catabasis QUARTERLY

Updates on edasalonexent and clinical trials

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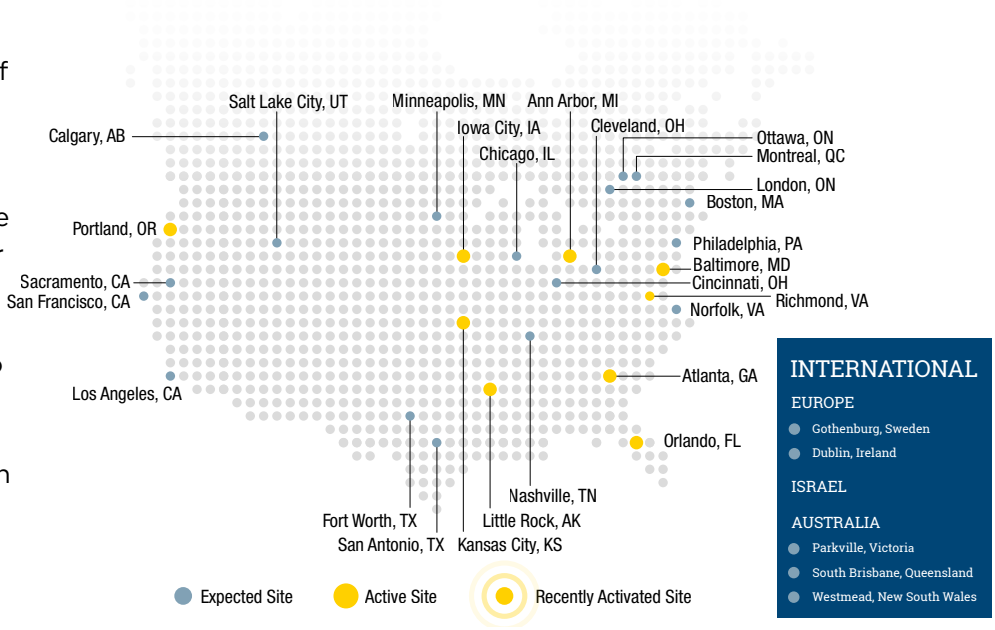
New Year, new trial sites open for enrollment for the Phase 3 PolarisDMD trial!

Happy New Year! As 2019 kicks off, our Phase 3 PolarisDMD clinical trial of edasalonexent in Duchenne muscular dystrophy (DMD) continues to gain momentum. We are excited to share that a total of 10 clinical trial sites have officially launched in the U.S., with our international sites coming soon. We are enrolling boys ages 4 to 7 (up to 8th birthday), any mutation type, who have not been on steroids for at least 6 months. Stay up to date with the latest site initiations by following us on social media (@CatabasisPharma on Facebook, Twitter, and Instagram).



HIGHLIGHTS FROM OUR DECEMBER INVESTIGATOR MEETING FOR SITES IN EUROPE, ISRAEL AND AUSTRALIA

Following our October Investigator Meeting for sites in North America, we closed out 2018 with the Investigator Meeting for sites in Europe, Israel and Australia. We were thrilled to tell our sites all about PolarisDMD! Here are some highlights and feedback from the event:

Emily Crossley, *Co-Founder of Duchenne UK,* gave the keynote address, speaking about life as a Duchenne parent and sharing how Duchenne UK strives to help support bringing treatments to patients and families.

> *"I especially liked the address made by Emily Crossley and found it really highlighted the importance of this new trial."*

Jon Rey-Hastie, *CEO of DMD Pathfinders*, spoke about life with Duchenne and the challenges he faces, speaking of the tremendous urgency there is to reduce the impact of Duchenne and improve quality of life as much as possible.

> *"The personal stories from Jon were invaluable. It's extremely important to relate this study to real life, as it's quite easy to get lost in the science."*

Jill Milne, *CEO of Catabasis*, talked about the PolarisDMD Phase 3 trial, and how passionate we are about the potential of edasalonexent to bring a new treatment option to boys living with DMD.

POTENTIAL CARDIAC BENEFITS OF EDASALONEXENT

In addition to potential skeletal muscle and diaphragm benefits, edasalonexent has the potential to improve cardiac function as well, which we are exploring further in our Phase 3 PolarisDMD trial. We understand that cardiac function is critical in those affected by Duchenne as, unfortunately, cardiac failure is the leading cause of mortality. We are passionate about learning more about the potential heart benefits of edasalonexent.

In young boys, tachycardia (an increase in the resting heart rate) is the first manifestation of cardiac disease, and **the Phase 2 MoveDMD trial and its open-label extension study had initial results suggesting that edasalonexent could have positive effects on cardiomyopathy. In the MoveDMD trial, the heart rate on the ECG significantly decreased on edasalonexent to approximately the average rate for unaffected boys.**

In Duchenne, fibrosis is associated with abnormalities in cardiac function and seen on MRI before any symptoms appear. In preclinical studies involving mouse and dog models of Duchenne, NF-kB inhibition—the mechanism of action of edasalonexent—reduced cardiac fibrosis. To further study preclinical cardiac benefits, Catabasis has partnered with Dr. Pradeep Mammen, MD, FACC, FAHA, a prominent cardiologist and founder and Medical Director of the Neuromuscular Cardiomyopathy Clinical at the University of Texas Southwestern in Dallas. The year-long collaboration will expand our understanding of the potential cardiac benefits of edasalonexent, and results are expected in the second half of 2019.

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 was designed as a stand-alone therapy and may also enhance the efficacy of dystrophin upregulation therapies.

MAKING COMMUNITY CONNECTIONS

Jett Foundation Family Workshops—This national education program brings together clinicians, researchers, and families affected by Duchenne to learn about care, crucial information, and resources. Coming up, Catabasis will be attending the workshop in **Houston, Texas on February 2**.

PPMD End Duchenne Tour—In an effort to reach every single family facing a Duchenne diagnosis in the US, PPMD hosts a community experience called the *End Duchenne Tour* to bring updates on research, advocacy, and care. Catabasis will be attending the tour in **Las Vegas, Nevada on February 2**.

CureDuchenne Cares—These one-day events provide an immersive education experience where clinicians and experts share their wealth of Duchenne knowledge and advancements in the field. Caregivers learn about physical therapy, get important updates about clinical trials, and learn valuable best practices in Duchenne care. Catabasis will be attending in **Raleigh, NC on February 8**, and **Portland, OR on February 23**.

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 (DMD). Edasalonexent is an investigational drug that is not yet FDA approved.

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