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CONNECTION

July 2020

The value of the North Star Ambulatory Assessment in Duchenne Muscular Dystrophy

At the Parent Project Muscular Dystrophy Conference this month, we shared more information about the value of the North Star Ambulatory Assessment (NSAA). The primary endpoint of our Phase 3 PolarisDMD trial is the NSAA, which is a validated scale designed to measure the physical performance of ambulatory boys with Duchenne.

We chose the NSAA as our primary endpoint for multiple reasons

For these reasons, we believe that using the NSAA as our primary endpoint means that we can learn about edasalonexent in Duchenne and measure change over time in a way that is meaningful and impactful to families. To see our presentation from the PPMD conference, visit their OnDemand Library, which is available to all registered conference attendees.

ABOUT EDASALONEXENT

In Duchenne, the lack 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 a monotherapy and for use with other therapies such as exon-skipping. We believe that based on its mechanism of action, edasalonexent has the potential for use with other approaches 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