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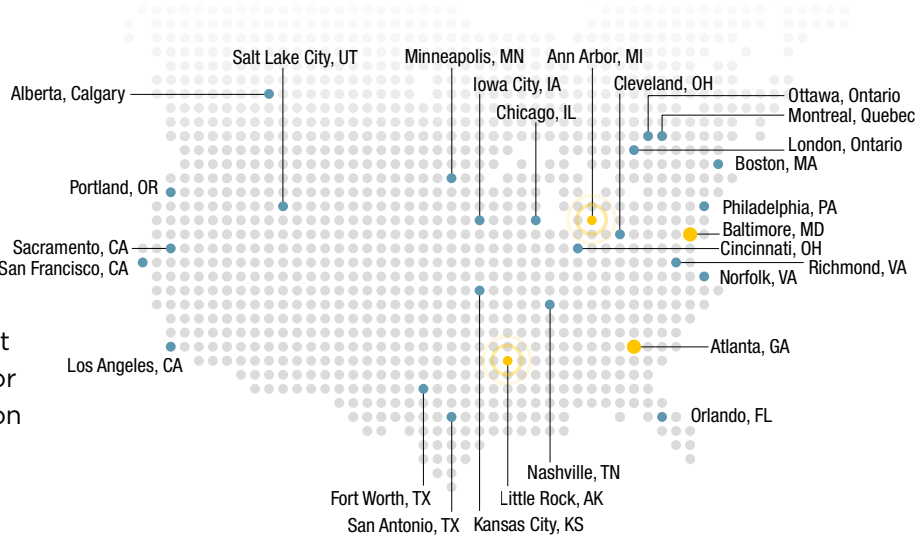
catabasis QUARTERLY

Updates on edasalonexent and clinical trials

PolarisDMD trial is actively recruiting patients for enrollment!

We are excited to be enrolling patients in our Phase 3 PolarisDMD clinical trial with edasalonexent in Duchenne muscular dystrophy (DMD). Sites located in Atlanta, Baltimore, Little Rock, and Ann Arbor are now enrolling boys ages 4 to 7 (up to 8th birthday), any mutation type, who have not been on steroids for at least 6 months. More sites are coming soon, and we expect to have approximately 40 sites globally. For more information on inclusion and exclusion criteria for our Phase 3 PolarisDMD trial, please visit **www.clinicaltrials.gov**; and reach out to our clinical team at **DMDtrials@catabasis.com** with any questions or if you are interested in participating in this trial.



NOW LAUNCHING: **ANN ARBOR & LITTLE ROCK**

PolarisDMD FREQUENTLY ASKED QUESTIONS

Q *How often would I travel to the clinical trial site?*

A Clinical trial site visits and key assessments are every three months after the first dose, for typically 1-2 days each visit. Travel costs to the sites will be provided for the study participant and a caregiver.

Q *How can I find out if the site closest to me is actively screening patients for the trial?*

A We're here to help—contact us at **DMDtrials@catabasis.com**.

Q *Why is edasalonexent taken with 8 grams of fat?*

A We have found that edasalonexent is absorbed best when each dose is taken with food that provides small amounts of fat; for example, a glass of whole milk, a grilled cheese sandwich, an egg, or even butter on a pancake!

Q *What could I do if my son has trouble taking pills?*

A We will provide a Medi-straw, which helps the boys explore how to swallow pills by drinking through this straw. The boys in our Phase 2 MoveDMD trial learned very quickly how to take edasalonexent capsules.

ABOUT EDASALONEXENT (CAT-1004)

Edasalonexent inhibits NF-kB, which plays a fundamental role in skeletal and cardiac muscle disease in DMD. NF-kB has a key role in skeletal and cardiac muscle disease in DMD, and inhibiting it has the potential to decrease inflammation and fibrosis and promote muscle regeneration. Edasalonexent was designed as a stand-alone therapy and may also enhance the efficacy of dystrophin upregulation therapies such as exon skipping and gene therapy.

MoveDMD Trial Data through 72 Weeks of Edasalonexent Treatment

We recently presented new data at the World Muscle Society Congress from the MoveDMD trial and open-label extension study:

- Positive efficacy results through 72 weeks of edasalonexent treatment showed preserved muscle function and slowing of disease progression in all assessments of muscle function (North Star Ambulatory Assessment, time to stand, 4-stair climb and 10-meter walk/run) compared to the off-treatment control period
- Significant decreases in muscle enzymes, including CK, through 72 weeks in boys treated with edasalonexent, supporting the durability of edasalonexent treatment effects
- Significantly decreased heart rate towards age-normative values, supporting the potential beneficial cardiac effects of edasalonexent
- Edasalonexent continued to be well tolerated with no safety signals observed in the trial. Boys treated with edasalonexent continue to follow age-appropriate growth curves with age-appropriate increases in weight and height and overall BMI has trended down to age-normative values

MAKING COMMUNITY CONNECTIONS

Catabasis and PPMD Webinar—We recently co-hosted a webinar with Parent Project Muscular Dystrophy to share information about our PolarisDMD Phase 3 clinical trial of edasalonexent in Duchenne Muscular Dystrophy. You can view our presentation **here** and watch the webinar **here**.

At the Jett Foundation Family Workshop—January 12 in Phoenix, AZ. The Jett Foundation hosts a national education program that brings clinicians, researchers, and families affected by Duchenne together to learn about care, crucial information and resources in many cities each year.

At the Parent Project International Conference on Duchenne and Becker Muscular Dystrophy—February 16-17 in Rome Italy. The Parent Project Italy will be hosting this conference for families, doctors, researchers and companies dedicated to care and therapies in DMD and BMD. Over 600 people from Italy and the world will gather to learn about the latest advances in research.

Now enrolling! **Our PolarisDMD trial studying edasalonexent in DMD is underway. To stay updated on developments, follow us @CatabasisPharma on Facebook and Twitter and join our mailing list at http://www.catabasis.com/patients-families/for-further-information.php. This newsletter can be downloaded from our website at www.catabasis.com. If you have questions or are interested in edasalonexent or our global Phase 3 PolarisDMD trial, contact our clinical team at DMDtrials@catabasis.com.**

The information provided here is for parents and caregivers of boys with Duchenne muscular dystrophy (DMD). Edasalonexent is an investigational drug that is not yet FDA approved.

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POLARIS DMD