catabasis
CONNECTION

September 2020

World Duchenne Awareness Day

DUCHENNE AWARENESS DAY
7 sept

September 7th was World Duchenne Awareness Day, a global educational day aimed to raise awareness for those affected by Duchenne and Becker muscular dystrophy.

This year's theme was *Duchenne and the Brain*. While the absence of normal dystrophin protein in skeletal muscle is considered to be the hallmark of Duchenne and Becker, a form of dystrophin is often also missing or altered in the brain. This can contribute to learning difficulties, behavioral issues, and neurocognitive impairment. We also must consider the impact that Duchenne can have on mental and emotional health, which in many instances has been exacerbated during this period of uncertainty with COVID-19.

At Catabasis, we support and advocate for appropriate research and education about Duchenne's impact beyond skeletal muscle, including the brain and mental health.

We are honored to have participated in WDAD this year and to be a part of the strong and resilient Duchenne community.

COMMUNICATING CLINICAL TRIAL RESULTS

We understand the importance of clearly communicating clinical trial results with the Duchenne community, and as we prepare for the planned release of top-line results from our Phase 3 PolarisDMD trial in Q4 of this year, we are sharing in advance our plans for how those results will be communicated. As a public, pre-commercial stage company, we are making every effort to balance our reporting obligations and restrictions with the desire to inform the Duchenne community of the trial results quickly, efficiently, and in easily accessible ways. Here are our plans:

We plan to share top-line results in our community newsletter and in a press release simultaneously. Anyone who would like to receive these materials directly from us by email can sign up here: **https://www.catabasis.com/patients-families/for-further-information.php**

We intend to immediately share both the newsletter and press release on our social media as well, so please follow us on social media @CatabasisPharma on Facebook, Twitter, and Instagram.

We also plan to host a webinar for the Duchenne community in conjunction with PPMD within a couple of days of the release of the top-line results. The webinar will include a Q&A portion where you can ask questions.

Families participating in the Phase 3 PolarisDMD trial can hear about next steps directly from their trial site personnel.

We understand the importance of providing our clinical trial results in a thoughtful and timely manner and are committed to doing our best for the community.

ABOUT EDASALONEXENT

Edasalonexent is an orally-administered small molecule designed to inhibit NF-kB. Activated NF-kB is a key link between the lack of dystrophin and resulting manifestation and progression of Duchenne. By inhibiting NF-kB in Duchenne, edasalonexent has the potential to limit muscle degeneration, promote muscle regeneration, and reduce inflammation and fibrosis. 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 trial are both ongoing. **Top-line results are expected in the fourth quarter of 2020.**

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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