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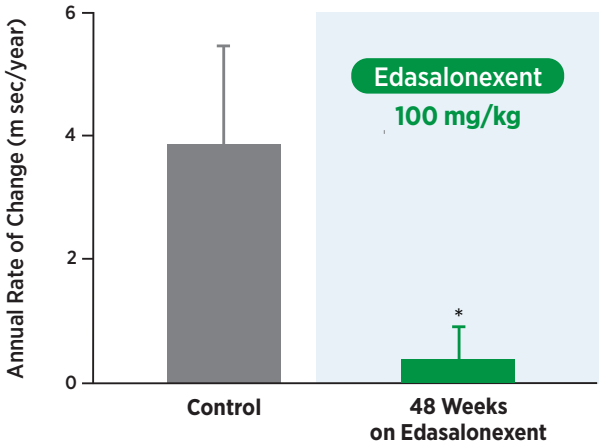
catabasis QUARTERLY

Updates on edasalonexent and clinical trials

Additional results from the MoveDMD trial with edasalonexent: MRI-measured preservation of muscle and age-appropriate growth through more than 1 year of treatment

MRI is a way to assess the muscles of boys with Duchenne and measures inflammation and fat infiltration. Two different types of MRI were used in the MoveDMD trial, MRI T2 and MRS fat fraction. Edasalonexent statistically significantly improved the rate of change of MRI T2 compared with the off-treatment control period ($p<0.05$ for 12, 24, 36 and 48 weeks of treatment). Decrease in progression of MRI T2 is consistent with slowing of disease progression observed in functional assessments. MRS fat fraction also showed less of an increase in boys on edasalonexent than during the off-treatment control period. **Assessments of muscle function and MRI results all consistently showed less disease progression on edasalonexent compared to the off-treatment control period**. As a reminder, the assessments of muscle function were the North Star Ambulatory Assessment and timed function tests (4-stair climb, 10-meter walk/run and time to stand).

MRI T2: Composite of 5 Lower Leg Muscles



Means ± SEM shown; * $p<0.05$ for mixed model comparison with off-treatment period

Height and weight growth through 60 weeks of edasalonexent treatment was **age-appropriate and on track with standard growth curves for unaffected boys in the same age range**. BMI trended towards a decrease. This profile is favorably differentiated from the typical profile associated with the corticosteroid standard of care in DMD, which includes weight gain and curtailed growth. To see complete results to date, visit www.catabasis.com.

Positive MoveDMD data support the planned Phase 3 trial for edasalonexent

We are preparing for a Phase 3 trial that will enroll approximately 125 boys with DMD between the ages of 4-7 years old regardless of mutation type and who have not been on steroids for at least 6 months. It is planned to be a single, global, placebo-controlled Phase 3 trial 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.

MAKING COMMUNITY CONNECTIONS

At the PPMD Every Single [One] Tour—March 24 in Grand Rapids, MI and coming up April 28 in St. Paul, MN. 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 Jett Foundation Regional Roundtable Series—April 21 in Charlotte, NC. 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 American Academy of Neurology 70th Annual Meeting—April 21-27 in Los Angeles, CA. The 2018 annual meeting is the world's largest gathering of neurologists and neuroscience professionals. Richard Finkel, M.D., Chief, Division of Neurology, Department of Pediatrics at Nemours Children's Health System and a Principal Investigator for the MoveDMD study, gave a presentation about edasalonexent and the trial results.

Catabasis is focused on moving edasalonexent forward for the treatment of Duchenne. We are preparing for the Phase 3 trial studying edasalonexent in DMD.

If you have questions about edasalonexent or the global Phase 3 trial, contact our clinical team at DMDtrials@catabasis.com.

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