

### **Corporate and Media Contact**

Andrea Matthews

Catabasis Pharmaceuticals, Inc.

T: (617) 349-1971

[amatthews@catabasis.com](mailto:amatthews@catabasis.com)