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catabasis QUARTERLY

Updates on the MoveDMD trial

Catabasis news for you. This month's Catabasis Quarterly highlights recent data from the MoveDMD℠ trial and will be of special interest to parents, advocates and friends in the DMD community. This trial enrolled ambulatory boys between ages 4 and 7 who are affected with Duchenne across a range of dystrophin mutations. The oral therapy it is investigating—CAT-1004—may have disease-modifying potential and the ability to regenerate muscle in boys with DMD with any mutation type.

Positive results from the recently completed Part A of the MoveDMD trial.

We are pleased to announce new study data: Part A of the MoveDMD trial assessed the safety, tolerability and pharmacokinetics (PK) of CAT-1004. PK assessment observes the absorption of a medication in the body. The boys in this study had either never taken a steroid before or had not used steroids for at least six months prior to starting the trial. The study observed seven days of three dosing levels. Part A of the trial was conducted at three sites in the U.S.

- All three doses of CAT-1004 tested were generally well tolerated with no safety signals observed
- The majority of adverse events were mild, and the most common adverse events were gastrointestinal, primarily diarrhea
- There were no serious adverse events and no drug discontinuations
- PK results demonstrated CAT-1004 average plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-kB was observed
- Results support initiating Part B of the MoveDMD trial, expected to start in the first half of 2016

Now identifying patients for Part B. We are currently identifying patients who are interested in participating in Part B of the trial. Entry criteria are expected to be similar to those in Part A. Clinical trial sites are expected to be active in Oregon (Shriners Hospitals for Children); Florida (University of Florida); and Pennsylvania (The Children's Hospital of Philadelphia). Additional sites in the U.S. may be available. Travel funding will be available for participants and their immediate families. **For more details go to www.clinicaltrials.gov or contact joanne.donovan@catabasis.com.**

> "The unmet medical need in Duchenne is profound and potential therapies that could make a meaningful difference are needed. Showing positive safety, tolerability and pharmacokinetics results is an important milestone in the development of CAT-1004. I look forward to the advancement of this novel potential therapy."

—Richard Finkel, M.D., Division Chief, Division of Neurology, Department of Pediatrics at Nemours Children's Health System

KEEP PACE WITH THE MoveDMD TRIAL

Part A of the trial is now complete. We are grateful to the participants and their families as well as to the clinical trial site staff who have made this possible. And we greatly appreciate the enthusiasm and support we have received from the DMD community.

Part B is designed as a randomized, double-blind, placebo-controlled trial to evaluate the safety and efficacy of CAT-1004 in DMD over a 12-week period. We anticipate that there will be 4 visits to the clinical trial sites over the 12-week period. The boys who participated in Part A of the trial will be asked to participate in Part B, and additional participants will also be enrolled. **Please contact us if you are interested in participating in Part B of the trial. Travel, lodging and meal expenses for participants as well as their families will be covered.**

Eyes on CAT-1004

The science behind the MoveDMD trial: CAT-1004 is an investigational oral therapy designed to inhibit activated NF-kB, a protein that plays an important role in muscle health. It is being studied to see if it will reduce muscle damage and improve function in boys with DMD; CAT-1004 has the potential to reduce muscle inflammation and degeneration and promote muscle regeneration in all DMD boys. In Phase 1 clinical trials (where the drug was given to an initial group of adults), CAT-1004 inhibited activated NF-kB and was well tolerated with no observable safety concerns.

CAT-1004 moves forward: We are pleased to report to the community touched by Duchenne Muscular Dystrophy that the European Commission has recently granted *Orphan Medicinal Product Designation* to CAT-1004 for the treatment of DMD. We thank both PPMD and MDA for their support, including grant funding, that has helped us to progress CAT-1004 and the MoveDMD trial.

COMMUNITY CONNECTIONS

At the Action Duchenne 2015 International Conference— November 6-7, London, UK, where Catabasis presented the clinical development program for CAT-1004.

At TREAT-NMD—December 4-8, 2015; Washington, DC, the first time this conference was in the US, with a focus on orphan drug development.

COMING SOON IN 2016...

Muscular Dystrophy Association Clinical Conference—March 20-23, Arlington VA. Mark your calendar!

Jett Foundation Roundtables—January 30 in Princeton, NJ in partnership with Ryan's Quest; February 20 in Houston, TX; March in Tampa, FL in partnership with University of Florida, date TBD. We hope to see you!

For more details on the MoveDMD trial or to inquire about Part B of the trial, go to www.clinicaltrials.gov or contact joanne.donovan@catabasis.com. You can also download this newsletter at www.catabasis.com.

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Move DMD