

# catabasis QUARTERLY

Updates on edasalonexent  
and clinical trials



## The PolarisDMD trial is underway!

We are pleased to announce that we have initiated our Phase 3 PolarisDMD clinical trial with edasalonexent in Duchenne muscular dystrophy and we are excited for the sites to begin enrolling participants ages 4 to 7 years old (up to 8th birthday), any mutation type, who have not been taking steroids for at least 6 months. We are not enrolling boys taking steroids due to a potential partial overlap in mechanism. As soon as a site has the required approvals, enrollment at that site can begin. In the coming days additional inclusion and exclusion criteria as well as the sites that are ready to begin enrollment and contact information will be available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

**The Phase 3 PolarisDMD trial:** We plan to enroll approximately 125 boys with DMD in this global, placebo-controlled Phase 3 trial with 2 boys receiving edasalonexent for every 1 boy receiving placebo. **The primary endpoint is the North Star Ambulatory Assessment, which was designed specifically to assess function in those affected by Duchenne.** The PolarisDMD trial also includes timed function tests (10-meter walk/run, 4-stair climb and time to stand) as well as assessments of muscle strength, growth, cardiac and bone health. After 12 months in the trial, all boys are expected to receive edasalonexent in an open-label extension with regulatory permission.

**Our goal** was to make the PolarisDMD trial accessible to interested families and to minimize trial burden and time away from home, so we have carefully considered the number of site visits and the time required for each visit. We expect to have approximately 40 clinical trial sites globally and we will cover travel costs for the clinical trial visits, which are scheduled every 3 months, and all study participants will have travel coordination. This trial does not include muscle biopsies, MRI or a 6-minute walk test. Contact our clinical team at [DMDtrials@catabasis.com](mailto:DMDtrials@catabasis.com) with any questions.

### North Star Ambulatory Assessment

Assessment measures— from most to least difficult

Hop right leg	Climb box step right
Hop left leg	Climb box step left
Stand on heels	Stand on one leg right
Rise from floor	Stand on one leg left
Run	Get to sitting
Jump	Rise from chair
Lift head	Walk
Descend box step right	Stand
Descend box step left	

#### How measures are scored

2 Can perform	1 Can perform with difficulty	0 Unable to perform
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**About edasalonexent (CAT-1004):** Inhibits NF- $\kappa$ B, which plays a fundamental role in skeletal and cardiac muscle disease in DMD. NF- $\kappa$ B is a signaling pathway responsible for disease progression in DMD. Edasalonexent has the potential to halt inflammation and fibrosis and to promote muscle regeneration. Edasalonexent was designed as a stand-alone therapy, but it may also enhance the efficacy of dystrophin upregulation therapies. Edasalonexent comes in small gel capsules that are taken orally with food three times per day.



**Edasalonexent has been shown to preserve muscle function and substantially slow Duchenne disease progression in the MoveDMD trial. Edasalonexent has shown a favorably differentiated tolerability profile from the corticosteroid standard of care with no safety signals and age-appropriate growth through more than 1 year of treatment.**



**Our vision:** Edasalonexent is a potential new standard of care therapy for all affected by Duchenne, regardless of mutation type and at all stages of life.

## MAKING COMMUNITY CONNECTIONS



**At the CureDuchenne Cares Family Workshop**—September 15 in Salt Lake City, UT and September 22 in Nashville, TN. An informative class on Duchenne muscular dystrophy, focused on educating parents and caregivers on the best standard of care for Duchenne management.

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**At the 23rd International Annual Congress of the World Muscle Society**—October 3-6 in Mendoza, Argentina. The premier annual congress on neuromuscular disorders, attended by physicians, researchers, therapists and neuropathologists from all over the world.

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**At the PPMD End Duchenne Tour**— September 21 in Billings, MT by webinar and October 13 in Dallas, TX. 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.

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**At the Jett Foundation Family Workshop**—also on October 13 in Louisville, KY. Every year, this multi-city national educational program brings clinicians, researchers, and families affected by Duchenne together to learn about care, crucial information and resources.

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**At the 47th Child Neurology Society Annual Meeting**—October 15-18 in Chicago, IL. More than 1,200 child neurologists and care team members from all over North America are expected to attend this year's meeting.

**Now enrolling: Our PolarisDMD trial studying edasalonexent in DMD has begun.** Follow us @CatabasisPharma on Facebook and Twitter for frequent updates about edasalonexent and our Phase 3 PolarisDMD trial. If you have questions about edasalonexent or are interested in our global Phase 3 PolarisDMD trial, contact our clinical team at [DMDtrials@catabasis.com](mailto: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](http://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 yet been approved by the US Food and Drug Administration.

