Issue 4 | July 2016

catabasis QUARTERLY

Updates on the MoveDMD® trial

Catabasis news for you. CAT-1004 now has a name.

In the past year since launching this newsletter, there has been much to share about CAT-1004 with families touched by Duchenne muscular dystrophy (DMD). CAT-1004 now has a generic name: edasalonexent (pronounced "ē-dah-saloh-*nex*-ent"). The MoveDMD trial has progressed, and we believe in the potential of edasalonexent to be disease-modifying, facilitating muscle regeneration 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.

Open-label extension in the MoveDMD trial has begun

We are pleased to report that the first patients enrolled in the MoveDMD trial are now receiving edasalonexent for 36 weeks following the 12-week, placebo-controlled, double-blind portion (Part B) of the trial. During the open-label extension, safety will be monitored as well as periodic additional assessments, including magnetic resonance imaging (MRI), timed function tests, muscle strength measures, the North Star Ambulatory Assessment and the pediatric outcomes data collection instrument (PODCI).

Part B of the MoveDMD trial continues on track, is open for enrollment, and plans to enroll approximately 30 boys ages 4-7 with DMD in the US to assess the effects of 12 weeks of edasalonexent compared to placebo. In addition to new participants, the boys who participated in Part A of the trial may enroll in Part B if they remain eligible. Favorable safety, tolerability, pharmacokinetics and positive biological marker results were observed in patients from Part A: No safety signals were reported with edasalonexent and adequate levels of drug in the blood were seen when it was taken orally. We are grateful to the participants, families and clinical trial site staff who are making the MoveDMD trial possible, and to Parent Project Muscular Dystrophy (PPMD) and the Muscular Dystrophy Association (MDA) for providing travel funding for Part A and Part B of the trial, respectively.

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 measures of 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.

KEEP PACE WITH THE MoveDMD® TRIAL

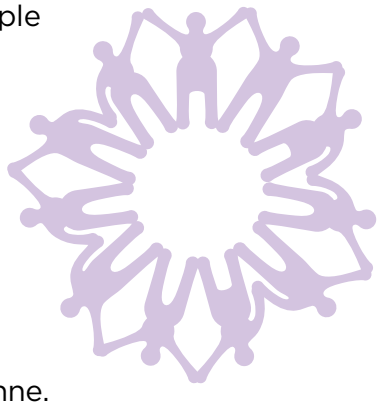
The MoveDMD trial is designed to assess the safety of edasalonexent in boys ages 4-7 with DMD and whether it produces signs of reduced muscle damage and inflammation on MRI measures. Other measures such as physical function and muscle strength will also be assessed; no muscle biopsies are needed for this trial. Eligibility criteria include not being on corticosteroids currently or in the past six months. **Please contact us (details below) or see contact information on clinicaltrials.gov if you are interested. Travel expenses for participants and families will be covered with support from the MDA, and we thank the MDA for this grant support.**

More information about the MoveDMD trial is also available in two archived webinars that are available for replay:

- with PPMD on June 8, 2016
- with CureDuchenne on June 22, 2016

We're grateful for the community's continuing support of MoveDMD

Last month, from June 26–29, Parent Project Muscular Dystrophy hosted a unique convergence of industry partners, scientific leaders, medical providers, people living with Duchenne, and their families at the Renaissance Orlando at Sea World Hotel in Orlando, Florida.



Catabasis thanks PPMD for the opportunity to present an update on the MoveDMD trial from the podium on June 27 and at the meeting's poster session as well as the chance to participate in and learn from many of the sessions.

At Catabasis, we remain grateful for our ongoing close partnerships with CureDuchenne, the Jett Foundation, MDA and PPMD and the opportunities that we continue to have at their events to meet those impacted by Duchenne.

Part B of the MoveDMD trial continues and the first patients who have completed the placebo-controlled 12-week portion are now being dosed in a 36-week extension. Part A showed positive safety, tolerability, pharmacokinetics and biomarker data. We are recruiting additional boys interested in participating in the trial. Clinical trial sites are active in Oregon (Shriners Hospitals for Children), Florida (University of Florida) and Pennsylvania (The Children's Hospital of Philadelphia) and are expected to be active soon at an additional location in Florida (Nemours Hospital) as well as in California (UCLA). Travel funding is available for participants and their immediate families.

For more information or enrollment 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