Baseline Characteristics of Patients Enrolled in PolarisDMD, a Phase 3 Trial of Edasalonexent for Duchenne Muscular Dystrophy

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Results

Study Design

MoveDMD®, a Phase 1/2 Trial with Open-Label Extension

Results

Reproducibility of Additional Functional Measures Between Screening and Baseline

Phase 3 PolarisDMD and Phase 2 MoveDMD Trials Have Similar Baseline Characteristics

Conclusions

With expansion from a US only trial to a global trial in 8 countries, both the Phase 2 MoveDMD and the Phase 3 PolarisDMD trial enrolled a similar population, without significant difference in baseline age or functional measures.

- NAA and timed function tests were highly reproducible in repeat measures at Screening and Baseline in boys as young as 4.
- As expected with the entry criteria, mean time to stand velocity was numerically faster in the Phase 3 trial. Distribution of baseline NAA and trend function tests were not variable in the Phase 3 trial from the Phase 2 trial.
- These findings support the design of the Phase 3 PolarisDMD trial in young boys regardless of mutation. Results are expected in 2020.

NSAA Reproducible Between Screening and Baseline Visits in Young Boys in PolarisDMD

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Presented at the 2020 MDA Clinical & Scientific Conference

Acknowledgements

- Patients and families
- Patient groups
- MoveDMD Phase 3 Site staff
- MoveDMD Phase 2 Site staff
- Catabasis team
- Thanks to PPMD and MDA for generous grant support for patient travel in the MoveDMD Phase 2 trial

Study Objectives

- Safety and PK in pediatric patients with DMD
- Proof-of-concept using MBB to assess changes in muscle health

Edasalonexent inhibits Nf-κB, A Key Driver of Muscle Disease in DMD

- Edasalonexent is a highly potent, orally administered small molecule that inhibits NF-κB activation through the NF-κB p105/p50 signalosome
- Edasalonexent has a unique pharmacodynamic profile and is an effective inhibitor of NF-κB in muscle

Background

Activation of NF-κB in Duchenne Muscular Dystrophy Is a Key Factor in Disease Progression

- NF-κB activation is a hallmark of Duchenne muscular dystrophy
- NF-κB activation contributes to muscle inflammation and fibrosis

Study Population

- Age: 4 to 7 years old
- Catabasis Pharmaceuticals, Boston, MA

Entry Criteria of Phase 2 MoveDMD and Phase 3 PolarisDMD Trials Were Similar

- Age 4 to 7 years old
- Catabasis Pharmaceuticals, Boston, MA

Conclusions

- NAA and timed function tests were highly reproducible in the Phase 3 PolarisDMD study population
- NSAA was more reproducible than the timed function tests in this population

Results

Physical Function Outcomes

- NAA 18 month Baseline
- NAA 18 month Follow-up
- 10 Meter Walk/Run speed (m/s)
- 4 Seat CLamp (g)
- Time to Stand (s)

Distribution of Functional Measures at Baseline Was Similar in MoveDMD and PolarisDMD Trials

- NAA: baseline NAA, and speeds of timed function tests (time to stand, 4-seat clamp, and 10 meter walk/run) were similar in both studies, shown difference in all measures between treatments. All measurements were performed by Kolehmainen-Smieles and colleagues from the Pediatric Bone Density Lab at the University of Pennsylvania.

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