



## **Catabasis Pharmaceuticals Presents Edasalonexent, a Potential Foundational Therapy for Duchenne Muscular Dystrophy**

**BOSTON, MA, May 12, 2020** – [Catabasis Pharmaceuticals, Inc.](https://www.catabasis.com) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today shared information on the edasalonexent program in Phase 3 development for the treatment of Duchenne muscular dystrophy (DMD) in poster presentations at the 2020 Muscular Dystrophy Association (MDA) Virtual Poster Session. The three posters include an analysis of the baseline characteristics of patients enrolled in the Phase 3 PolarisDMD trial, age-normative growth and normal adrenal function seen in the Phase 2 MoveDMD trial and open-label extension, and experience with capsule swallowing in both clinical trials for edasalonexent in boys with DMD. Top-line results from the Phase 3 trial are expected in Q4 2020.

“As we advance our Phase 3 PolarisDMD trial, we gain valuable insights into the full potential of edasalonexent,” said Joanne Donovan, M.D., Ph.D., Chief Medical Officer of Catabasis. “We are pleased to share additional information supporting the design of the Phase 3 PolarisDMD trial and showing long-term safety of edasalonexent. In addition to being well-tolerated in over 100 cumulative years of patient exposure, edasalonexent has shown potential to both preserve muscle function as well as positive cardiac effects. Those living with Duchenne have limited treatment options, and our goal is for edasalonexent to be a new foundational therapy.”

An analysis of the baseline characteristics of the patients enrolled in the Phase 3 PolarisDMD trial was performed compared to the patients enrolled in the previous Phase 2 MoveDMD trial and found overall similar baseline characteristics in the patient populations in the two trials. Both the Phase 3 PolarisDMD trial and the Phase 2 MoveDMD trial enrolled boys affected by DMD ages 4 to 7 (up to 8<sup>th</sup> birthday) with any mutation type who had not been on steroids for the previous 6 months. There were no significant differences between the two trials in baseline age, North Star Ambulatory Assessment (NSAA) score and timed function test values (time to stand, 4-stair climb, and 10-meter walk/run). These findings are believed to support the assumptions on which the Phase 3 trial was powered.

Catabasis also shared long-term safety and tolerability data from the MoveDMD trial and open-label extension. Edasalonexent was associated with age-normative growth, without negative impact on bone health, and normal adrenal function in boys with DMD as it does not impact the glucocorticoid receptor. There was no evidence of adrenal insufficiency for up to 150 weeks of edasalonexent treatment, and no clinically significant changes in cortisol or ACTH. Additionally, edasalonexent treatment in a *mdx* mouse model of DMD preserved bone length and bone density. In more than 100 years of cumulative patient exposure, edasalonexent has been well-tolerated, with no serious adverse events on treatment, and without the adverse effects associated with high-dose steroids. In both the Phase 2 MoveDMD trial and the ongoing Phase 3 PolarisDMD trial, 97% of 4 to 7 year-old boys with DMD screened for the studies were able to swallow soft-gel

study drug capsules. There have been no discontinuations due to capsule burden. This demonstrates that edasalonexent has the potential to be broadly adopted by those affected by DMD and supports the long-term safety and tolerability of edasalonexent.

Posters from the MDA Virtual Poster Session are available under “Our Science” at [www.catabasis.com](http://www.catabasis.com).

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

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. Our ongoing global Phase 3 PolarisDMD trial is evaluating the efficacy and safety of edasalonexent for registration purposes. Edasalonexent is also being dosed in the GalaxyDMD open-label extension trial. 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 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 Phase 3 development for the treatment of Duchenne muscular dystrophy. 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, the Company’s global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, anticipated timing for top-line results 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 Annual Report on Form 10-K for the year ended December 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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