Issue 10 | February 2018

catabasis QUARTERLY

Updates on edasalonexent and clinical trials

Catabasis is pleased to share new data: Edasalonexent preserved muscle function in DMD through more than 1 year of treatment in the MoveDMD trial

100 mg/kg/day oral edasalonexent substantially slowed Duchenne muscular dystrophy disease progression through 60 weeks of treatment. Across all assessments of muscle function, consistent improvements were observed in the trajectory of disease progression in boys on edasalonexent treatment compared to the rate of change in the control period when boys were not receiving edasalonexent. Assessments of muscle function:

- **North Star Ambulatory Assessment**
- **10-meter walk/run**
- **4-stair climb**
- **Time to stand**

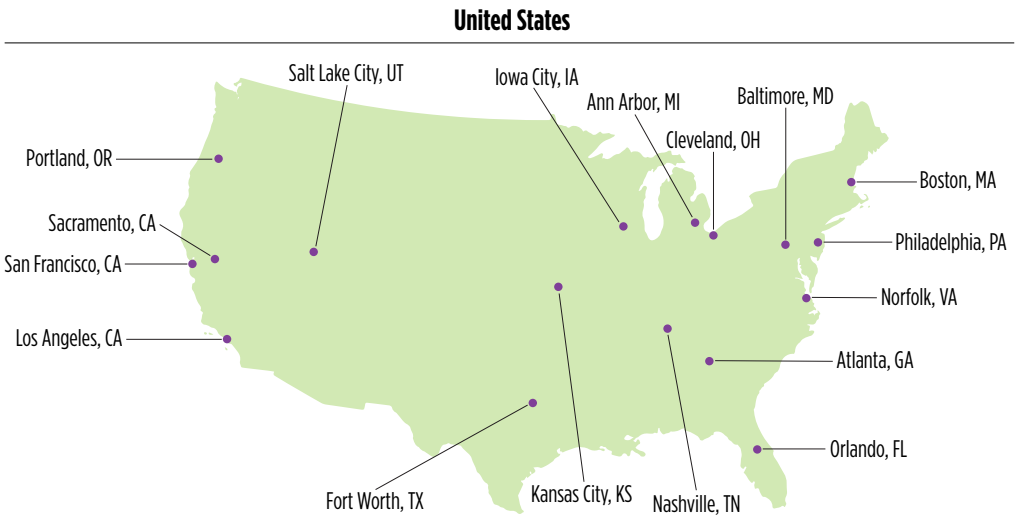
Additional measures of muscle health were consistent with positive edasalonexent treatment effects including significantly decreased muscle enzymes, suggesting a decrease in muscle injury; significantly decreased C-reactive protein, a global assessment of inflammation; and significantly improved rate of change in lower leg muscle MRI T2, suggesting a decrease in inflammation. Edasalonexent continues to be well tolerated with no safety signals observed. To see complete results to date, visit www.catabasis.com.

PREPARING FOR PHASE 3 CLINICAL TRIAL WITH EDASALONEXENT IN DMD

Trial start-up activities are underway to conduct a single global Phase 3 trial with edasalonexent in 4- to 7-year-old boys with DMD regardless of mutation type and who have not been on steroids for at least 6 months. This trial, based on the consistency of the MoveDMD results, will enroll approximately 125 patients and be placebo-controlled with 2 boys receiving edasalonexent for every 1 boy receiving placebo. After 12 months in the trial, all boys are expected to receive edasalonexent in an open-label extension. We are excited and anticipate enrolling the first patients in the first half of 2018.

- **Key enrollment criteria**
  - Age 4 to 7th birthday
  - Able to complete timed function tests
  - Not on corticosteroids for at least 6 months
  - Not on other investigational therapies for at least 1 month
  - Can be on stable eteplirsen
- **Key assessments at trial visits every 3 months**
  - North Star Ambulatory Assessment, time to stand, 4-stair climb, 10-meter walk/run, muscle strength
  - Safety measures
  - Assessments of growth, cardiac and bone health
- **Trial sites are planned for the US, Canada, Europe and Australia**
  - Trial sites will be listed o www.clinicaltrials.gov once ready to enroll patients
  - Travel support will be provided

EXPECTED GLOBAL PHASE 3 CLINICAL TRIAL SITES



International

Canada
London, Ontario
Hamilton, Ontario
Montreal, Quebec
Calgary, Alberta

Ireland
Dublin

United Kingdom
Bristol
Manchester
London

Sweden
Gothenburg

Germany
Munich
Hamburg

Israel
Jerusalem

Australia
Melbourne
Sydney

MAKING COMMUNITY CONNECTIONS

At the Jett Foundation Regional Roundtable Series—January 20 in Hartford, CT and coming up on February 24 in Philadelphia, PA. Partnering with Duchenne organizations, clinicians, institutions, industry, and other experts, these forums examine issues and curate information around treatment options, recently completed trials, and those underway for families affected by DMD.

At the PPMD Every Single [One] Tour—February 3 in Morristown, NJ. In an effort to reach every single family facing a Duchenne diagnosis in the U.S., PPMD launched this multi-year community experience bringing updates on research, advocacy, and care to cities nationwide.

At the upcoming 16th Annual International Conference on Duchenne and Becker Muscular Dystrophy—February 17 and 18 in Rome, Italy, an event where 600+ people in the international community gather to learn and share experiences and information. Attendees include patients, family members, doctors, researchers and the companies dedicated to therapeutic advancements.

Catabasis Supports Rare Disease Day 2018 on February 28th

RARE DISEASE DAY®

Trial start-up activities are underway for the Phase 3 trial studying edasalonexent in DMD and we anticipate beginning enrollment in the first half of 2018. If you have questions about edasalonexent or the global Phase 3 trial, contact our clinical team at DMDtrials@catabasis.com.
For more information on the MoveDMD trial go to https://clinicaltrials.gov/ct2/show/NCT02439216.
And stay updated on edasalonexent developments by joining our mailing list: http://www.catabasis.com/patients-families/for-further-information.php. You can also download this newsletter from our website at www.catabasis.com.

The information provided here is for parents and caregivers of boys with Duchenne muscular dystrophy (DMD). Edasalonexent is an investigational drug that has not been approved by the US Food and Drug Administration.

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Move DMD