



## **Catabasis Pharmaceuticals Provides Update on Global Phase 3 PolarisDMD Trial for Edasalonexent at Parent Project Muscular Dystrophy 25<sup>th</sup> Annual Conference**

*-- Patient Identification Nearing Completion for Global Phase 3 PolarisDMD Trial for Edasalonexent in Duchenne Muscular Dystrophy --*

**CAMBRIDGE, MA, June 27, 2019** – [Catabasis Pharmaceuticals, Inc.](https://www.catabasis.com) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, shared an update on the clinical development of edasalonexent, a novel NF- $\kappa$ B inhibitor for the treatment of Duchenne muscular dystrophy (DMD) this week at the Parent Project Muscular Dystrophy (PPMD) 25<sup>th</sup> Annual Conference.

Catabasis shared progress in the edasalonexent Phase 3 PolarisDMD trial:

- Screening is ongoing for the randomized, double-blind, placebo-controlled trial in the United States, Canada, Australia, the United Kingdom, Ireland, Sweden, Germany and Israel and making strong progress.
- Clinical trial sites globally are enrolling quickly and sites in the United Kingdom, Ireland, Sweden, Germany and Israel are at capacity and no longer accepting additional patients.
- The trial is active in all planned countries with 37 sites open for enrollment.
- Top-line results from the study are expected in the second half of 2020 and are anticipated to support an NDA filing in early 2021.

“We are very pleased with the progress made in our Phase 3 PolarisDMD trial for edasalonexent in Duchenne. There has been significant interest from families and enthusiasm from investigators globally, and enrollment is going very well with some countries already at capacity,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “We appreciate the hard work of our study sites and the commitment of participating families as we look to develop a new treatment to benefit all boys affected by Duchenne.”

“There is clear need for a therapy that could benefit all boys affected by Duchenne, regardless of mutation type, by slowing disease progression while being well tolerated. We are glad to be participating in the Phase 3 PolarisDMD trial for edasalonexent and are pleased that Kennedy Krieger is a top enrolling site,” said Kathryn Wagner, M.D., Ph.D., Director of the Center for Genetic Muscle Disorders at the Kennedy Krieger Institute, a Professor of Neurology and Neuroscience at the Johns Hopkins School of Medicine and a Principal Investigator in the Phase 3 PolarisDMD trial for edasalonexent. “We have found the PolarisDMD trial to be thoughtfully designed to be family friendly as well as straightforward for our clinical team.”

Catabasis also shared insights from a recent blinded qualitative research project that collected information from in-depth interviews with approximately 30 physicians treating boys affected by DMD, caregivers and patient advocacy representatives in the United States that was sponsored by Catabasis. A key observation from the research was that members of the DMD community

prioritized treatments that will go beyond skeletal muscle and positively impact additional important aspects of DMD, including cardiac and pulmonary effects. Participants also shared their hope that treatments will provide durable benefits and improve quality of life. Additionally, physicians predicted that the majority of boys will receive combination therapy for the treatment of DMD within the next few years.

Data from the edasalonexent MoveDMD Phase 2 open-label extension study were also presented at the PPMD conference. Through 72 weeks of treatment compared to an off-treatment control period, patients treated with edasalonexent demonstrated preserved muscle function and substantially slowed DMD disease progression across all four assessments of muscle function (the North Star Ambulatory Assessment, time to stand, 4-stair climb and 10-meter walk/run). Preclinical data and clinical biomarker data from the MoveDMD trial suggest that edasalonexent could have potential benefits in skeletal muscle, diaphragm and heart. Edasalonexent has been well tolerated through more than 55 patient-years of treatment.

In the Phase 3 PolarisDMD trial, Catabasis plans to enroll approximately 125 patients ages 4 to 7 (up to 8<sup>th</sup> birthday) regardless of mutation type who have not been on steroids for at least 6 months. Boys on a stable dose of eteplirsen may be eligible to enroll. Boys from the MoveDMD open-label extension and their eligible siblings can now enroll in the GalaxyDMD open-label extension trial. Boys who complete the Phase 3 PolarisDMD trial as well as their eligible siblings will also have the opportunity to participate in the GalaxyDMD open-label extension trial. Boys can begin or continue treatment with an approved exon skipping therapy in the GalaxyDMD trial. The GalaxyDMD trial has a streamlined schedule with visits to trial sites every six months and is designed to collect long-term safety data to support registration filings. The trial is also monitoring assessment of muscle function and bone health.

### **About Edasalonexent (CAT-1004)**

Edasalonexent (CAT-1004) is an investigational oral small molecule that is being developed as a potential therapy for all patients affected by DMD, regardless of their underlying mutation. Edasalonexent inhibits NF- $\kappa$ B, which is a key link between loss of dystrophin and disease progression in DMD. NF- $\kappa$ B has a fundamental role in skeletal and cardiac muscle disease in DMD. 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](http://www.catabasis.com).

### **About Phase 3 PolarisDMD Trial**

The global Phase 3 PolarisDMD trial is a one-year, randomized, double-blind, placebo-controlled trial evaluating the efficacy and safety of edasalonexent in patients with DMD. Catabasis plans to

enroll approximately 125 patients ages 4 to 7 (up to 8<sup>th</sup> birthday) regardless of mutation type who have not been on steroids for at least 6 months. Boys on a stable dose of eteplirsen may be eligible to enroll. The primary efficacy endpoint is change in the North Star Ambulatory Assessment score after 12 months of treatment with edasalonexent compared to placebo. Key secondary endpoints include the age-appropriate timed function tests: time to stand, 4-stair climb and 10-meter walk/run. Assessments of growth, cardiac and bone health are also included as important potential areas of differentiation. Two boys are receiving 100 mg/kg/day of edasalonexent for each boy that receives placebo, and, after 12 months, all boys are expected to receive edasalonexent in the open-label extension study GalaxyDMD. The PolarisDMD trial design was informed by discussions with regulators as well as input from treating physicians, patient organizations and families of boys affected by Duchenne. Top-line results from the Phase 3 PolarisDMD trial are expected in the second half of 2020. More information about the Phase 3 PolarisDMD clinical trial is available on [clinicaltrials.gov](https://clinicaltrials.gov) and contact the team with any questions at [DMDtrials@catabasis.com](mailto:DMDtrials@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-κB inhibitor in development for the treatment of Duchenne muscular dystrophy. Our 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](http://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 global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, including the anticipated timing for completion of enrollment and 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 March 31, 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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