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catabasis QUARTERLY

Updates on edasalonexent and the MoveDMD® trial

To the DMD community: we are incredibly happy about the recent approval of EXONDYS 51.

Catabasis news for you and a new website!

The MoveDMD trial with edasalonexent (CAT-1004) in Duchenne muscular dystrophy (DMD) continues to progress, advancing our belief in the potential of edasalonexent to be disease-modifying in boys with DMD with any mutation type. Edasalonexent is an oral investigational drug that has not been approved by the US Food and Drug Administration. Now you can stay abreast with Catabasis news online at our recently launched new website! Check it out at **catabasis.com**.

Enrollment is now complete for Part B of the MoveDMD trial

We are pleased to report that we have completed target enrollment in the MoveDMD trial. This trial is the 12-week, placebo-controlled, double-blind portion of the MoveDMD trial to evaluate the safety and efficacy of edasalonexent. We expect to announce top-line results in the first half of Q1 2017, once all patients have completed the 12-week placebo-controlled portion of the trial and an analysis of the data has been performed.

More news on Part B of the MoveDMD trial. Patients who complete the placebo-controlled 12-week portion of the MoveDMD trial are continuing on to the open-label extension part of the trial, which consists of 36 weeks of dosing with edasalonexent. In addition to safety monitoring, the same periodic efficacy-related assessments will be performed as in Part B of the trial including magnetic resonance imaging (MRI), timed function tests, muscle strength measures, the North Star Ambulatory Assessment and the pediatric outcomes data collection instrument (PODCI).

T2 MRI to measure muscle changes in patients in the MoveDMD trial

Edasalonexent is designed to inhibit NF-κB, a protein that plays an important role in inflammation and muscle health. We are studying edasalonexent to see if targeting NF-κB will reduce muscle damage and improve function in boys affected by DMD. The primary measurement tool will be MRI, a non-invasive imaging technique to view muscle structure and composition and help assess disease progression in children with DMD. Changes in MRI measures have been associated with clinically meaningful, longer-term changes in functional activity. We will be using a certain type of MRI measure, known as T2, since studies with steroids have shown that muscle change is observable in a relatively short period of time (less than 12 weeks) using T2.

UNPRECEDENTED COLLABORATION BETWEEN COMPANIES IN DUCHENNE

Catabasis and Sarepta announced a joint research collaboration in DMD. This unprecedented research collaboration in DMD will explore a combination drug treatment approach for DMD in preclinical models. The two companies will contribute their respective expertise to study an exon-skipping treatment developed by Sarepta together with an oral NF-κB inhibition treatment developed by Catabasis in a mouse model of DMD. We are excited to work with Sarepta on this joint research collaboration, which to our knowledge is the first time two companies are testing a combination of investigational therapies to treat Duchenne.

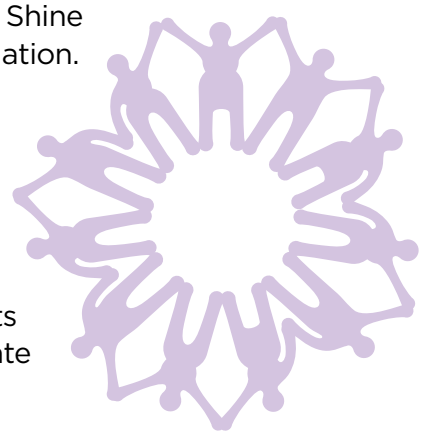
MAKING COMMUNITY CONNECTIONS

At the 22nd annual PPMD Connect Conference—June 26-29 in Orlando, FL. Much of this year's conference was live-streamed, enabling more people in the DMD community to hear important updates in research, advocacy and care.

At the CureDuchenne Cares Workshop on July 9 in Chicago, IL and July 30 in Sacramento, CA. This program provides learning on the standard of care for loved ones with DMD. We appreciated the opportunity to have participated in both of these summer workshops.

Supporting World Duchenne Awareness Day in Cambridge, MA with the Shine a Light On Duchenne event on September 7 organized by the Jett Foundation.

At the Jett Foundation Regional Roundtable Series on September 10 in Waltham, MA. Partnering with Duchenne organizations, clinicians, institutions, industry, and other experts, these forums examine issues and curate information around recently completed trials or those underway.



At the 14th Action Duchenne International Conference 2016 in London, UK on November 11th and 12th; families will hear from international experts and learn from other families and those living with Duchenne how to create the best life possible. Academics and clinicians will learn about the latest research and share expertise in treating those affected by Duchenne.

Enrollment is complete for Part B of the MoveDMD trial and the 36-week open-label extension continues. Additional trials being planned. Depending on results from Part B of the MoveDMD trial, we are planning a Phase 3 trial in 4-7 year-old boys as well as a trial in non-ambulatory boys and men affected by Duchenne in 2017. If you are interested in possible future clinical trials with edasalonexent, you are welcome to join our mailing list: http://www.catabasis.com/patients-families/for-further-information.php.

For more information or questions on the MoveDMD trial go to https://clinicaltrials.gov/ct2/show/NCT02439216 or contact joanne.donovan@catabasis.com. You can also download this newsletter at www.catabasis.com.

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

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Move DMD