Issue 17 | March 2019

catabasis CONNECTION

Updates on edasalonexent and clinical trials

Get the story behind the PolarisDMD experience!

Here's an example of what it would be like for a family to participate in our PolarisDMD trial:

John's mom or dad visits **clinicaltrials.gov** to see if PolarisDMD is a good fit for John based on the eligibility criteria.

His parent reaches out to **DMDtrials@catabasis.com** for answers to any questions and is connected with the clinical trial site that's closest to home.

John's family learns that Catabasis will pay for expenses, including **flights**, **transportation**, **hotels**, and **meals** for all site visits.

At John's Screening visit, he does timed tests like...

Standing up from the ground

Walking 10 meters

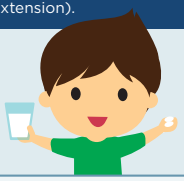
Climbing four stairs

There are no muscle biopsies or MRIs!

John meets the entry criteria during the Screening visit and returns for the **Baseline visit** where he is enrolled in the PolarisDMD trial. John starts on study drug that day (**2 boys receive edasalonexent for each boy that receives placebo**).

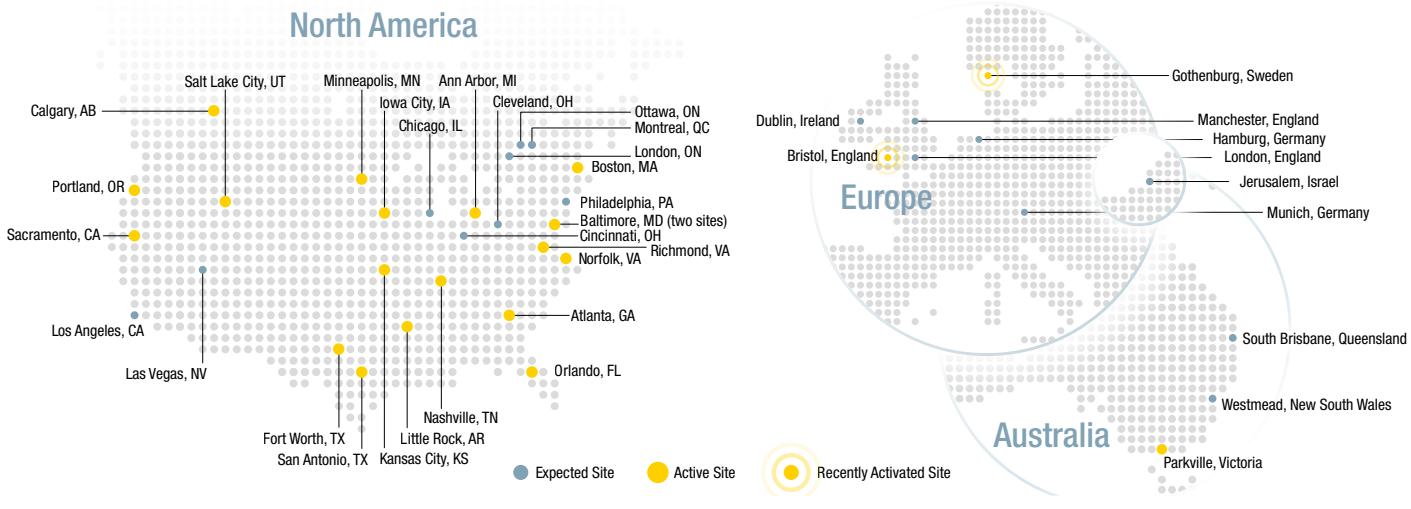
John and his family return to the clinical trial site **once every three months** during the trial. John and his family talk with the site in between visits to share how they are doing in the trial.

At home, **John takes 2 or 3 capsules with food** 3 times a day throughout the trial. After completing the PolarisDMD trial, John would be eligible to participate in the GalaxyDMD study (to receive edasalonexent in an open-label extension).



PHASE 3 GLOBAL PolarisDMD SITE UPDATE!

Enrollment of boys ages 4 to 7 (up to 8th birthday) with Duchenne muscular dystrophy continues, with **most planned sites enrolling in the U.S. and a number of international sites now open!** PolarisDMD is enrolling boys regardless of mutation type that have not been on steroids for the past 6 months. Please reach out to **DMDtrials@catabasis.com** with questions and to find the site closest to you.



THE LATEST SUPPORT FOR EDASALONEXENT AS A POTENTIAL FOUNDATIONAL THERAPY

Last month we shared additional results from our Phase 2 MoveDMD trial and open-label extension at the XVII International Conference on Duchenne and Becker Muscular Dystrophy. In this clinical trial, boys receiving edasalonexent grew age appropriately in both height and weight: They grew an average of 2.1 inches taller per year, and gained 2.9 pounds per year. This resulted in decreased BMI that approached the average BMI for unaffected boys. **We are excited to see boys on edasalonexent continue to grow like their unaffected peers, while also showing slowed disease progression compared to an off-treatment control period.**

About edasalonexent (CAT-1004)

Edasalonexent inhibits NF-kB, a protein which plays a fundamental role in skeletal and muscle disease in DMD. By inhibiting NF-kB, edasalonexent has the potential to decrease inflammation and fibrosis, promote muscle regeneration, and slow disease progression. Edasalonexent was designed as a stand-alone therapy and may also enhance the efficacy of dystrophin targeted therapies.

MAKING COMMUNITY CONNECTIONS

March 28 Webinar! Catabasis is co-hosting a webinar with CureDuchenne—Want to learn all about PolarisDMD and our new GalaxyDMD trial? Be sure to sign up!

CureDuchenne Cares—Catabasis will attend on **April 27th in Chicago, IL,** and on **May 4 in Albuquerque, NM**. Learn all about these immersive, educational workshops at www.cureduchenne.org/workshops.

Jesse's Journey—Join us at the Defeat Duchenne Family Forum on **May 25th in London, Ontario** for research and clinical trial updates tailored to Canadian families affected by Duchenne. Learn more at www.jessesjourney.com/familyforum.

Jett Foundation Family Workshops—Catabasis will attend workshops held in **Atlanta, GA on April 6th,** and **Seattle, Washington on May 18th.** Learn all about the Jett Foundation's national education program at www.jettfoundation.org/familyworkshops.

MDA Clinical and Scientific Conference—Catabasis is excited to attend **April 13-17 in Orlando, FL.** Learn more at mda.org/conferences/2019-clinical-and-scientific-conference.

PPMD End Duchenne Tour—Catabasis will be on the tour in **Portland, ME on April 27th.** To learn about PPMD's effort to reach every single family facing a Duchenne diagnosis in the US, visit www.parentprojectmd.org/get-involved/attend-events/end-duchenne-tour.

Stay in touch!

Join our mailing list: http://www.catabasis.com/patients-families/for-further-information.php

Follow us on social media: @CatabasisPharma.

Ask a question about the trial: DMDtrials@catabasis.com

The information provided here is for parents and caregivers of boys with Duchenne muscular dystrophy. Edasalonexent is an investigational drug that is not yet approved in any territory.

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POLARIS DMD