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catabasis QUARTERLY

Updates on edasalonexent and the MoveDMD® trial

Catabasis news for you. The MoveDMD trial with edasalonexent (CAT-1004) in Duchenne muscular dystrophy (DMD) has just completed Part B, and Catabasis is releasing the top-line results. For participating families in this trial, we know you will have questions about what this news means for your son. The open-label extension study will continue to further evaluate the potential treatment effects of edasalonexent. Your principal investigator (study doctor) can provide you with more information and answer any questions you may have. Edasalonexent is an oral investigational drug that has not yet been approved by the US Food and Drug Administration.

Results for Part B of the MoveDMD trial

We are disappointed to share that Part B of the MoveDMD trial did not meet its primary endpoint, which was to demonstrate a statistically significant decrease in T2 MRI with edasalonexent after 12 weeks compared to placebo in boys affected by Duchenne. MRI T2 measures muscle inflammation. Edasalonexent was well tolerated with no safety signals observed. We did observe some potential treatment-associated effects at 12 weeks in the 100 mg/kg/day treatment group in both the timed function tests and North Star Ambulatory Assessment when compared to placebo. Therefore we will look to see if the signals strengthen in the longer-term data from the ongoing open-label extension. Today's results are the top-line statistical analysis results, and we plan to complete a comprehensive analysis to fully understand the effects of edasalonexent. We will also share additional results after our full assessment. Above all, we remain grateful to the boys and their families as well as the clinical trial staff who are making this trial possible. For more information and continued updates, visit us at **catabasis.com**.

MoveDMD OPEN-LABEL EXTENSION (PART C) CONTINUES

The open-label extension is currently ongoing and at this time there are no changes to the extension study visit schedule. In addition to safety monitoring, MRI, timed function tests (10-meter walk/run, 4-stair climb and time to stand) and the North Star Ambulatory Assessment, muscle strength measures and the pediatric outcomes data collection instrument (PODCI) are also being collected in the open-label extension. Results from the open-label extension portion of the MoveDMD trial will be shared periodically throughout 2017.

We will assess the Part C open-label extension data as it becomes available and determine next steps for edasalonexent in DMD. If you are interested in possible future clinical trials with edasalonexent, please join our mailing list: **http://www.catabasis.com/patients-families/for-further-information.php**.

RECENT PUBLICATIONS

Two recent scientific publications have additional information on the effects of edasalonexent:

- Edasalonexent program preclinical results published in *JCI Insight* **https://www.ncbi.nlm.nih.gov/pubmed/28018975**
- Phase 1 data on edasalonexent in adult subjects, which informed on the dose and dose schedule for the current MoveDMD trial, published in *The Journal of Clinical Pharmacology* **https://www.ncbi.nlm.nih.gov/pubmed/28074489**

Coming up: we anticipate announcing a webinar with PPMD soon that will focus on the Part B MoveDMD results.

For more information and 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