Catabasis Pharmaceuticals Presents New Edasalonexent Clinical Biomarker Data Showing NF-kB Inhibition and Target Engagement in the MoveDMD® Trial

CAMBRIDGE, MA, June 25, 2018 – Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported new NF-kB inhibition biomarker results showing edasalonexent target engagement in the MoveDMD Phase 2 trial and open-label extension in boys affected by Duchenne muscular dystrophy (DMD). NF-kB is a fundamental driver of disease progression in DMD. These results add further support to the significant NF-kB biomarker results observed in Phase 1 of the MoveDMD trial and are consistent with significantly decreased C-reactive protein (CRP) with edasalonexent treatment. These data were presented today at the New Directions in Biology and Disease of Skeletal Muscle Conference in New Orleans, LA.

The NF-kB pathway is the key link between loss of dystrophin and disease manifestation and progression in DMD. Lack of dystrophin combined with mechanical stress activates NF-kB, which promotes inflammation and fibrosis, suppresses muscle regeneration and drives muscle degeneration. Edasalonexent is an oral small molecule that inhibits NF-kB and improves skeletal, diaphragm and cardiac disease in mouse and dog models of DMD.

During the off-treatment control period when boys were not receiving edasalonexent, transcripts of NF-kB-regulated genes increased in whole blood, consistent with systemic inflammation in DMD. Gene expression analysis was performed following 12 and 24 weeks of 100 mg/kg of edasalonexent treatment and transcripts of NF-kB-regulated genes significantly decreased by 2-fold ($p<0.005$), showing decreased NF-kB signaling in boys receiving edasalonexent and consistent with edasalonexent reducing inflammation.

“We are pleased to see additional clinical evidence demonstrating the activity of edasalonexent in inhibiting NF-kB in boys affected by Duchenne, consistent with the observed substantial slowing of Duchenne disease progression in the assessments of muscle function and magnetic resonance,” said Andrew Nichols, Ph.D., Chief Scientific Officer of Catabasis. “Building on the results previously reported for edasalonexent treatment in patients in the MoveDMD trial, these new biomarker data further support the consistent positive edasalonexent results. We look forward to advancing edasalonexent in a single global Phase 3 trial this year with the goal of improving the quality and length of life for those affected by Duchenne.”

The decrease in NF-kB-regulated transcripts with edasalonexent treatment is consistent with biomarker results that showed CRP was significantly decreased with edasalonexent at 12, 24, 36 and 48 weeks compared to baseline in the 100 mg/kg treatment group ($p\leq0.001$). CRP is a well-characterized blood marker that provides a global assessment of inflammation, and CRP is elevated in boys affected by DMD. The significant decrease observed in CRP supports the biological activity of NF-kB inhibition by edasalonexent treatment decreasing inflammation.

In the Phase 2 and open-label extension of the MoveDMD trial, edasalonexent substantially slowed DMD disease progression in boys on 100 mg/kg through more than a year of treatment compared to the rate of change in the off-treatment control period. Across all assessments of muscle function, consistent improvements were observed in the rate of decline after 12, 24, 36, 48 and 60 weeks of oral 100 mg/kg edasalonexent treatment. Edasalonexent was well tolerated with no safety signals observed in the trial. The MoveDMD trial investigated the safety and efficacy

of edasalonexent and enrolled boys not on steroids ages 4 to 7 affected with DMD regardless of mutation type.

About Edasalonexent (CAT-1004)
Edasalonexent (CAT-1004) is an investigational oral small molecule that is being developed as a potential disease-modifying therapy for all patients affected by DMD, regardless of their underlying mutation. Edasalonexent inhibits NF-kB, a protein that is activated in DMD and drives inflammation, fibrosis and muscle degeneration and suppresses muscle regeneration. Edasalonexent continues to be dosed in an open-label extension of the MoveDMD Phase 2 clinical trial, and Catabasis is preparing to initiate a single global Phase 3 trial in the second half of 2018 to evaluate the efficacy and safety of edasalonexent for registration purposes. 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 reported to-date, 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. Edasalonexent was designed using our SMART (Safely Metabolized And Rationally Targeted) Linker drug discovery platform that enables us to engineer molecules that simultaneously modulate multiple targets in a disease. For more information on edasalonexent or our drug discovery platform, 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 including, among other things, statements about the Company's plans to commence a single global Phase 3 trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, 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 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; the Company's ability to obtain financing on acceptable terms and in a timely manner to fund the Company's planned Phase 3 trial of edasalonexent in DMD for registration purposes; 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 quarter ended March 31, 2018, 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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