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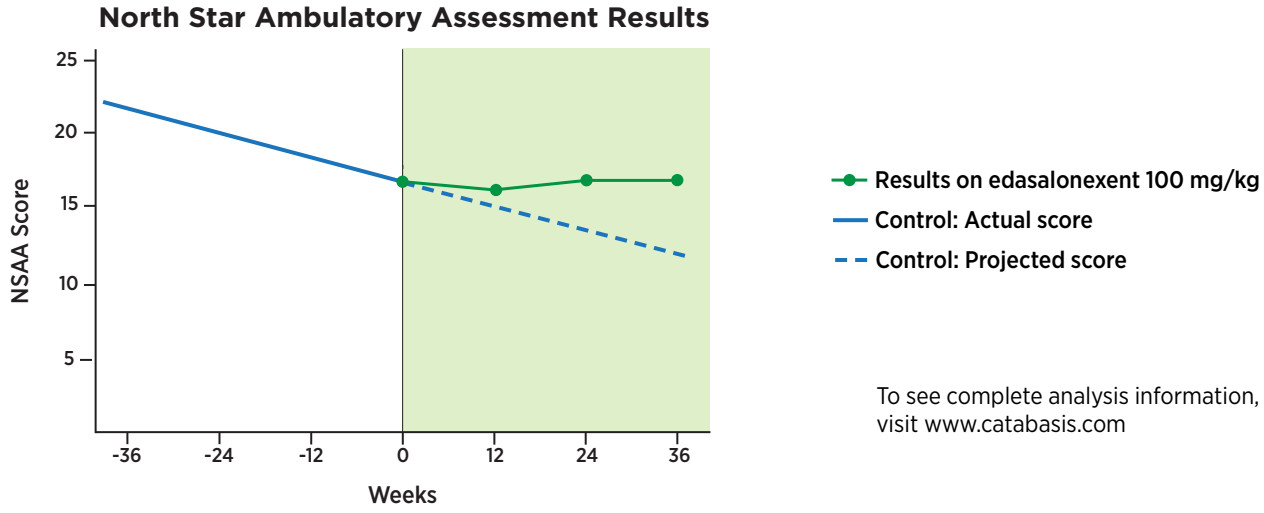
catabasis QUARTERLY

Updates on edasalonexent and the MoveDMD® trial

Catabasis is pleased to share positive results from open-label extension of MoveDMD trial with edasalonexent

New positive efficacy results were reported following 24 and 36 weeks of treatment with 100 mg/kg/day oral edasalonexent in the MoveDMD Phase 2 trial open-label extension.

Edasalonexent substantially slowed Duchenne muscular dystrophy disease progression through 36 weeks of treatment



To see complete analysis information, visit www.catabasis.com

- Improvements were seen across multiple assessments of muscle function compared to the rate of change in the control period when boys were not receiving edasalonexent, including:
  - —North Star Ambulatory Assessment
  - —10-meter walk/run
  - —4-stair climb
  - —Time to stand
- Additional measures of muscle health were seen, all consistent with positive edasalonexent treatment effects
  - —Muscle enzymes significantly decreased at 12 weeks and later time points, suggesting a decrease in muscle injury
  - —Rate of change in lower leg muscle MRI T2 was significantly improved in comparison to progression when not receiving edasalonexent, suggesting a decrease in inflammation
- Edasalonexent continues to be well tolerated with no safety signals observed in the trial
  - —Height, weight and BMI continued along age-appropriate percentile curves

"We are excited to see positive effects on muscle function with edasalonexent, as we know from research that effects on muscle function are the most important aspect of a therapy for Duchenne for the affected patients and their families. We look forward to learning more about edasalonexent as Catabasis begins its Phase 3 trial. With the disease-modifying effects and safety and tolerability profile observed to date for edasalonexent, it has the potential to be a foundational therapy for all people affected by Duchenne."

Pat Furlong, Founding President and CEO of Parent Project Muscular Dystrophy (PPMD)

PHASE 3 CLINICAL TRIAL WITH EDASALONEXENT IN DMD PLANNED TO START IN FIRST HALF OF 2018

Based on the consistency of the MoveDMD results and supportive regulatory input from FDA, we plan to start 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. The trial is anticipated to be placebo-controlled, with 2 boys receiving edasalonexent for every 1 boy receiving placebo. The **primary endpoint for the trial is anticipated to be North Star Ambulatory Assessment after 12 months of treatment with edasalonexent**. After 12 months in the study, all boys are expected to receive edasalonexent in an open-label extension. Clinical trial sites are being identified in North America and additional locations are being determined.

MAKING COMMUNITY CONNECTIONS

Coming up: webinar with PPMD October 11 at 1 pm EST on the open-label extension results in the MoveDMD trial

At the Jett Foundation Regional Roundtable Series—September 9 in Boston, MA. 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 upcoming PPMD Every Single [One] Tour—October 14 in Boston, MA. 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 across the country.

At the upcoming 15th Action Duchenne International Conference—November 10-12 in Birmingham, U.K. Here, at the preeminent Duchenne & Becker muscular dystrophy event in the UK, families and supporters meet and learn about the condition from international experts and from other families and those living with Duchenne. For academics and clinicians, this conference provides the opportunity to discuss the latest findings in genetic research and to share expertise in optimal standards of care.

The placebo-controlled portion of Phase 2 of the MoveDMD trial with edasalonexent in DMD is complete. The open-label extension is ongoing and participating boys are expected to transition to a higher dose of 133 mg/kg/day, pending IRB approval.

We plan for the Phase 3 trial studying edasalonexent in DMD to start in the first half of 2018. If you have questions about edasalonexent or interest in 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. The content is intended for a US audience only.

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Move DMD