



Catabasis Pharmaceuticals Phase 1 Data on Edasalonexent (CAT-1004), a Potential Disease-Modifying Therapy Being Developed for Duchenne Muscular Dystrophy, Published in the Journal of Clinical Pharmacology

January 19, 2017

-- *Edasalonexent Was Safe, Well Tolerated and Generated Positive Biomarker Results in Adult Subjects* --

-- *Top-Line Phase 2 Results in Boys with Duchenne Muscular Dystrophy On Track: Expected in the First Half of Q1 2017* --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 19, 2017-- [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced the publication of Phase 1 data on edasalonexent in adult subjects. Edasalonexent is a potential disease-modifying therapy being developed for Duchenne muscular dystrophy (DMD). The Phase 1 trials demonstrated that edasalonexent (CAT-1004), an oral inhibitor of NF-kB, was safe, well tolerated, and inhibited activated NF-kB in adult subjects and the data are presented in an article titled "A Novel NF-kB Inhibitor, Edasalonexent (CAT-1004), in Development as a Disease-Modifying Treatment for Patients with Duchenne Muscular Dystrophy: Phase 1 Safety, Pharmacokinetics, and Pharmacodynamics in Adult Subjects" in the *Journal of Clinical Pharmacology* (J Clin Pharmacol. 2017 Jan 11. doi: 10.1002/jcph.842.)

In Duchenne muscular dystrophy (DMD), NF-kB is activated in muscle from infancy regardless of the underlying dystrophin mutation and drives inflammation and muscle degeneration while inhibiting muscle regeneration. Edasalonexent (CAT-1004) is a bifunctional orally administered small molecule that covalently links two compounds known to inhibit NF-kB, salicylic acid and docosahexaenoic acid (DHA). The three placebo-controlled trials in adult subjects assessed the safety, pharmacokinetics and pharmacodynamics of single or multiple edasalonexent doses up to 6000 mg (approximately 100 mg/kg). Seventy-nine adult subjects received edasalonexent and 25 received placebo. The NF-kB pathway and proteasome gene expression profiles in peripheral mononuclear cells were significantly decreased after 2 weeks of edasalonexent treatment. NF-kB activity was inhibited following a single dose of edasalonexent but not by equimolar doses of its component bioactives salicylic acid and DHA dosed in combination. Edasalonexent was well tolerated, and the most common adverse events were mild diarrhea and headache.

"These Phase 1 safety, tolerability and positive NF-kB biomarker results support edasalonexent development in Duchenne muscular dystrophy and potentially other diseases. The Phase 1 results in adults informed on the dose and dose schedule for the current MoveDMD trial in 4-7 year-old boys affected by Duchenne, where similar Phase 1 results were seen," said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. "We look forward to the results from the edasalonexent Phase 2 clinical trial in boys affected by Duchenne, which are expected in the first half of Q1 2017."

About Edasalonexent (CAT-1004)

Edasalonexent (CAT-1004) is an oral small molecule that has the potential to be a disease-modifying therapy for all patients affected by Duchenne muscular dystrophy (DMD or Duchenne), regardless of their underlying mutation. Edasalonexent inhibits NF-kB, a protein that is activated in Duchenne and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. In animal models of DMD, edasalonexent produced beneficial effects in skeletal, diaphragm and cardiac muscle and improved function. The FDA has granted orphan drug, fast track and rare pediatric disease designations and the European Commission has granted orphan medicinal product designation to edasalonexent for the treatment of DMD. We have previously reported safety, tolerability and reduction in NF-kB activity in Phase 1 trials in adults. We are currently conducting the MoveDMD® trial of edasalonexent in 4-7 year-old boys affected by Duchenne. From Part A of the MoveDMD trial, we have reported that edasalonexent was generally well tolerated with no safety signals observed and we observed NF-kB target engagement. Pharmacokinetic results demonstrated edasalonexent plasma exposure levels consistent with those previously observed in adults, at which inhibition of NF-kB was observed.

About Catabasis

At Catabasis Pharmaceuticals, our mission is to bring hope and life-changing therapies to patients and their families. 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 for rare diseases and plan to pursue partnerships to develop additional product candidates. For more information on the Company's drug discovery platform 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," "may" 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 period ended September 30, 2016, 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.

View source version on businesswire.com: <http://www.businesswire.com/news/home/20170119005231/en/>

Source: Catabasis Pharmaceuticals, Inc.

Catabasis Pharmaceuticals, Inc.
Andrea Matthews, 617-349-1971
amatthews@catabasis.com