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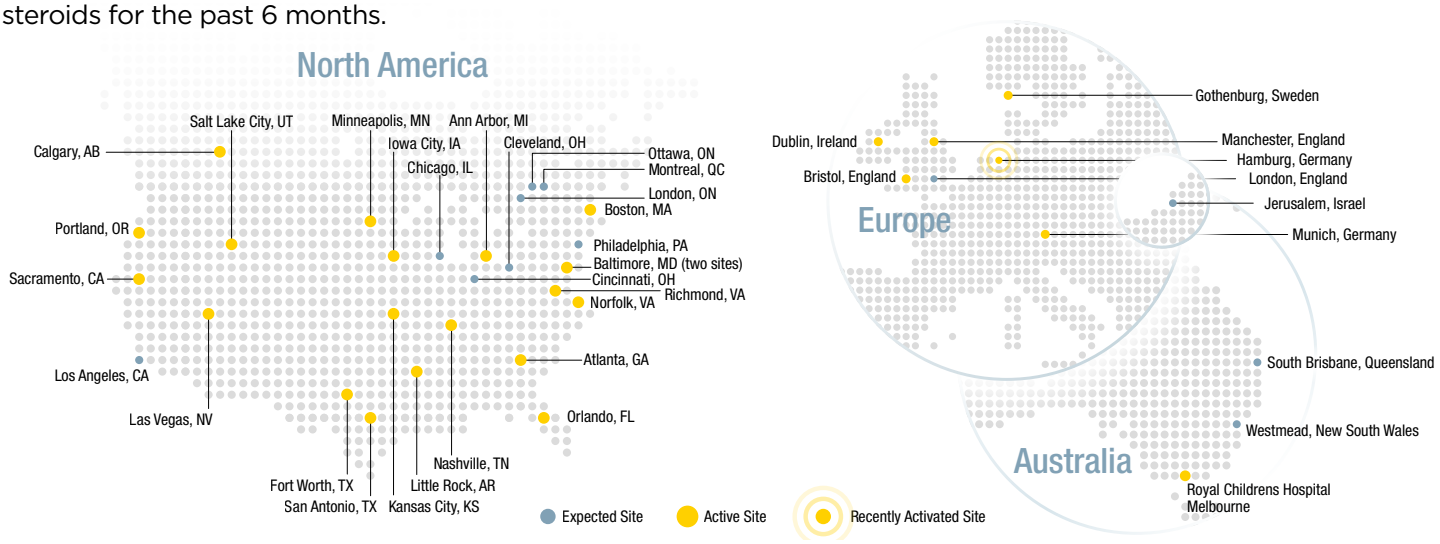
CONNECTION
Updates on edasalonexent and clinical trials
Issue 18 | May 2019

PolarisDMD Welcomes European Sites!

27 global sites currently open for enrollment

Clinical trial sites in Europe are now open for enrollment in Bristol and Manchester, England; Munich and Hamburg, Germany; Dublin, Ireland; and Gothenburg, Sweden, with additional sites in Europe expected in the coming weeks. This means that PolarisDMD is now active in 7 countries! The Phase 3 PolarisDMD clinical trial of edasalonexent in Duchenne is enrolling boys ages 4 to 7 (up to 8th birthday), regardless of mutation type, who have not been on steroids for the past 6 months.



AN EASY TRANSITION FOR BOYS AND THEIR FAMILIES

INTRODUCING GALAXY DMD

GalaxyDMD is a recently-launched, open-label extension study. It is designed to collect long-term data in boys taking edasalonexent as they get older and to provide the opportunity to stay on drug while edasalonexent is in clinical development. All boys in the Phase 2 MoveDMD trial will transition to GalaxyDMD once they complete their final visit in MoveDMD, which can often be performed at the same visit. When boys in the Phase 3 PolarisDMD trial complete the 12-month trial, they will also have the opportunity to transition to GalaxyDMD. For greater convenience, now that additional sites are open for the Phase 3 PolarisDMD study, there is flexibility for boys who participated in the Phase 2 MoveDMD trial to relocate to a site closer to home, if desired. And we are especially pleased to share that once boys from MoveDMD and PolarisDMD enter GalaxyDMD, their brothers ages 4 to 10 (up to 11th birthday) who meet the inclusion criteria are also able to participate in GalaxyDMD and receive edasalonexent! Site visits for GalaxyDMD are once every 6 months.

EDASALONEXENT: THE POTENTIAL TO PRESERVE MUSCLE FUNCTION IN BOYS WITH DUCHENNE

The primary outcome measurement in the Phase 3 PolarisDMD trial (also called the "endpoint") is the North Star Ambulatory Assessment (NSAA), which was designed to assess muscle function in ambulatory boys affected by Duchenne. The NSAA consists of 17 different measures similar to activities in daily life, such as standing on one leg or rising from a chair.

Why did we select NSAA as the Phase 3 primary endpoint?

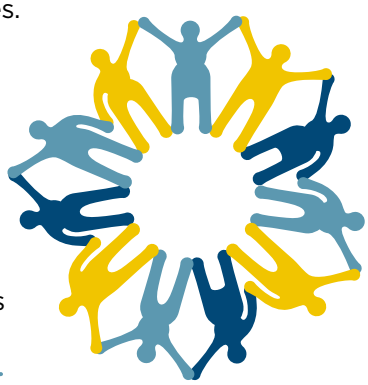
- In our Phase 2 MoveDMD study and open label extension, off-treatment we observed a decrease in boys' average NSAA score, suggesting that muscle function was declining.
- Once boys were on edasalonexent through 72 weeks, we saw their NSAA scores largely stabilized, suggesting that muscle function was preserved.
- We also saw evidence supporting preservation of muscle function with edasalonexent in all of the other timed-function tests including time to stand, 10-meter walk/run, and 4-stair climb, compared to off-treatment control.

We are assessing NSAA and these same timed-function tests in the Phase 3 PolarisDMD trial.

About edasalonexent (CAT-1004)

Edasalonexent inhibits NF-kB, a protein which plays a fundamental role in skeletal and cardiac 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 is being developed as a potential stand-alone therapy and also has the potential to be combined with dystrophin-targeted therapies.

MAKING COMMUNITY CONNECTIONS



Did you miss out on our recent webinar with CureDuchenne?
Check it out here

CureDuchenne Cares—Catabasis will attend on **May 4th in Albuquerque, NM** and **June 1st in Detroit, MI**. 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 be at the **May 18th Seattle, Washington workshop.** Learn all about the Jett Foundation's national education program at www.jettfoundation.org/familyworkshops

PPMD Annual Conference—Join us **June 26th through 30th in Orlando, FL** at PPMD's 25th Annual Conference for families, physicians, researchers, caregivers, industry partners, and those living with Duchenne to gather and discuss all topics Duchenne! For more about this event, go to www.parentprojectmd.org/get-involved/attend-events/annual-conference/

Stay in touch!

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