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CONNECTION

May 2020

Catch up on the latest from Catabasis!

Earlier this month, we shared data from three scientific posters during the Muscular Dystrophy Association (MDA) Virtual Poster Session. Learn more about edasalonexent and its potential to provide positive effects in Duchenne below.

Boys with Duchenne demonstrate capsule swallowing abilities

In our experience with edasalonexent clinical trials, we saw that 4- to 7-year-old boys were able to learn how to swallow soft-gel capsules of two different sizes. We understand that learning to swallow capsules for the first time can be a challenge, and we are thrilled that so many boys were successful at it! In both the Phase 2 MoveDMD trial and the Phase 3 PolarisDMD trial, 97% of boys screened were able to swallow capsules and age did not impact the ability to take the larger 250 mg capsules. We also collected advice from families and physicians about teaching young boys to swallow capsules and included tips on social media and in our newsletter. We plan on sharing information with other investigators to provide educational resources for other clinical trials.

Phase 3 PolarisDMD trial enrolled expected patient population

In clinical trials, it is important to understand the group of patients enrolled. We compared the populations of the boys in our current Phase 3 PolarisDMD trial and the previous Phase 2 MoveDMD trial and found that baseline age, North Star Ambulatory Assessment (NSAA) score, and timed function test values (time to stand, 4-stair climb, and 10-meter walk/run) were similar with no significant differences in patient populations between the two trials. We believe these findings support the design of the Phase 3 trial. We intend to continue sharing information about the enrolled population of boys in the future as we learn more.

Age-normative growth and normal adrenal function observed with edasalonexent

Dr. Erika Finanger, a Principal Investigator for our Phase 2 MoveDMD, Phase 3 PolarisDMD, and open-label extension GalaxyDMD trials, reviewed safety data of boys taking edasalonexent over 3 years. In our Phase 2 MoveDMD trial and open-label extension, edasalonexent treatment was well-tolerated and associated with favorable growth patterns and normal adrenal function, because it does not impact the glucocorticoid receptor.

ABOUT EDASALONEXENT

In Duchenne, the loss of dystrophin leads to chronic activation of NF-kB, which is a key driver of skeletal and cardiac muscle disease progression. 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 both a monotherapy and for use with other therapies, including exon-skipping therapies and other dystrophin-targeted therapies in development such as gene therapy.

The Phase 3 PolarisDMD trial and GalaxyDMD open-label extension study are both ongoing.

Top-line results for PolarisDMD are expected in the fourth quarter of 2020.

POLARIS DMD

GALAXY DMD

RESOURCES FOR THE DUCHENNE COMMUNITY

For the latest resources and information related to COVID-19 and Duchenne, check out these links that are being shared by several advocacy organizations.

CureDuchenne: https://www.cureduchenne.org/coronavirus-health-update-guide/

Duchenne UK: https://www.duchenneuk.org/pages/category/coronavirus-covid-19-resources

Jett Foundation: https://www.jettfoundation.org/covid19

PPMD: https://www.parentprojectmd.org/care/ppmd-covid-19-coronavirus-information-center/

Stay in touch!

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

For questions: advocacy@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.

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GALAXY DMD

POLARIS DMD