Issue 7 | April 2017

catabasis QUARTERLY

Updates on edasalonexent and the MoveDMD® trial

Catabasis news for you. Parts A and B of the MoveDMD trial with edasalonexent (CAT-1004) in Duchenne muscular dystrophy (DMD or Duchenne) are complete. Part C is ongoing as we learn more about edasalonexent 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. For more information, visit us at **catabasis.com**.

Additional results for Part B of the MoveDMD trial

We are reassured by recently reported safety, tolerability and plasma exposure data for edasalonexent in patients with DMD from Part B of the MoveDMD trial. While the MRI T2 composite endpoint at 12 weeks in Part B was not achieved, we are encouraged that all 6 function-associated exploratory endpoints assessed in Part B showed numerical improvement in the higher dose group compared to placebo although the changes were not statistically significant. This includes the 4 function-associated endpoints we presented at the end of January—the 4-stair climb, 10-meter walk/run, time to stand and the North Star Ambulatory Assessment—as well as 2 additional exploratory endpoints, pediatric outcomes data collection instrument (PODCI) and muscle strength, for which we recently obtained data. We are hopeful that extended exposure in Part C will provide further evidence of functional benefits.

Update on Part C of the MoveDMD trial

At this point, the majority of the boys have been on edasalonexent for at least 24 weeks. We are extending Part C of the MoveDMD trial for an additional 24 weeks beyond the original 36-week, open-label extension. This way, the boys that started the open-label extension in July of last year can continue to receive edasalonexent treatment. Also, we are transitioning the boys in the lower dose group of 67 mg/kg/day in Part C to the 100 mg/kg/day dose so we can assess the higher dose in all Part C participants. Importantly, safety signals were not observed in Part B in either the 67 or the 100 mg/kg/day treatment groups.

PODCI ASSESSES INFORMATION FROM PARENTS ABOUT MUSCLE FUNCTION IN EVERYDAY SITUATIONS

The PODCI is a questionnaire for parents that asks about their observations of their son's daily activities. Based on questions designed to gather information, the data are analyzed to understand six different areas: upper extremity function, basic transfers and mobility, sports and physical function, comfort and pain, and happiness and satisfaction, as well as a global composite score. We focused on the basic transfers and mobility scale, which has been shown to correlate with disease progression in Duchenne and loss of milestones. Numerical improvements in this scale were observed in both edasalonexent treatment groups compared to placebo in Part B. We are continuing to perform the same assessments from Part B, including PODCI, in Part C.

MAKING COMMUNITY CONNECTIONS

At the 2017 MDA Scientific Conference—March 19-22 in Arlington, VA. Here, academics, industry professionals, clinicians and government representatives discussed the latest therapeutic targets and technologies with the aim to accelerate drug development for neuromuscular diseases. Catabasis' presentation included the Part B data for PODCI and muscle strength, which showed numerical improvement versus placebo for both edasalonexent treatment groups at 12 weeks. Those materials are available **here** and **here**.

At the Jett Foundation Regional Roundtable Series—April 2 in Nashville, TN and May 6 in St. Louis, MO. Partnering with Duchenne organizations, clinicians, institutions, industry, and other experts, these forums examine issues and curate information around treatment options, recently completed trials, and those underway for families affected by DMD.

At the 2017 PPMD Annual Connect Conference—June 29 to July 2 in Chicago, IL. This event is a unique convergence of industry partners, scientific leaders, medical providers, people living with Duchenne, and their families and we look forward to participating.

Part C (the open-label extension) of the MoveDMD trial is ongoing. During Part C of the trial, boys are receiving edasalonexent for 60 weeks in addition to what they received in Parts A and B. Safety is being monitored as well as MRI, timed function tests, muscle strength measures, the North Star Ambulatory Assessment and PODCI. We plan to share results from Part C of the MoveDMD trial periodically throughout the year with an interim update in Q3.

We will assess the Part C 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**.

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