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catabasis QUARTERLY

Updates on edasalonexent and the MoveDMD® trial

Catabasis news for you. The placebo-controlled portion of Phase 2 of the MoveDMD trial with edasalonexent (CAT-1004) in Duchenne muscular dystrophy (DMD or Duchenne) is complete. The open-label extension is ongoing and continues to generate learnings about edasalonexent in boys with DMD regardless of mutation. 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**.

Multiple improvements in assessments of muscle function seen in edasalonexent-treated boys in the Phase 2 MoveDMD trial

In Phase 2 of the MoveDMD trial, clinically meaningful improvements were observed following 12 weeks of edasalonexent treatment in well-established, pre-specified assessments of muscle function: 4-stair climb, 10-meter walk/run, time to stand, North Star Ambulatory Assessment (NSAA) and the Pediatric Outcomes Data Collection Instrument (PODCI). These improvements are seen both in the placebo-controlled comparison as well as a crossover analysis, summarized in the table on the right. The crossover analysis compared changes during an off-treatment period to those during edasalonexent treatment for boys who were also in Phase 1 of the trial. The MoveDMD trial was not powered for functional assessments and these analyses were generally not statistically significant. Edasalonexent was well tolerated, with no safety signals in this study.

In the 12-week placebo-controlled results we saw positive trends in MRI T2 composite of the 5 lower leg muscles, consistent with edasalonexent reducing inflammation, which did not reach statistical significance. In the crossover analysis, we saw a positive difference in the MRI T2 rate of change for boys on edasalonexent, which reached statistical significance. These data improve our understanding of MRI and will be helpful in future trials in Duchenne.

	Placebo-Controlled^	Crossover
	Edasalonexent 100 mg/kg/day (n=10)	Edasalonexent Pooled (n=12)
10-meter walk/run	+	+
4-stair climb	+	+
Time to stand	+	+
NSAA	+	+
PODCI	+	+*
MRI T2	+	+*

*p <0.05

\+ Indicates numerical improvement with edasalonexent compared to placebo or off-treatment period.

^ Reflects open-label extension dose. To see full results for placebo-controlled analysis, please go to www.catabasis.com.

We are enthusiastic about the functional results, as functional assessments are what is important both to those affected by Duchenne as well as the future clinical trial program with edasalonexent. Phase 2 of the MoveDMD trial includes functional assessments that have been endpoints in pivotal trials in DMD. We believe that the MoveDMD 12-week Phase 2 clinical results are consistent with the goal of treatment, which is to delay the predictable, sequential loss of function in Duchenne.

UPDATE ON THE OPEN-LABEL EXTENSION OF THE MoveDMD TRIAL

All participants in the open-label extension are now on the 100 mg/kg/day dose. Pending IRB approval, we plan to extend the open-label extension for an additional year. The first eligible MoveDMD trial participant has begun treatment with EXONDYS 51™ while continuing to receive edasalonexent as part of the open-label extension.

We expect to share 24-week results from the open-label extension of the MoveDMD trial in Q3 as well as announce Phase 3 clinical trial plans later this year.

NORTH STAR AMBULATORY ASSESSMENT INCORPORATES MULTIPLE ACTIVITIES

The NSAA score is a combination of 17 physical function tests with increasing difficulty. The activities tested range from the ability to stand or walk; to the ability to run or hop on one leg. Each activity is assessed by the clinical evaluator as: "perform", "perform with difficulty", or "unable to perform". In the 12-week Phase 2 trial, numerical improvements with edasalonexent treatment compared to placebo were observed in NSAA. We are continuing to perform the same assessments from the 12-week placebo-controlled Phase 2, including NSAA, in the open-label extension.

MAKING COMMUNITY CONNECTIONS

At the 2017 PPMD Annual Connect Conference—June 29 to July 2 in Chicago, IL. We appreciated the opportunity to present, participate and talk with attendees at this convergence of families and people affected by Duchenne, industry partners, scientific leaders, and medical providers.

At the upcoming 15th Action Duchenne International Conference—November 10-12 in Birmingham, U.K. Here, at the preeminent Duchenne & Becker muscular dystrophy event in the UK, families and supporters meet and learn about the condition from international experts and from other families and those living with Duchenne. For academics and clinicians, this conference provides the opportunity to discuss the latest findings in genetic research and to share expertise in optimal standards of care.

Catabasis is proud to support World Duchenne Awareness Day coming up on September 7, 2017.

The open-label extension of the MoveDMD trial is ongoing. Safety is being monitored as well as assessments of muscle function. We plan to share results from 24 weeks of edasalonexent treatment in the open-label extension of the MoveDMD trial in Q3.

We expect to announce our Phase 3 trial plan with edasalonexent in DMD later this year. The Phase 3 trial is expected to start in 2018. If you have questions about edasalonexent or are interested in the anticipated Phase 3 trial, contact our clinical team at **DMDtrials@catabasis.com**. If you would like to stay up to date on edasalonexent developments, please join our mailing list: **http://www.catabasis.com/patients-families/for-further-information.php**.

For more information on the MoveDMD trial, visit https://clinicaltrials.gov/ct2/show/NCT02439216. You can also download this newsletter from our website 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