Positive Preclinical Research on the Edasalonexent (CAT-1004) Program, a Potential Disease-Modifying Therapy for Duchenne Muscular Dystrophy, Published in JCI Insight

January 4, 2017

-- Preclinical Data Demonstrate Disease Modifying Effects in Two Animal Models of Duchenne Muscular Dystrophy --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 4, 2017-- Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced the publication of preclinical data on the edasalonexent program, a potential disease-modifying therapy for Duchenne muscular dystrophy (DMD). The preclinical data demonstrate that edasalonexent (CAT-1004) and an analog, CAT-1041, oral inhibitors of NF-kB, are effective in ameliorating the dystrophic process in two animal models of DMD in an article titled “Disease Modifying Effects of Orally Bioavailable NF-kB Inhibitors in Dystrophin-Deficient Muscle” in JCI Insight (JCI Insight 2016 Dec 22;1(21):e90341).

This research was led by H. Lee Sweeney, Ph.D., then at the University of Pennsylvania. Edasalonexent (CAT-1004) and CAT-1041, which represent a novel class of NF-kB inhibitors, were evaluated in both *mdx* mouse and golden retriever muscular dystrophy (GRMD) dog models of DMD. Initial studies with edasalonexent and CAT-1041 in *mdx* mice demonstrated nearly identical *in vitro* and *in vivo* efficacy, and CAT-1041 was selected for further evaluation in the treatment of dystrophic muscle. *In vivo*, CAT-1041 effectively improved the phenotype of *mdx* mice undergoing voluntary wheel running, in terms of activity, muscle mass and function, damage, inflammation, fibrosis and cardiac pathology. The researchers identified significant increases in dysferlin as a possible contributor to the protective effect of CAT-1041 against sarcolemmal damage. Furthermore, CAT-1041 improved the more severe GRMD phenotype in a canine case study, where muscle mass and diaphragm function were maintained in a treated GRMD dog.

“There remains a large unmet need in Duchenne for therapies that can treat all affected boys and slow disease progression. The orally bioavailable NF-kB inhibitors, edasalonexent and CAT-1041, improve the severe dystrophic phenotype found in both mechanically-damaged *mdx* mice and a GRMD dog and create an environment that can support more successful muscle regeneration,” said Dr. Sweeney, currently Myology Institute Director, University of Florida. “We believe that these *in vivo* preclinical results support edasalonexent as a candidate for the treatment of DMD.”

“We very much appreciate the research performed by Dr. Sweeney and his colleagues,” said Andrew Nichols, Ph.D., Chief Scientific Officer of Catabasis. “We agree that these data support edasalonexent as a potential treatment to improve both the quantity and quality of muscle fibers in boys affected by DMD and look forward to the Phase 2 clinical trial results with edasalonexent 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/20170104005260/en/

Source: Catabasis Pharmaceuticals, Inc.

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