Catabasis Pharmaceuticals and the Jain Foundation Announce a Preclinical Research Collaboration to Study Edasalonexent in Dysferlinopathy

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-- Edasalonexent Inhibits NF-kB, a Potential Driver of Disease Progression in Dysferlinopathy --

CAMBRIDGE, Mass. & SEATTLE--(BUSINESS WIRE)--Sep. 26, 2019-- Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company, and the Jain Foundation, a non-profit foundation whose mission is to cure muscular dystrophies caused by dysferlin protein deficiency, announced a preclinical research collaboration to study edasalonexent (CAT-1004) in Dysferlinopathy. Dysferlinopathy (Limb-girdle muscular dystrophy type 2B / Miyoshi myopathy) is a serious rare disease that causes progressive muscle weakness for which there are currently no approved treatment options. Edasalonexent is in Phase 3 development for the treatment of Duchenne muscular dystrophy (DMD) and has the potential to benefit patients with other diseases, such as Dysferlinopathy.

In Dysferlinopathy, muscles lack dysferlin and as a result NF-kB is chronically activated. Edasalonexent, an oral small molecule designed to inhibit NF-kB, has the potential to slow disease progression in dysferlin-deficient populations. Under this collaboration, Catabasis and the Jain Foundation are conducting a preclinical study to evaluate the potential of edasalonexent as a therapeutic intervention for Dysferlinopathy by measuring disease progression in dysferlin-deficient mice treated with edasalonexent. The study will utilize magnetic resonance imaging (MRI) and magnetic resonance spectroscopy (MRS) to measure muscle volume, fat accumulation, and other changes in the dysferlin-deficient mice. Initial results are expected in the first half of 2020.

“We look forward to working with Catabasis to advance research for Dysferlinopathy,” said Laura Rufibach, Ph.D., and Doug Albrecht, Ph.D., Co-Presidents of the Jain Foundation. “Patients with Dysferlinopathy (LGMD2B / Miyoshi myopathy) experience a progressive and debilitating decline in muscle function which significantly impacts their lives. As there are currently no treatment options, we are excited to explore the potential of edasalonexent to benefit those living with this disease.”

“The chronic activation of NF-kB is a key driver in many neuromuscular disorders, including Duchenne muscular dystrophy. Evidence of NF-kB activation in Dysferlinopathy suggests a similar disease mechanism and opportunity for intervention. In the MoveDMD trial and open-label extension, treatment with edasalonexent slowed disease progression compared to the off-treatment control period. Through this collaboration, we look forward to learning more about the potential of edasalonexent in Dysferlinopathy, where, similar to Duchenne, there is also a significant unmet need,” said Andrew Nichols, Ph.D., Chief Scientific Officer at Catabasis Pharmaceuticals.

About Edasalonexent

Edasalonexent (CAT-1004) is an investigational oral small molecule designed to inhibit NF-kB that is being developed as a potential foundational therapy for all patients affected by DMD, regardless of their underlying mutation. In DMD the loss of dystrophin leads to chronic activation of NF-kB, which is a key driver of skeletal and cardiac muscle disease progression. We are currently enrolling our global Phase 3 PolarisDMD trial to evaluate the efficacy and safety of edasalonexent for registration purposes. Edasalonexent is also being dosed in the open-label extension trial GalaxyDMD. In our MoveDMD Phase 2 trial and open-label extension, we observed that edasalonexent preserved muscle function and substantially slowed disease progression compared to rates of change in a control period, and significantly improved biomarkers of muscle health and inflammation. 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. For a summary of clinical results, please visit www.catabasis.com.

About Catabasis

At Catabasis Pharmaceuticals, our mission is to bring hope and life-changing therapies to patients and their families. Our lead program is edasalonexent, an NF-kB inhibitor in development for the treatment of Duchenne muscular dystrophy. The global Phase 3 PolarisDMD trial is currently enrolling boys affected by Duchenne. For more information on edasalonexent and our Phase 3 PolarisDMD trial, please visit www.catabasis.com.

About the Jain Foundation

The Jain Foundation is a non-profit foundation whose mission is to cure muscular dystrophies caused by dysferlin protein deficiency, which includes the clinical presentations Limb-girdle muscular dystrophy type 2B (LGMD2B) and Miyoshi muscular dystrophy 1 (MMD1). Collectively these are referred to as Dysferlinopathy. The foundation’s focused strategy includes financing and actively directing the progress of projects in key pathways towards a cure. These include identification and assessment of promising drug candidates, the development and maintenance of a global patient registry (the Dysferlin Registry), as well as a natural history study of patients with Dysferlinopathy to better understand disease progression and identify the best outcome measures for use in clinical trials. For more information about the Jain Foundation, the Dysferlin Registry, and the projects we support, please visit www.jain-foundation.org.

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 including, among other things, statements about the Company’s global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, including the anticipated timing for top-line results, potential timing for the filing of an NDA, 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; whether interim results from a preclinical or 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 year ended June 30, 2019, 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.

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Source: Catabasis Pharmaceuticals, Inc.

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

Jain Foundation Contacts
Doug Albrecht or Laura Rufibach
Jain Foundation Inc.
T: (425) 882-1492
admin@jain-foundation.org