Background

**Edasalonexent: Potential Disease-Modifying Oral Therapy for Duchenne Muscular Dystrophy**
- Potential disease-modifying agent across age groups and mutations
- Orphan drug for treating a deadly, incurable DMD pathophysiology
- Positive effects on muscle, respiratory, and cardiac functions observed in preclinical studies

**Part A: Efficacy**
- NF-κB activity assessed in Phase 1 clinical trials in adults and boys with DMD
- Phase 2a/3b clinical ongoing

**Inhibition of NF-κB Prolongs Disease-Modifying Effects in Duchenne Muscular Dystrophy**
- NF-kB activity is intracellularly activated in DMD due to loss of function of the dystrophin protein
- NF-kB activity is inversely correlated with disease severity and progression
- NF-kB-mediated translation involves multiple signaling pathways
- NF-kB inhibitors can be used to modulate NF-kB activity

**Activated NF-κB - Not Just Absence of Dystrophy – Plays a Central Role in DMD Pathophysiology**
- NF-κB activation is associated with disease progression in DMD
- NF-κB inhibition can lead to improved muscle function and reduced disease severity

Disclosures:
- * Edasalonexent (CAT-1004) is an investigational agent being developed for the treatment of Duchenne muscular dystrophy, and not approved for treatment.
- Dr. Joanne Donovan is an employee of Catabasis Pharmaceuticals, Inc.

Part A Results

**Edasalonexent (CAT-1004) Initiates NF-κB and Shows Disease-Modifying Effects in DMD Preclinical Models**
- NF-κB activity assessed in preclinical models
- Edasalonexent (CAT-1004) reduces NF-κB activity in muscle, reduces muscle pathological changes, and improves muscle function

**Edasalonexent (CAT-1004) Initiation in Boys with Duchenne Muscular Dystrophy**
- Part A results of a 12-month phase 2 clinical trial in boys with DMD
- Edasalonexent inhibits NF-κB activity in muscle, reduces muscle pathological changes, and improves muscle function

**MoveDMD Study Population**
- Initial approach to assess safety, pharmacokinetics and efficacy of edasalonexent in boys with DMD
- Design: A Phase 1 dose escalation study followed by a Phase 2a/3b dose verification study
- Safety: Assess edasalonexent safety profile in boys with DMD
- Efficacy: Assess edasalonexent efficacy in boys with DMD

**MoveDMD Endpoints: Assessments of Disease**
- T1-weighted muscle imaging: Baseline to month 12
- Short-term EF: Baseline to month 12
- Activity: Assess edasalonexent activity in boys with DMD
- Efficacy: Assess edasalonexent efficacy in boys with DMD

**Part B Design**
- Randomized, double-blind, placebo-controlled, multinational, multi-center trial
- Participants: Efficacy: Boys aged 4-6 years, Baseline DMD MFM-200 score \\* Initial and final DMD MFM-200 score difference ≥ 6
- Safety: Boys aged 12-15 years, Baseline DMD MFM-200 score \\* Initial and final DMD MFM-200 score difference ≥ 6
- Duration: 2 years
- Primary outcome: Change in DMD MFM-200 score from baseline to month 12
- Secondary outcomes: Change in DMD MFM-200 score from baseline to month 24

Design of MoveDMD Part B

Summary

**Emphasized**
- Evaluate efficacy in boys aged 4-6 years
- Evaluate safety in boys aged 12-15 years

**Disclosures**
- Catabasis Pharmaceuticals
- University of Florida
- Oregon Health & Science University
- Children’s Hospital of Philadelphia
- Nemours Children’s Hospital, Orlando

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- Myotonic Muscular Dystrophy Association
- National MD Alliance
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- MDA MD Award: $10,000
- MDA Fund: $10,000
- MDA Grant: $10,000

**References**
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