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catabasis QUARTERLY

Updates on edasalonexent and clinical trials

INTRODUCING THE POLARIS DMD TRIAL

We are pleased to announce that the Phase 3 clinical trial with edasalonexent in Duchenne will be known as the POLARIS DMD trial. The new name is fitting because Polaris is the brightest star in its constellation and is also known as the North Star, which is the trial's planned primary endpoint (the North Star Ambulatory Assessment). We are preparing for POLARIS DMD and we anticipate beginning enrollment in the second half of 2018.

Next steps for edasalonexent and boys with DMD: We are preparing for the POLARIS DMD trial to enroll approximately 125 boys with DMD between the ages of 4-7 years old regardless of mutation type and who have not been on steroids for at least 6 months. POLARIS DMD is planned to be a single, global, placebo-controlled Phase 3 trial with 2 boys receiving edasalonexent for every 1 boy receiving placebo. Endpoints are planned to be similar to the Phase 2 MoveDMD trial, including the North Star Ambulatory Assessment and timed function tests. After 12 months in the trial, all boys are expected to receive edasalonexent in an open-label extension.

ABOUT EDASALONEXENT (CAT-1004): A potential foundational therapy for all affected by Duchenne regardless of mutation type, edasalonexent is administered orally and inhibits NF-kB, decreasing muscle degeneration and enhancing muscle regeneration with expected benefits in skeletal muscle, diaphragm and heart. Edasalonexent could be taken on its own and may also enhance the efficacy of dystrophin upregulation therapies.

Edasalonexent has been shown to preserve muscle function and substantially slow Duchenne disease progression in the MoveDMD trial.

EDASALONEXENT CLINICAL RESULTS TO DATE: Clinically meaningful slowing of disease progression on edasalonexent compared to off-treatment control period through more than 1 year of treatment.

- North Star Ambulatory Assessment stabilized
- Timed function tests stabilized (10-meter walk/run, 4-stair climb and time to stand)

- **Additional measures of muscle health support positive edasalonexent effects**
  - Magnetic resonance spectroscopy (MRS) fat fraction in the soleus (lower leg) and vastus lateralis (upper leg) muscles showed less fat accumulation when boys were taking edasalonexent versus the off-treatment control period. Fat accumulation correlates with impaired functional abilities
  - Statistically significant improvement in muscle magnetic resonance imaging (MRI) T2 versus off-treatment control period. Decrease in progression of MRI T2 is consistent with slowing of disease progression observed in functional assessments
  - Statistically significant decrease in muscle enzymes compared to baseline
  - Statistically significant decrease in CRP, a marker of systemic inflammation, compared to baseline

Edasalonexent has shown a favorably differentiated tolerability profile from the corticosteroid standard of care with no safety signals and age-appropriate growth through more than 1 year of treatment. Height and weight growth through 60 weeks of edasalonexent treatment was age-appropriate and on track with standard growth curves for unaffected boys in the same age range. BMI trended towards a decrease. This profile is favorably differentiated from the typical profile associated with the corticosteroid standard of care in DMD, which includes weight gain and curtailed growth. To see complete results to date, visit www.catabasis.com.

MAKING COMMUNITY CONNECTIONS

At the 2018 New Directions in Biology and Disease of Skeletal Muscle Conference—June 25-28 in New Orleans, LA. This eighth biannual meeting highlighted current developments in muscle biology, disease, and therapy with presentations by leading international researchers.

At the PPMD Annual Connect Conference—June 28-July 1 in Scottsdale, AZ. The 2018 PPMD Annual Conference marks the 24th time families, physicians, researchers, caregivers, industry partners, and those living with Duchenne will gather to discuss all topics Duchenne.

At the upcoming MDA Summer Camp—August 8 and 15 in Amston, CT. Catabasis will participate to connect with the community at MDA summer camp, offering a unique residential camping experience to children with neuromuscular disease.

Coming soon: We are preparing for POLARIS DMD, the Phase 3 trial studying edasalonexent in DMD, and we anticipate beginning enrollment in the second half of 2018.

If you have questions about edasalonexent or the global Phase 3 trial, contact our clinical team at DMDtrials@catabasis.com.

Stay updated on edasalonexent developments by joining our mailing list: http://www.catabasis.com/patients-families/for-further-information.php.
You can also download this newsletter from our website at www.catabasis.com.

The information provided here is for parents and caregivers of boys with Duchenne muscular dystrophy (DMD). Edasalonexent is an investigational drug that has not been approved by the US Food and Drug Administration.

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POLARIS DMD