MoveDMD Results: Effects of Edasalonexent, an NF-κB Inhibitor, in 4 to 7 Year Old Patients with Duchenne Muscular Dystrophy

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Background and Objectives

- Evaluate the safety and tolerability of edasalonexent, an NF-κB inhibitor, in 4 to 7 year old patients with Duchenne Muscular Dystrophy (DMD).
- Evaluate the efficacy of edasalonexent on muscle strength, function, and disease progression.

Part B Design

- Study design: Randomized, double-blind, placebo-controlled trial.
- Duration: 2 years (24 months).
- Participants: 4 to 7 year old boys with DMD.
- Treatment: Edasalonexent or placebo.

Part A Results

- No significant change in muscle strength, function, or disease progression observed.
- Changes in 6MWD and 2MWT were not statistically significant.

Part B Results

- Significant improvements in muscle strength and function observed.
- Changes in 6MWD and 2MWT were statistically significant.

Summary

- Edasalonexent is safe and well-tolerated in young children with DMD.
- Efficacy results suggest potential benefit in muscle strength and function.

Acknowledgments

- Patients and Families
- Patient groups
- Imaging/DMD Staff
- Catabasis team

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