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catabasis QUARTERLY

Updates on the MoveDMD℠ trial

Catabasis news for you. Our latest newsletter is designed to update families touched by Duchenne Muscular Dystrophy (DMD) with the latest information about CAT-1004 and the MoveDMD trial. We believe that CAT-1004 may have disease-modifying potential and the ability to facilitate muscle regeneration in boys with DMD with any mutation type. CAT-1004 is an investigational drug that has not been approved by the US Food and Drug Administration.

MoveDMD Part B: first patient dosed

We are pleased to report the start of the MoveDMD trial's Part B, which will assess the effects of 12 weeks of CAT-1004 in boys with DMD. This announcement comes on the heels of Catabasis' announcement of positive biological marker results from Part A of the MoveDMD trial. In Part A, biomarkers showed that genes driven by NF-κB—the biological target of CAT-1004—decreased in boys taking the drug for a week at the doses that are now being studied in Part B. These data follow previously reported safety, tolerability and pharmacokinetics results from Part A; in those studies, no safety signals were reported with CAT-1004 in boys with DMD, 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 made MoveDMD Part A possible, and to PPMD for providing travel funding for the trial.

Part B of the MoveDMD trial is expected to enroll approximately 30 boys in the US. The boys who participated in Part A of the trial may enroll in Part B, if they remain eligible.

EYES ON CAT-1004

A look at the science behind the MoveDMD trial: CAT-1004 is designed to inhibit NF-κB, a protein that plays an important role in inflammation and muscle health. We are studying CAT-1004 to see if targeting NF-κB will reduce muscle damage and improve function in boys with DMD; we believe that CAT-1004 has the potential both to reduce muscle inflammation and degeneration and to promote muscle regeneration in boys with DMD regardless of mutation type. In previous studies with adults, CAT-1004 showed no safety signals and was generally well tolerated. In Part A of the MoveDMD trial, CAT-1004 reached blood plasma drug levels consistent with those at which NF-κB inhibition was seen in adults.

MDA Clinical Conference brings DMD community together: The MDA recently hosted this confluence of patients, caregivers, and the top researchers in neurodegenerative diseases March 20–23 at the Hyatt Regency in Arlington, Virginia. Many new advances in basic research, patient care, and treatments for those suffering from neuromuscular diseases such as DMD were discussed in this premier scientific venue.

Catabasis is grateful to the MDA for the opportunity to present data from Part A of our MoveDMD trial from the podium on March 23, as well as at the poster session of the meeting.

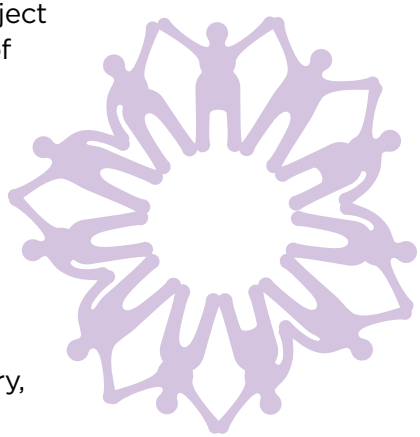
KEEP PACE WITH MoveDMD℠ TRIAL

The aim of the MoveDMD clinical trial is to assess the safety of CAT-1004 in boys ages 4-7 with DMD and whether there will be signs of reduced muscle damage and inflammation in MRI images. Part B is now underway and we are identifying families who may be interested in participating in the trial. Other measures such as physical function and muscle strength will also be assessed at visits about every four weeks over the 12 weeks of treatment; 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 (contact 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.**

MAKE COMMUNITY CONNECTIONS

At the CureDuchenne Cares Workshop—May 14, Waltham, MA and June 10 in Uniondale, NY. **The Cares** program educates core members of the patient's care team on the best standard of care for loved ones with DMD. One upcoming workshop for families and caregivers is being supported by Catabasis right in our backyard at the Westin Waltham, and another at the Long Island Marriott. Register at: http://cureduchennecares.org/event/waltham-ma-workshop-for-duchenne-families/ or at http://cureduchennecares.org/event/long-island-ny-duchenne-current-understanding-and-treatment/.

At the PPMD Connect Conference—June 26-29, Orlando, Florida. Parent Project Muscular Dystrophy's Annual Connect Conference is a unique convergence of industry partners, scientific leaders, medical providers, people living with Duchenne, and their families. This exceptional meeting has grown to be recognized worldwide, and Catabasis is pleased to announce that we will be presenting MoveDMD results there.



At the Jett Foundation's Regional Roundtables Series

- June 18, Columbus, Ohio
- August 27, Los Angeles, California

Partnering with fellow Duchenne organizations, clinicians, institutions, industry, and other experts, Jett Foundation forums examine issues and curate new information around clinical trial options currently underway or recently completed.

Part B of the MoveDMD trial has now begun. Part A showed positive safety, tolerability, pharmacokinetics and biomarker data. Five sites in the United States are anticipated to participate in Part B of the MoveDMD trial. The locations are: Los Angeles, California; Gainesville, Florida; Orlando, Florida; Portland, Oregon (recruiting) and Philadelphia, Pennsylvania. Travel costs for participants and their immediate families are being provided with support from the Muscular Dystrophy Association.

For more information on the MoveDMD trial or enrollment questions go to https://clinicaltrials.gov/ct2/show/NCT02439216 or contact us at 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). CAT-1004 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