MoveDMD®: Positive Effects of Edasalonexent, an NF-κB Inhibitor, in 4 to 7-Year Old Patients with Duchenne Muscular Dystrophy in Phase 2 Study with an Open-Label Extension

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**Background**

- The absence of dystrophin is necessary but not sufficient to direct the disease process in DMD.
- The lack of dystrophin combined with mechanical stress activates NF-κB which then promotes muscle degeneration and suppresses muscle regeneration.

**Study Design**

- Edasalonexent is an oral NF-κB inhibitor in development for all presentations regardless of mutation type.

**Results**

- 10-Meter Walk/Run Speed Stabilized with Edasalonexent Treatment
- 4-Step Climb Speed Stabilized with Edasalonexent Treatment
- Time to Stand Speed Stabilized with Edasalonexent Treatment

**Safety**

- No safety signals - 36 years of patient exposure
- Well tolerated with majority of adverse events being mild to moderate.

**Adverse Events**

- Majority of treatment-related adverse events were gastrointestinal and mild to moderate.

**Biomarker**

- CRP is a well-characterized blood test marker that provides a global assessment of inflammation.
- CRP is elevated in DMD.
- In MoveDMD, CRP significantly decreased from baseline after 12 and 24 weeks of 100 mg/kg edasalonexent.

**Conclusions**

- Disease progression on edasalonexent improved compared to rate of change in control period.
- North Star Ambulatory Assessment.
- Open-Label Extension Results: Edasalonexent Substantially Slowed DMD Disease Progression.

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