MoveDMD®: Clinical Trial of Edasalonexent (CAT-1004) in Boys with Duchenne Muscular Dystrophy

February 2017

Forward Looking Statements

This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, including statements regarding our expectations and beliefs about our business, future financial and operating performance, clinical trial plans, product development plans and prospects. The words "believe", "anticipate", "plans," "expect", "could", "should", "will", "would", "may", "intend" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements contained in this presentation and in remarks made during this presentation and the following Q&A session are subject to important risks and uncertainties that may cause actual events or results to differ materially from our current expectations and beliefs, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development of our product candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products; availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements; other matters that could affect the availability or commercial potential of our product candidates; and general economic and market conditions. These and other risks are described under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, which is on file with the Securities and Exchange Commission, and in other filings that we may make with the Securities and Exchange Commission in the future.

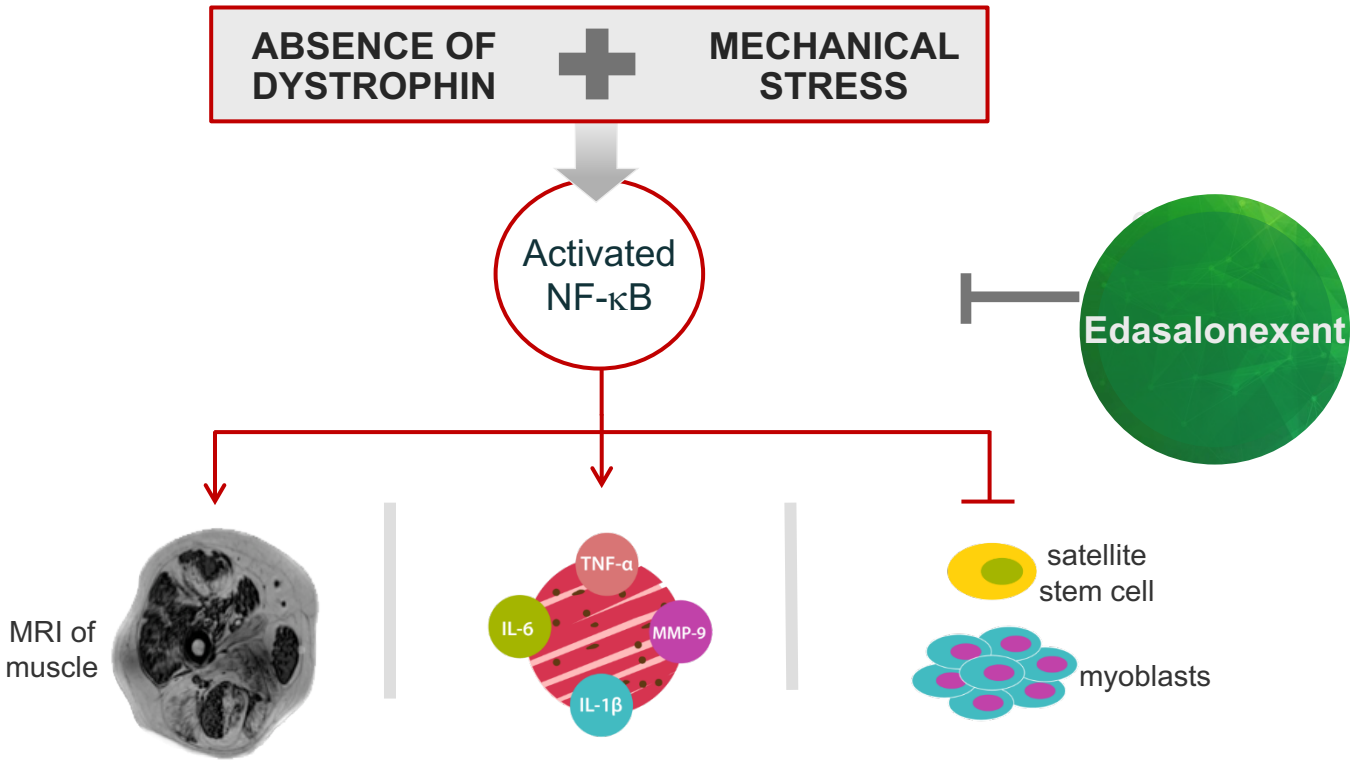
In addition, the forward-looking statements included in this presentation represent our views as of the date of this presentation. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we specifically disclaim any obligation to do so. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this presentation.

Edasalonexent (CAT-1004), an Investigational Drug Candidate Being Developed for DMD

- **Why Edasalonexent?**
  - In Duchenne, lack of dystrophin and mechanical stress activate NF-κB in muscles, leading to muscle degeneration, inflammation, fibrosis and inhibition of muscle regeneration and ultimately loss of function
  - Steroids suppress inflammation but have significant side effects
  - Phase 1 studies in adults showed no safety signals and that edasalonexent targets NF-κB
- **Catabasis is conducting the MoveDMD study to understand the effects of edasalonexent in young boys with Duchenne**

Move DMD

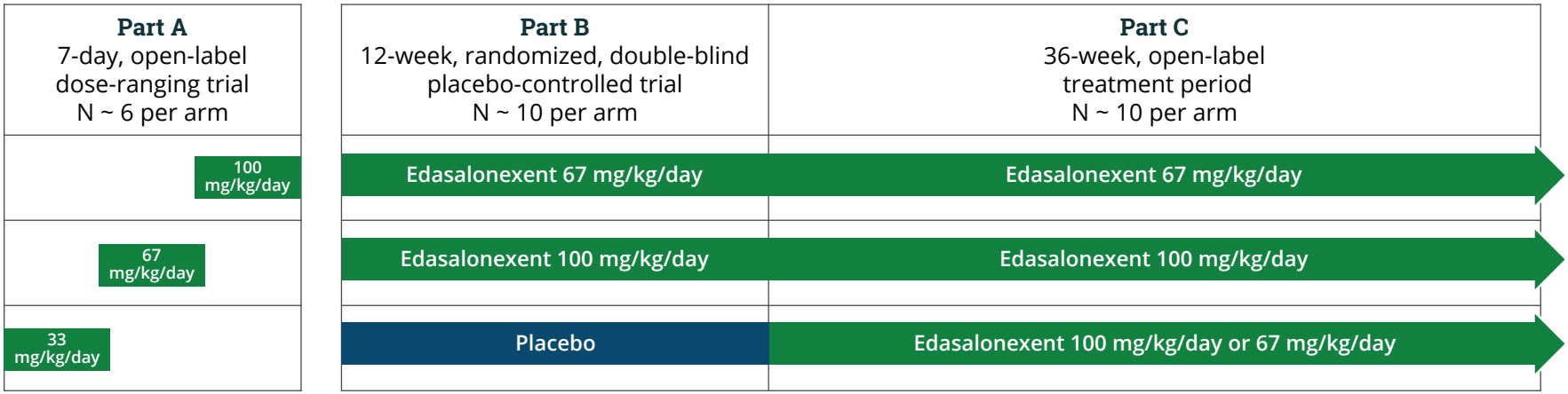
Edasalonexent Inhibits NF-κB and Shows Disease-Modifying Effects in DMD Models



Edasalonexent pre-clinical studies	Reduced muscle degeneration	Reduced inflammation and fibrosis	Enhanced muscle regeneration
	✓	✓	✓

MoveDMD Trial Design

Study Population: All DMD mutations, ages 4 – 7, steroid naïve or off steroids for ≥6 months

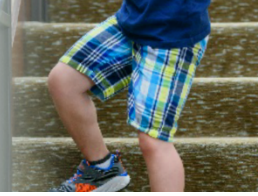


- Assess the safety and PK of edasalonexent in ~18 boys with Duchenne ages 4-7
- Showed positive PK, NF-kB biomarker effects, safety and tolerability

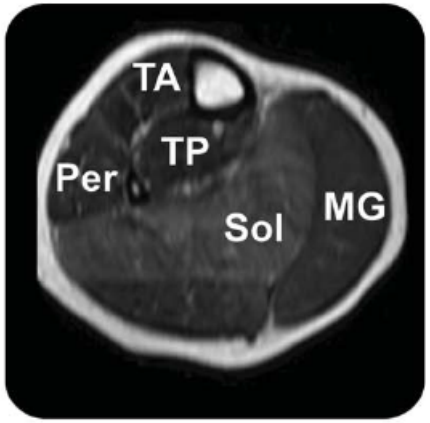
- Assess the safety and efficacy of edasalonexent versus placebo using MRI as an early biomarker; trial was powered only for the primary end point of change from baseline in MRI T2 of composite of lower leg muscles
- Other measures: timed function tests (10-meter walk/run, 4-stair climb, time to stand), NSAA, muscle strength, PODCI

- Measure the same safety and efficacy parameters as in Part B of the trial to assess treatment effects over a longer time

MoveDMD Part B
Key Efficacy Endpoints



- **Primary**
  - MRI Change from baseline to Week 12 in the lower leg composite of T2 relaxation time



- **Secondary:**
  - Timed function tests (TFT) speed and times for
    - completing the 10-meter walk/run (10MWT)
    - climbing 4 stairs (4SC)
    - standing from supine (time to stand: TTS)
  - North Star Ambulatory Assessment (NSAA)
  - Muscle strength testing
  - Pediatric outcomes data collection instrument (PODCI)
  - MRI/MRS fat fraction and correlation to functional status

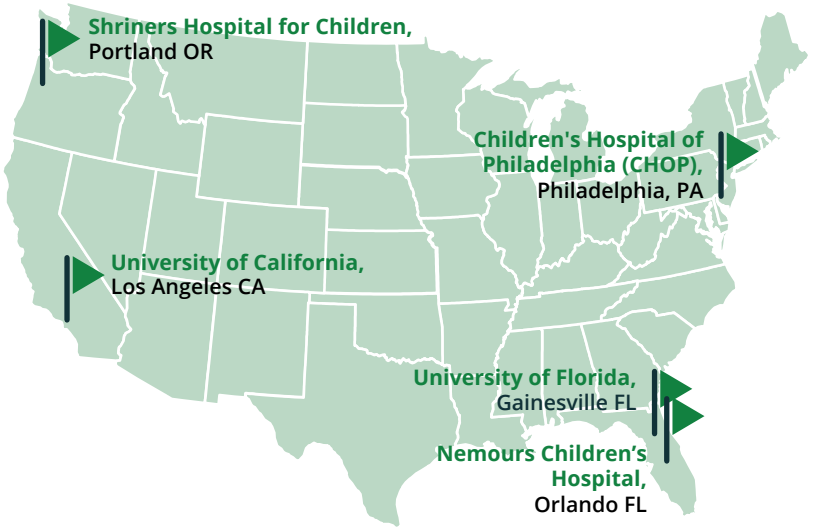
MoveDMD Part B Results
Study Metrics and Baseline Characteristics

- Enrolled total of 31 boys at 5 sites for Part B of the trial, 16 of whom also participated in Part A

- All 31 boys who enrolled completed the trial

- There were no missed visits and all boys completed the planned MRIs and other assessments

- All boys were able to take the capsules during the trial



Baseline Demographics and Values

Treatment Group	Placebo	Edasalonexent 67 mg/kg/day	Edasalonexent 100 mg/kg/day	Overall Edasalonexent
	(n =11)	(n =10)	(n =10)	(n =20)
Age at Week 0 (years)[1]	6.3	6.0	6.0	6.0
Age at Symptom Onset (years)[2]	3.7	3.0	2.0	2.5
Age at Diagnosis (years) [2]	4.6	3.5	3.0	3.3
Weight at randomization (kg)	21.4	22.1	22.0	22.1
10-meter walk/run (10MWR in seconds)[1]	6.9	6.3	6.8	6.6
4-stair climb (4SC in seconds)[2]	5.0	4.5	6.3	5.4
Time to stand (TTS in seconds)[2]	6.5	7.0	12.0	9.4

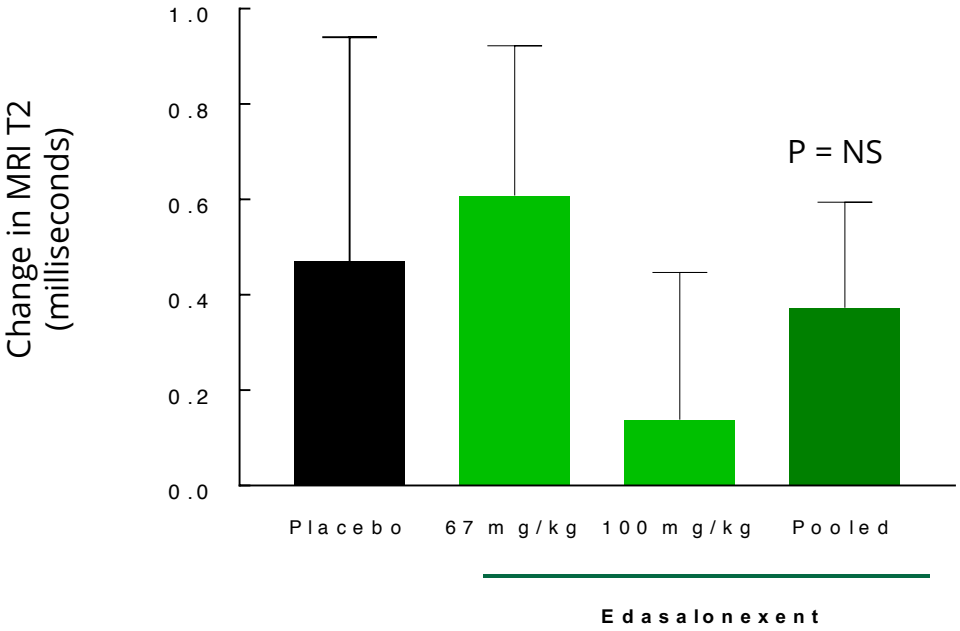
Values shown are means
Patients were all male and steroid-naive and predominantly Caucasian
For context, mean times for timed function tests in normal boys of similar age as those in the MoveDMD trial are 10MWR: 3.4 seconds; 4-stair climb: 1.4 seconds; and TTS: 2.1 seconds (ImagingDMD data presented at Catabasis Investor Day Nov 2016)

[1]Patient randomization was stratified for baseline age and 10-meter walk/run

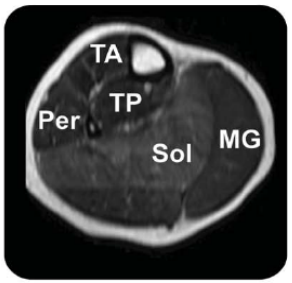
[2]Patients in the edasalonexent 100 mg/kg/day group were symptomatic at a younger age and did not perform as well on the 4-stair climb and the time to stand function tests at baseline; characteristics consistent with more advanced disease

MoveDMD Trial Part B Results
Primary Efficacy End Point

Change in MRI T2 from Baseline to Week 12 in Composite of 5 Lower Leg Muscles



Smaller increase in MRI T2 correlates with less muscle inflammation

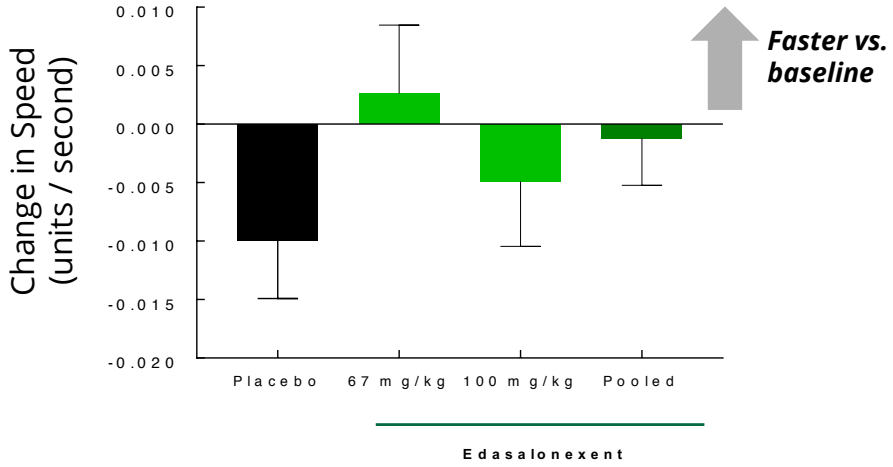


- No significant change in the primary end point, average change from baseline to Week 12 in the MRI T2 measure for a composite of lower leg muscles for the pooled edasalonexent treatment groups vs. placebo.
- Primary end point numerically better for edasalonexent 100 mg/kg/day vs. placebo, although not statistically significant.

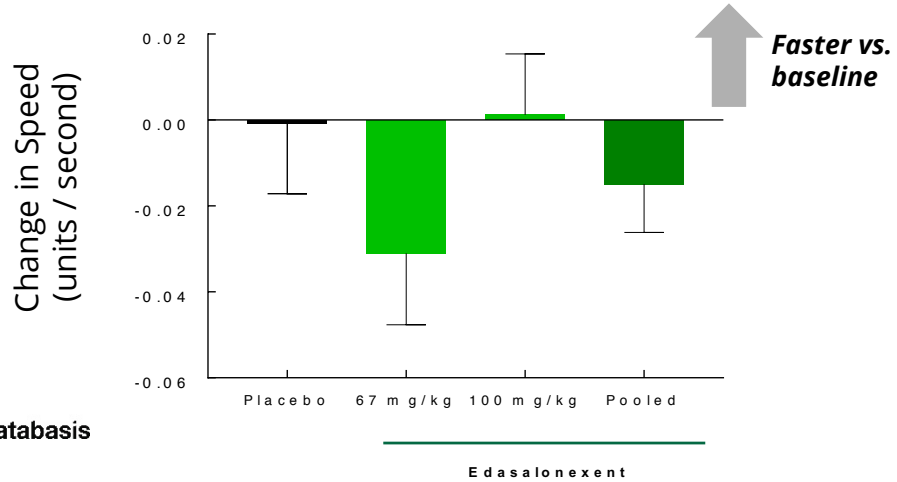
Error bars in chart denote SEM

10-meter Walk/Run Speed and Time to Stand Speed

Change in 10-meter Walk/Run Speed from Baseline to Week 12



Change in Time to Stand Speed from Baseline to Week 12

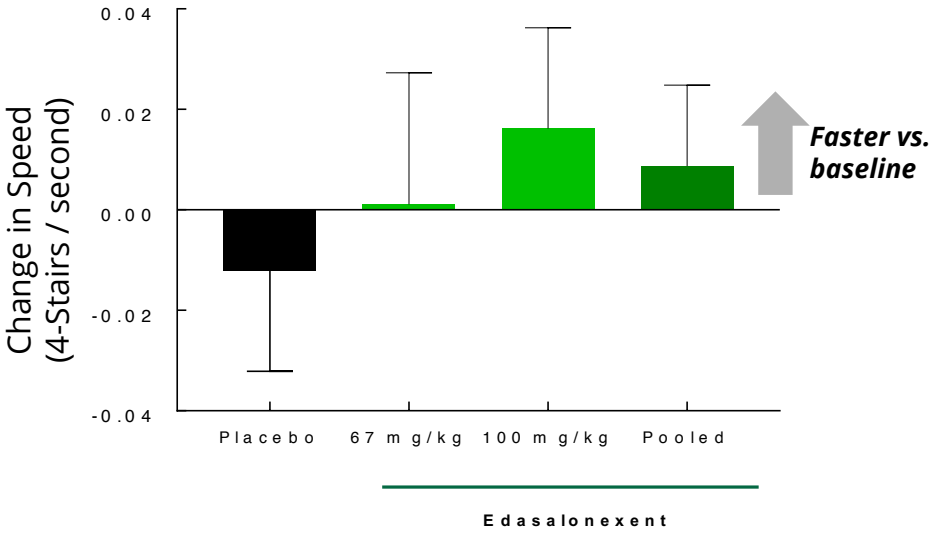


- Change in timed function test Speed at Week 12 was a pre-defined end point
- Speed is the reciprocal of the time to perform the function test. In contrast to Time, Speed allows for accounting for boys who are unable to perform tests
- Change in 10-meter walk/run Speed and change in time to stand Speed numerically better for edasalonexent 100 mg/kg/day vs. placebo although neither was statistically significant

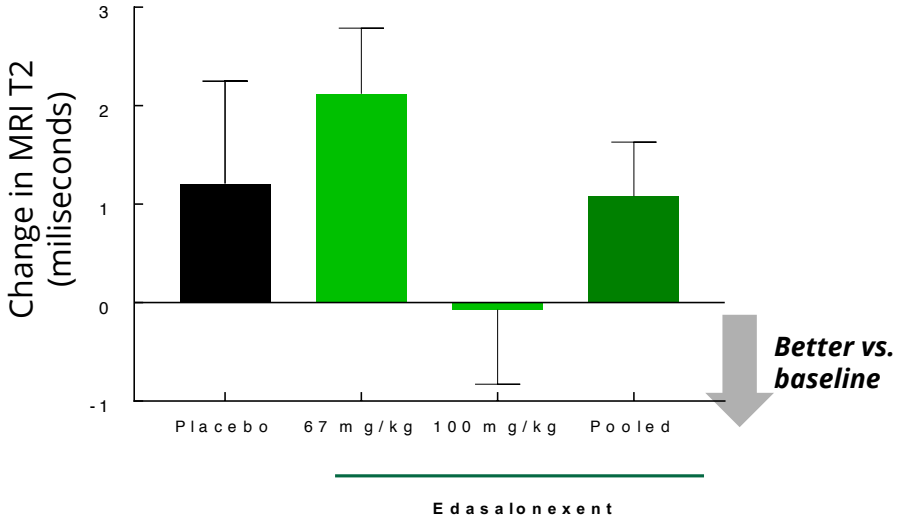
Error bars in chart denote SEM

4-Stair Climb Speed and Change in MRI T2 of Vastus Lateralis (Upper Leg Muscle Enlisted in 4-Stair Climb)

Change in 4-Stair Climb Speed from Baseline to Week 12



Change in MRI T2 of Vastus Lateralis from Baseline to Week 12



- Change in 4-stair climb Speed and change in MRI T2 of vastus lateralis numerically better for edasalonexent 100 mg/kg/day vs. placebo, although not statistically significant.

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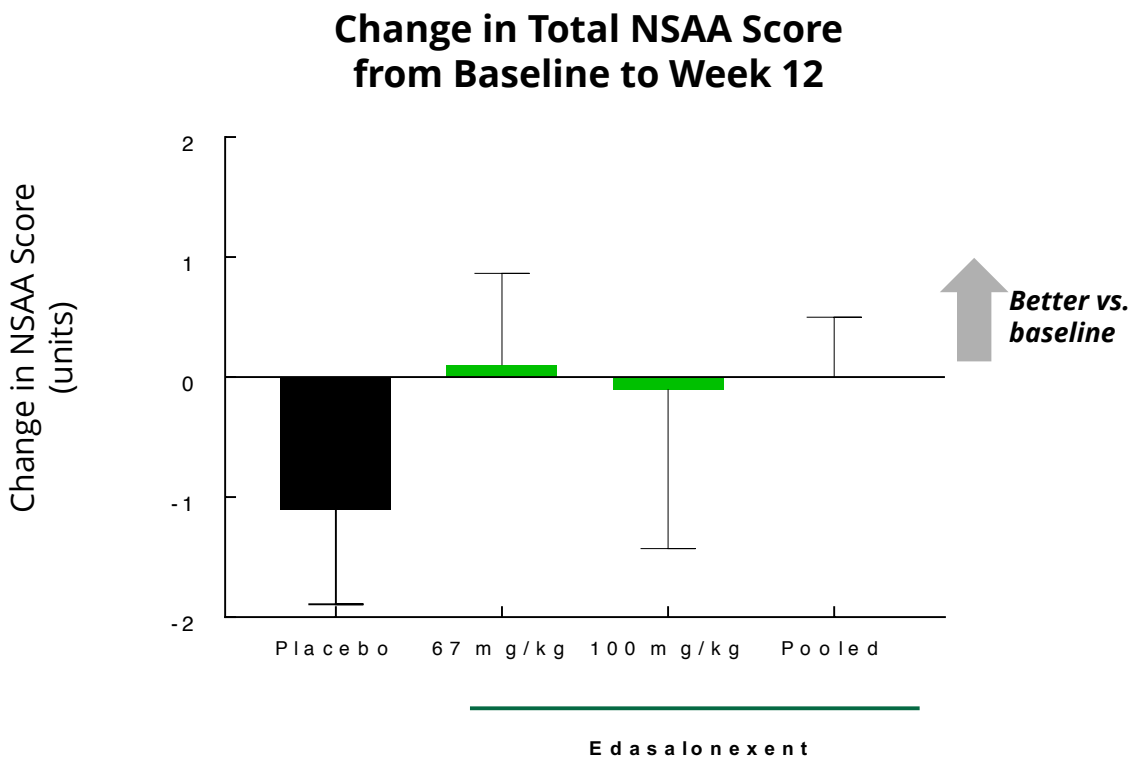
North Star Ambulatory Assessment (NSAA)



- Change in NSAA was numerically better for edasalonexent 100 mg/kg/day vs. placebo although not statistically significant.

Error bars in chart denote SEM

Adverse Events: No Safety Signals and Well Tolerated

- **No safety signals**
- **Well tolerated with majority of adverse events being mild in nature**
  - Most common treatment-related adverse events were mild diarrhea and vomiting
- **No serious treatment-related adverse events**
- **No dose reductions**
- **No discontinuations**

Summary of Top-Line Results for MoveDMD Trial Part B and Anticipated Next Steps

- **If we had seen a change in the primary MRI end point at 12 weeks, this could have provided a path for more efficient trials in Duchenne.**
  - Shorter trials
  - No muscle biopsy
- **Unfortunately, we did not see any significant change in the primary end point of change from baseline in MRI T2 of the composite of lower leg muscles for pooled edasalonexent doses vs. placebo.**
- **However, we did see that the edasalonexent 100 mg/kg/day treatment group was consistently numerically better vs. placebo across multiple measures although the changes were not statistically significant.**
- **We also saw that the 67 mg/kg/day group had mixed results compared with both the 100 mg/kg/day treatment group and placebo, which in each case was not statistically significant.**
- **Importantly, no safety signals were seen and edasalonexent was well tolerated with an adverse event profile consistent with prior findings. There were no dose reductions or discontinuations.**
- **Our plan is to complete the full analyses of data from Part B of the trial.**
- **The open-label extension portion of the MoveDMD trial is ongoing to assess effects in patients on edasalonexent over a longer time.**

Thank You!

- Patients and families
- Patient groups
- ImagingDMD Investigators and Staff
- For questions: Email our Chief Medical Officer, Joanne Donovan, M.D., Ph.D. and the Clinical Team: **joanne.donovan@catabasis.com**

Jett Foundation

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

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