

## FOR IMMEDIATE RELEASE

# Catabasis Pharmaceuticals Will Present CAT-1004, a Potential Disease-Modifying Agent in Development for the Treatment of Duchenne Muscular Dystrophy, at the XIV International Conference on Duchenne and Becker Muscular Dystrophy

**CAMBRIDGE, MA, February 10, 2016** – <u>Catabasis Pharmaceuticals, Inc.</u> (NASDAQ:CATB), a clinical-stage biopharmaceutical company focused on bringing transformative therapies to patients with rare diseases, today announced that Catabasis will present CAT-1004 for the treatment of Duchenne muscular dystrophy (DMD) and the MoveDMD<sup>SM</sup> trial design at the XIV International Conference on Duchenne and Becker Muscular Dystrophy. The conference will be held February 13 – 14, 2016, in Rome, Italy, at the Church Palace.

 Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis, will give a presentation titled "CAT-1004, an Oral Agent Targeting NF-kB in Development for Treatment of Duchenne Muscular Dystrophy." The presentation will take place as part of a session on Sunday, February 14, 2016, from 8:40am – 11:00am local time.

#### About CAT-1004

CAT-1004 is an oral small molecule that has the potential to be a disease-modifying therapy for all patients affected with Duchenne muscular dystrophy (DMD or Duchenne), regardless of the underlying mutation. CAT-1004 inhibits NF-κB, 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-κB, 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 are currently conducting the MoveDMD<sup>SM</sup> trial of CAT-1004 in 4-7 year-old boys affected by Duchenne and have previously reported safety, tolerability and reduction in NF-kB activity in Phase1 trials.

#### About MoveDMD

MoveDMD<sup>SM</sup> 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 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 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 when used in conjunction with clinical measures 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. Our corporate focus is to discover, develop and commercialize transformative therapies for rare diseases. 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 in rare diseases and aim to target broader disease opportunities via external collaborations. For more information on the Company's drug discovery platform and pipeline of drug candidates, please visit <u>www.catabasis.com</u>.

###

Corporate and Media Contact Andrea Matthews Catabasis Pharmaceuticals, Inc. T: (617) 349-1971 amatthews@catabasis.com