MoveDMD®: Phase 2 Trial of Edasalonexent, an NF-κB Inhibitor, in 4 to 7-Year Old Patients with Duchenne Muscular Dystrophy

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Background & Study Design

Objectives: To evaluate the safety and tolerability of edasalonexent in 4-7-year-old patients with Duchenne muscular dystrophy (DMD). To assess the effect of edasalonexent on muscle function and wasting.

Study Design: Randomized, double-blind, placebo-controlled Phase 2 trial with a 12-month treatment period followed by a 4-month post-treatment follow-up.

Primary Endpoints:
- Muscle function - 6-minute walk test
- Muscle wasting - MRI muscle cross-sectional area

Secondary Outcomes:
- Safety and tolerability
- Muscle strength
- Quality of life

Results

Safety & Tolerability

- No safety signals in MoveDMD® trial to date
- Well-tolerated, with majority of adverse events judged to be mild
- Most adverse events were related to study medications
- No severe events

Summary

Safety & Tolerability

- Summary: Edasalonexent substantially slowed disease progression in DMD aged 4-7.

Phase 3 Plans

Hepatitis B Virus (HBV) Vaccine Phase 3 Registration Trial for Edasalonexent

Conclusions

- Edasalonexent: Potential to Slow Disease Progression for All Ages Affected by Dystrophin
- Inhibits endoplasmic reticulum stress-mitigating agents for treatment of DMD, regardless of mutation type.
- Edasalonexent is being developed for patients who do not respond to current therapies.