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catabasis QUARTERLY

Updates on the MoveDMD trial

Catabasis news for you. If you have a child enrolled in the MoveDMD[SM] trial, or if you are considering participating, this newsletter will keep you in the loop on our efforts to develop CAT-1004, an investigational oral therapy that may have disease modifying potential and the ability to regenerate muscle in boys affected by Duchenne with any mutation type.

The MoveDMD trial is open for enrollment and boys from all over the country are participating, with sites in Oregon (Shriners Hospitals for Children); Florida (University of Florida); and planned in the near future, Pennsylvania (The Children's Hospital of Philadelphia). If you have a son affected by Duchenne with any mutation ages 4-7 and not taking steroids, he may be eligible for this study. Please contact us at joanne.donovan@catabasis.com to learn more.

A bit about Catabasis. We are a group driven to bring hope and life-changing therapies in rare diseases such as Duchenne muscular dystrophy (DMD). The people behind CAT-1004 at Catabasis know that no one will change this disease alone. Catabasis wants to work with parents, advocates, and the DMD community to make a difference by developing a medicine that improves the lives of all patients affected by Duchenne.

Keep pace with the MoveDMD trial

The MoveDMD trial is a 2-part clinical trial of CAT-1004 to assess its safety and whether it will reduce muscle damage and improve function in boys affected with Duchenne ages 4-7. Thanks to advocacy organizations such as Action Duchenne, CureDuchenne, the Jett Foundation, Muscular Dystrophy Association (MDA), and Parent Project Muscular Dystrophy (PPMD), awareness of the MoveDMD trial is catching on. If you are not already participating in this important trial, keep in mind that enrollment continues. For those already taking part in the MoveDMD trial, the good news is we're making progress. In the first part of the trial, there are three cohorts and we are currently scheduling eligible patients to participate in the third cohort. And we're here to help make trial participation more manageable with travel funding, supported by PPMD, available for participants and their immediate families. We also have logistical support available through the travel agency Colpitts.

> "We had a great experience with Colpitts World Travel. I had a lot of questions and they answered them right away, even on weekends. It was a very smooth process and easy to handle. They were flexible to our needs. They were very professional and I would love to work with them again."
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> —Parent of a child participating in the MoveDMD trial

EYES ON CAT-1004

The science behind the MoveDMD trial: CAT-1004 is designed to inhibit activated NF-kB, a protein that plays an important role in muscle health. Inhibiting activated NF-kB is being studied to see if it will reduce muscle damage and improve function in boys affected by Duchenne; CAT-1004 has the potential to reduce muscle inflammation and degeneration and promote muscle regeneration in DMD. 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.

Catch the latest on CAT-1004: The U.S. Food and Drug Administration (the FDA) recently granted *Orphan Drug, Fast Track and Rare Pediatric Disease Designations* to CAT-1004 for the treatment of DMD. This means that there is strong recognition of the need to develop therapies for DMD as quickly as possible.

COMMUNITY CONNECTIONS

At the Parent Project Muscular Dystrophy Conference, Washington DC, June 18-21—PPMD's 21st Connect Conference. This year Catabasis again joined other presenters to showcase the many ways that PPMD's rallying cry, "We see strength," is alive and well.

At the Jett Foundation's 2015 Duchenne Roundtables— Catabasis was there, at UCLA in Los Angeles on August 22nd; in Boston on August 29th; and most recently, in Pittsburgh on September 12th where parents, experts and industry representatives discussed the regulatory process and the latest clinical trials.

At the 20th International World Muscle Society Congress— September 30 to October 4, Brighton, UK, where experts in DMD convened and new information in the field was presented. During the October 2 session, Catabasis was proud to present a poster about the MoveDMD trial design.

COMING SOON...

The Action Duchenne 2015 International Conference— November 6-7, London, UK...we hope to see some of you there!

For more information on the MoveDMD 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