



## **Catabasis Pharmaceuticals Phase 3 PolarisDMD Clinical Trial for Edasalonexent in Duchenne Muscular Dystrophy Progress Update and Additional Trial Sites Open for Enrollment**

*-- Patient Enrollment Underway in U.S. with Nine Locations Open for Enrollment --*

*-- Global Start-Up Activities Ongoing with Clinical Trial Application Approvals Received in Canada and Multiple European Countries --*



**CAMBRIDGE, Mass., January 3, 2019** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today shared an update on clinical trial sites in the United States and recent international clinical trial application approvals to perform the Phase 3 PolarisDMD trial for edasalonexent (CAT-1004) in Duchenne muscular dystrophy (DMD). PolarisDMD clinical trial sites in the United States are actively enrolling patients. The PolarisDMD trial is evaluating the efficacy and safety of edasalonexent in patients with DMD and is intended to support an application for commercial registration of edasalonexent. Top-line results from the Phase 3 PolarisDMD trial are expected in the second quarter of 2020.

A total of nine PolarisDMD clinical trial sites are now open for enrollment across the United States and at least ten additional sites are expected to open shortly. Additionally, Catabasis has received approval of clinical trial applications to conduct the PolarisDMD trial in Canada and multiple countries in Europe. Clinical trial sites are expected to open in Canada this month and sites in Europe as well as Australia and Israel, pending regulatory approval, are expected to open in first quarter of this year. In total, the PolarisDMD trial is expected to include approximately 40 clinical trial sites globally with enrollment expected to be completed in 2019.

“We are very excited to be screening and dosing patients in our Phase 3 PolarisDMD trial as we believe edasalonexent has tremendous potential for all those affected by Duchenne, regardless of mutation type and from the time of diagnosis throughout their lifespan. We have received extensive inbound interest from families interested in the study and we are actively referring these families to the enrolling clinical trial sites,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “Edasalonexent inhibits NF-kB, which plays a fundamental role in skeletal and cardiac muscle disease in Duchenne. We are monitoring bone and heart health in the

PolarisDMD trial, in addition to the assessments of skeletal muscle function, as we see these as important potential differentiating benefits of edasalonexent.”

“There is a great need for new treatment options for Duchenne and we are very pleased that the PolarisDMD trial for edasalonexent will enroll patients at sites in Canada,” said Perry Esler, Executive Director of Jesse’s Journey, the only charity in Canada solely focused on funding research into Duchenne. “We are very supportive of treatment options, like edasalonexent, that have the potential to benefit all boys and men affected by Duchenne, and we know that these trials are a high priority for families.”

The global Phase 3 PolarisDMD trial is a one-year, randomized, double-blind, placebo-controlled 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. 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 will receive 100 mg/kg/day of edasalonexent for each boy that receives placebo, and, after 12 months, all boys are expected to receive edasalonexent in an open-label extension study. 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.

The Phase 3 PolarisDMD trial is designed to further validate the positive efficacy seen in the MoveDMD Phase 2 trial and open-label extension evaluating edasalonexent. In the MoveDMD trial, edasalonexent has been shown to preserve muscle function and substantially slow 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) through 72 weeks of treatment compared to an off-treatment control period. Preclinical data and clinical biomarker data from the MoveDMD Phase 2 trial suggest that edasalonexent could have potential benefits in skeletal muscle, diaphragm and heart. Edasalonexent has been well tolerated through more than 50 patient-years of treatment with no safety signals observed.

More information about the Phase 3 PolarisDMD clinical trial is available on [clinicaltrials.gov](https://clinicaltrials.gov) and in a recently recorded [webinar](#) with PPMD. Contact the Catabasis clinical team with any questions at [DMDtrials@catabasis.com](mailto:DMDtrials@catabasis.com).

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

Edasalonexent (CAT-1004) is an investigational oral small molecule that is being developed as a potential new standard of care for all patients affected by DMD, regardless of their underlying mutation. Edasalonexent inhibits NF-κB, which is a key link between loss of dystrophin and disease progression in DMD. NF-κB 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. 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. Edasalonexent continues to be dosed in the open-label extension of the MoveDMD trial. 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 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- $\kappa$ B 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) or [www.twitter.com/catabasispharma](https://www.twitter.com/catabasispharma).

### **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, 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; 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 September 30, 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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