



Catabasis Pharmaceuticals Presents Positive Data from Part A of the MoveDMD[®] Trial of Edasalonexent (CAT-1004), a Potential Disease-Modifying Therapy for DMD, at the World Muscle Society Congress

CAMBRIDGE, MA, October 6, 2016 – [Catabasis Pharmaceuticals, Inc.](http://www.catabasis.com) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced that Catabasis is presenting positive data from Part A of the MoveDMD trial of edasalonexent for the treatment of Duchenne muscular dystrophy (DMD) at the World Muscle Society Congress. The World Muscle Society Congress is being held October 4 – October 8, 2016, in Granada, Spain, at the Palacio de Congresos de Granada.

Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis, will present the poster “CAT-1004, an oral agent targeting NF-κB: MoveDMD trial results in Duchenne muscular dystrophy (DMD)” and Pradeep Bista, Ph.D., Principal Scientist at Catabasis, will present the poster “Serum pro-inflammatory proteins have potential utility as biomarkers for NF-κB targeting approaches in DMD.” Both poster presentations will take place in Poster Presentations 3: Parallel Sessions today from 3:00pm – 4:30pm local time in the Poster area of the Palacio de Congresos de Granada.

Catabasis has previously announced positive biomarker results from Part A of the MoveDMD clinical trial demonstrating successful NF-κB target engagement and detailed data are being presented at the World Muscle Society Congress. After one week of dosing with edasalonexent in 4-7-year-old boys affected by DMD, a pre-selected NF-κB gene set was significantly inhibited in whole blood mRNA at each of the two higher doses (67 mg/kg and 100 mg/kg). The evaluated gene set is 200 expressed genes known to be regulated by NF-κB that was curated by the Broad Institute. This gene set was significantly decreased when compared to all other expressed genes. In addition to gene expression, serum proteins were also evaluated. In the combined group of patients on 67 mg/kg and 100 mg/kg, levels of NF-κB-regulated proteins, IL-12 and osteopontin, were significantly reduced following 7 days of dosing with edasalonexent. These data further support the ongoing evaluation of the 67 and 100 mg/kg edasalonexent doses in the Phase 2 MoveDMD clinical trial.

Baseline data for boys from Part A of the trial who are also participating in Part B are being presented at the World Muscle Society Congress as well. Timed function tests were assessed before the boys had started dosing with edasalonexent in Part A of the trial as well as at baseline before they started dosing with edasalonexent in Part B of the trial. For all three of the timed function tests that we are evaluating, 10-meter walk/run, 4-step climb and time-to-stand, there was an increase in the average time to complete the tests from Part A baseline to Part B baseline. The time elapsed between Part A baseline and Part B baseline ranged from 4 to 11 months for these 15 boys. Although the MoveDMD trial is not powered to show statistically significant changes in these three timed function tests in Part B, the observations provide data on disease progression within the MoveDMD trial that shows a decline in function when these boys are predominantly not on treatment and may provide additional context for assessing these secondary end points during the on-treatment part of the trial. The primary end point of Part B of the MoveDMD trial is change in MRI T2 signal of the lower leg muscles for edasalonexent as compared with placebo.

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- κ B, 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- κ B 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- κ B target engagement. Pharmacokinetic results demonstrated edasalonexent plasma exposure levels consistent with those previously observed in adults, at which inhibition of NF- κ B was observed.

About MoveDMD[®]

The MoveDMD trial is a three-part clinical trial investigating the safety and efficacy of edasalonexent in boys ages 4 – 7 affected with DMD (any confirmed mutation). Part A of the MoveDMD trial evaluated the safety, tolerability and pharmacokinetics of, and NF- κ B target engagement with, edasalonexent and showed positive results. Sixteen of the 17 boys enrolled in Part A continued to Part B of the trial, which is a Phase 2 trial to evaluate the safety and efficacy of edasalonexent in DMD over a 12-week period in approximately 30 boys. The primary end point is change in MRI of the lower leg muscles, and the secondary end points are age-appropriate timed function tests: 10-meter walk/run, 4-stair climb and time to stand. Additional assessments include muscle strength, the North Star Ambulatory Assessment and the pediatric outcomes data collection instrument (PODCI). Part C is an open-label extension that includes dosing with edasalonexent for 36 weeks beyond the 12-week placebo-controlled portion of the trial (Part B) and will evaluate longer term safety and efficacy with the same clinical end points as Part B.

About MRI

Magnetic resonance imaging (MRI) is a non-invasive imaging technique that can assess 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 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 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 June 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.

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