



## **Catabasis Pharmaceuticals Presents Preclinical Data Showing Potential for Bone Preservation with Edasalonexent in Duchenne Muscular Dystrophy**

**CAMBRIDGE, MA, June 21, 2019** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today presented new preclinical data showing preserved bone health with edasalonexent in contrast to negative effects of the corticosteroid prednisolone in a mouse model of Duchenne muscular dystrophy (DMD). Edasalonexent is a novel NF- $\kappa$ B inhibitor in Phase 3 development for the treatment of DMD. The data were presented at the Symposium on Muscle-Bone Interaction in Duchenne Muscular Dystrophy.

Bone health is important to those affected by Duchenne as many patients experience long bone and/or vertebral fractures before the age of 13. In the preclinical study sponsored by Catabasis, prednisolone treatment negatively impacted bone health in *mdx* mice (mouse model of DMD) compared to control, whereas edasalonexent treatment showed bone sparing effects compared to the bone loss seen with prednisolone. Mice treated with prednisolone had significantly weaker bones (both cortical density and cortical thickness) and also grew less as assessed by femur length compared to control *mdx* mice. These results were seen following 6 months of treatment of clinically relevant doses of prednisolone or edasalonexent. The mice receiving 6 months of edasalonexent had preserved cortical density, cortical thickness and femur length, similar to the control mice.

Corticosteroids, such as prednisolone, can negatively impact bone health by increasing osteoclast apoptosis, reducing ossification and leading to increased bone resorption and osteoporosis. Catabasis believes that the treatment of DMD by inhibiting NF- $\kappa$ B with edasalonexent has the potential to reduce bone loss and enhance new bone growth in those affected by DMD by increasing osteoblast maturation and decreasing osteoclast differentiation and function.

Boys with DMD typically have decreased height compared to boys that do not have DMD and the adverse effects of corticosteroids include decreased vertical growth. In the Phase 2 MoveDMD<sup>®</sup> trial and open-label extension, over a 72-week period, boys receiving edasalonexent grew taller by an average of 2.1 inches per year, increases that were consistent with the growth curves of unaffected boys. The effects of edasalonexent on bone health are being further studied in DMD in the ongoing global Phase 3 PolarisDMD trial, which includes dual-energy X-ray absorptiometry (DXA) to assess bone density and body composition and standardized lumbosacral spine films to assess for fractures at baseline and following 1 year of treatment.

The 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. Top-line results from the Phase 3 PolarisDMD trial are expected in the second half of 2020.

#### **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-kB, which is a key link between loss of dystrophin and disease progression in DMD. NF-kB 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 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. 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, the effect of edasalonexent on the preservation of bone in those affected by Duchenne muscular dystrophy, 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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