

## What's next for edasalonexent in 2020?

### Planning a new Phase 2 non-ambulatory clinical trial in partnership with Duchenne UK!

We're excited to share that Catabasis and Duchenne UK are partnering to study edasalonexent in a Phase 2 non-ambulatory Duchenne muscular dystrophy trial. Duchenne UK has granted over \$600,000 in funding to support patient and clinical trial site costs. The exploratory Phase 2 study is designed to be a 1-year, randomized, double-blind, placebo-controlled trial to assess safety, pharmacokinetics, and exploratory measures of function including cardiac, skeletal muscle, and pulmonary function in non-ambulatory DMD patients.



Approximately 16 non-ambulatory boys and men ages 10 and older regardless of mutation type who have not been on steroids for at least 6 months are planned to be enrolled at clinical trial sites in the United Kingdom. It is intended that upon completion of this trial, boys will have the option to transition to the GalaxyDMD open-label extension trial and receive edasalonexent.

To learn more about Duchenne UK, please visit their website: <https://www.duchenneuk.org/>

## Phase 3 PolarisDMD trial enrolled expected patient population

With our Phase 3 PolarisDMD trial fully enrolled, we are sharing measurements and information about the boys enrolled from the beginning of the trial. The analysis of baseline characteristics shows that patients enrolled in the Phase 3 trial have similar characteristics to the patients that enrolled in the previous Phase 2 MoveDMD trial. Both trials enrolled boys affected by Duchenne ages 4-7 (up to 8th birthday) with any mutation type who had not been on steroids for the past 6 months. Baseline age, North Star Ambulatory Assessment score, and timed function test values (time to stand, 4-stair climb, and 10-meter walk/run) were similar in both trials. There were no significant differences in these baseline characteristics between the two trials.



Top-line results from the Phase 3 PolarisDMD trial are expected in Q4 2020 and are anticipated to support an NDA filing in 2021.



# THANK YOU FOR YOUR SUPPORT IN 2019

We are so very grateful for the ongoing support of the Duchenne community. Let's take a look at what we accomplished together in 2019.



## WE'RE LOOKING FORWARD TO AN EXCITING 2020!

Catch us soon at these events:

**The CureDuchenne Cares** workshops on January 18th in Tampa, FL. Learn all about these immersive, educational workshops at [www.cureduchenne.org/workshops](http://www.cureduchenne.org/workshops)

**The Jett Foundation Family Workshops** in Worcester, MA on January 25th, Ridgeland, MS on February 8th, and Portland, OR on February 22nd. Learn all about Jett Foundation's national education program at [www.jettfoundation.org/familyworkshops](http://www.jettfoundation.org/familyworkshops)

**The PPMD End Duchenne Tour** in Houston, TX on January 18th. To learn all about PPMD's efforts to reach every single family facing a Duchenne diagnosis in the US, visit [www.parentprojectmd.org/get-involved/attend-events/end-duchenne-tour](http://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>

For trial-related questions: [DMDtrials@catabasis.com](mailto:DMDtrials@catabasis.com)

Follow us on social media: @CatabasisPharma.



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.

