Edasalonexent

Parent Project Muscular Dystrophy's 2017 Connect Conference
June 30, 2017

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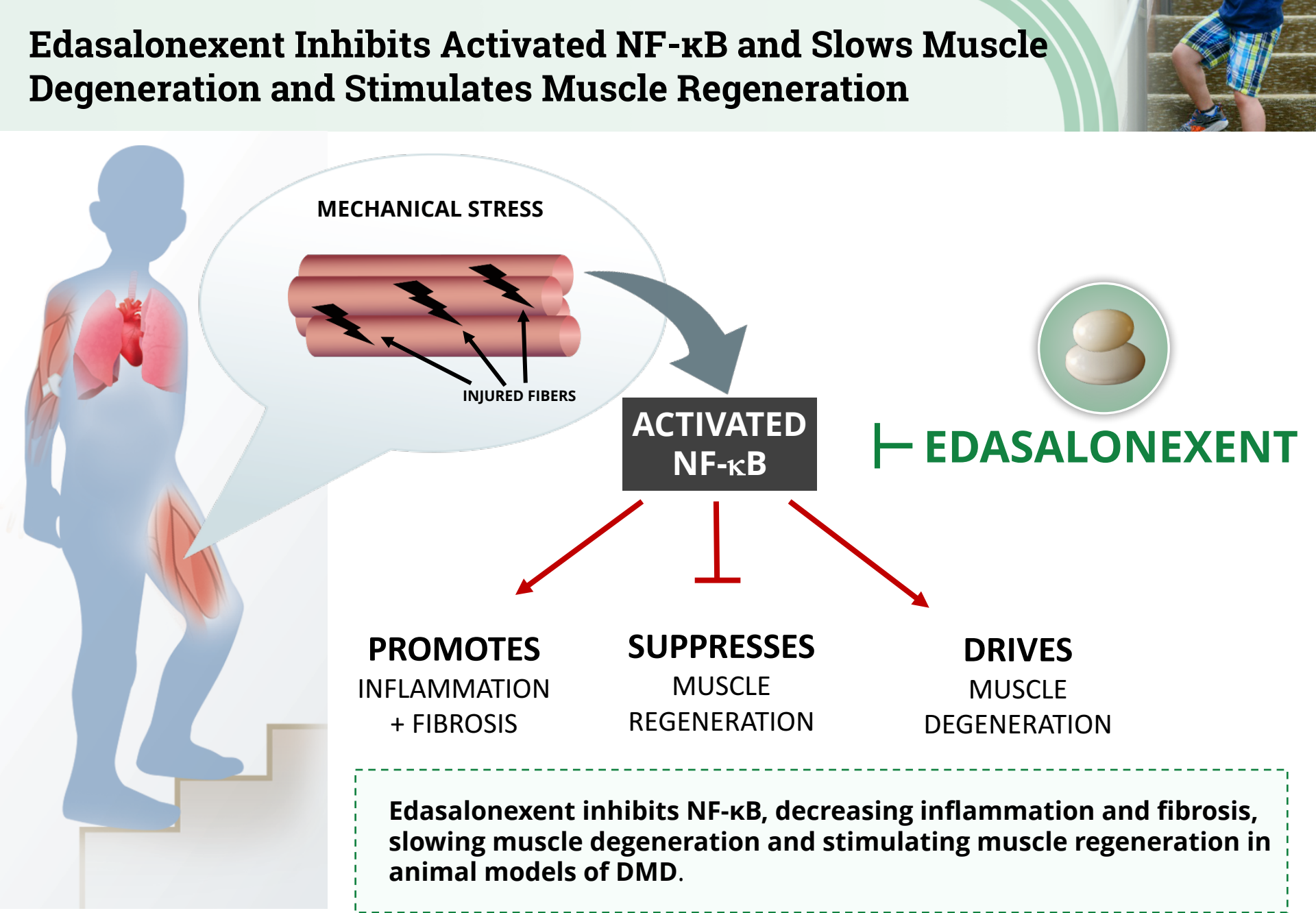
Forward Looking Statements

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 period ended March 31, 2017, 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.

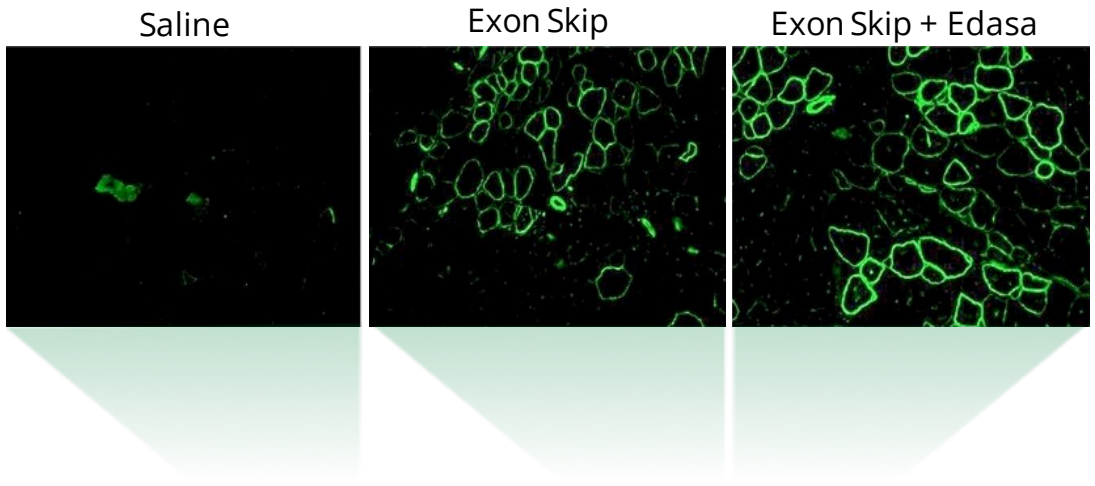
Edasalonexent Inhibits Activated NF-κB and Slows Muscle Degeneration and Stimulates Muscle Regeneration

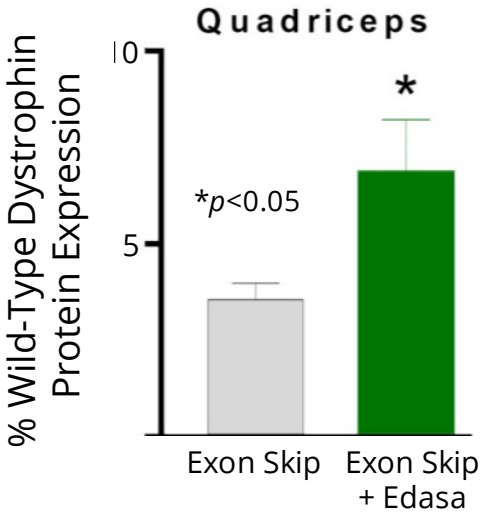


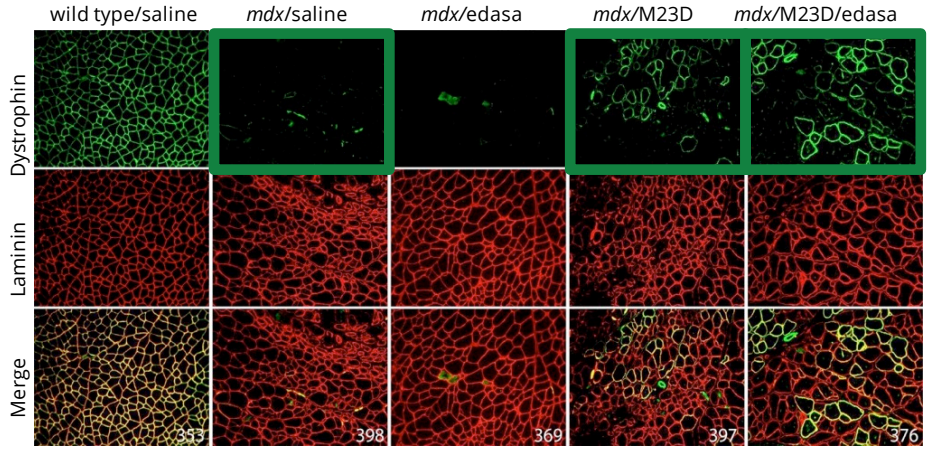
Edasalonexent inhibits NF-κB, decreasing inflammation and fibrosis, slowing muscle degeneration and stimulating muscle regeneration in animal models of DMD.

Hammers, et al. JCI Insight 2016 1(21): e90341

Edasalonexent Increases Dystrophin Expression in Combination with Exon-Skipping





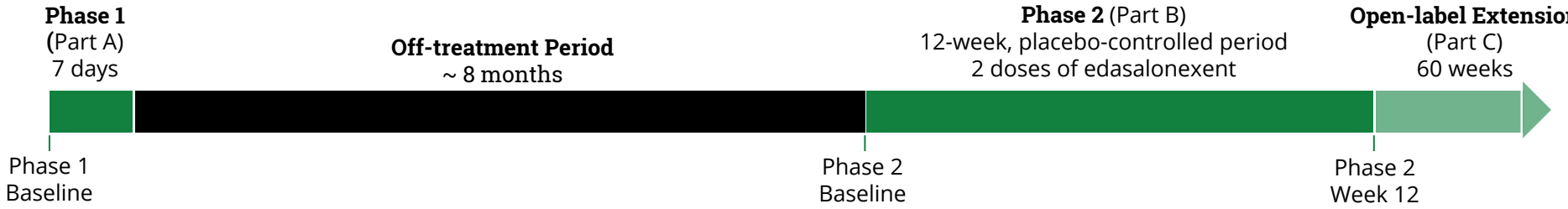


M23D: exon skipping specific for *mdx*; Nelsa Estrella, Sarepta (Unpublished observations)

- Activated NF-κB increases the expression of several microRNAs that suppress dystrophin production
- Edasalonexent may increase dystrophin levels in patients in combination with dystrophin targeted therapies including exon skip and gene therapy
- Thus, inhibiting NF-κB may enhance dystrophin expression in DMD and in Becker's

MoveDMD Phase 2 Study Design

Study Design:



- Study enrolled 31 boys with any Duchenne mutation ages 4 to 7 not on corticosteroids

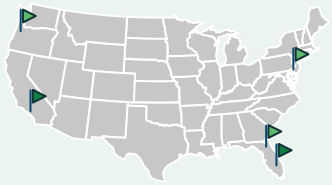
Study designed to allow two pre-specified analyses:

- **Placebo-controlled:** Comparison of changes between edasalonexent and placebo for the 31 boys enrolled in Phase 2

- **Crossover:** Comparison of changes during off-treatment period to edasalonexent treatment period for 12 boys who were in both parts of the study

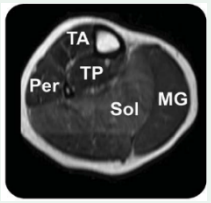
Phase 2 Key Study Metrics and Efficacy End Points

Key Study Metrics



- Enrolled total of 31 boys at 5 sites for Phase 2 of the trial, 16 of whom also participated in Phase 1. In Phase 2, patients were randomized to:
  - Edasalonexent 67 mg/kg/day given as twice per day dosing
  - Edasalonexent 100 mg/kg/day given as three times per day dosing
  - Placebo
- All 31 patients who enrolled completed Phase 2 of the trial

Primary Efficacy End Point



- Average change from Baseline to Week 12 in MRI T2 relaxation time for the composite of lower leg muscles:
  - Soleus (Sol)
  - Medial gastrocnemius (MG)
  - Tibialis posterior (TP)
  - Tibialis anterior (TA)
  - Peroneals (Per)

- What is MRI T2?
  - T2 is a measurement which helps us understand the properties of muscle including inflammation

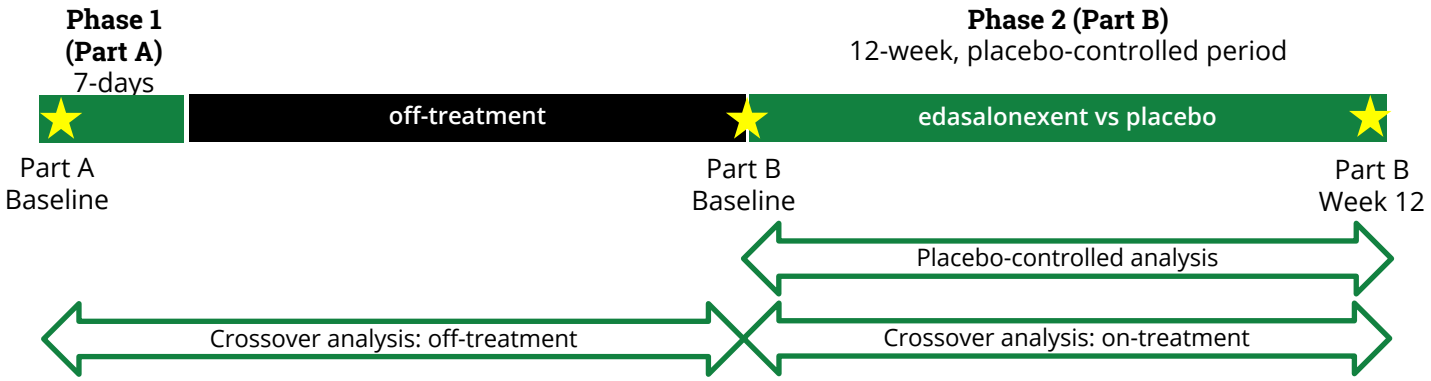
Additional Efficacy End Points

- Speeds and times for timed function tests (TFTs):
  - Completing the 10-meter walk/run test (10MWT)
  - Climbing 4 stairs (4SC)
  - Standing from supine (time to stand: TTS)
- Pediatric outcomes data collection instrument (PODCI)
- North Star Ambulatory Assessment (NSAA)
- Muscle strength testing
- Other MRI/MRS measures in lower and upper leg muscles

MRS: Magnetic Resonance Spectroscopy

Analysis Plan:
Placebo-Controlled and Crossover Predefined Analyses

Study Design:



- During the approximately 8-month off-treatment period from the Part A Baseline to Part B Baseline, patients were off-treatment except for the initial week of dosing in Part A.

Prespecified analyses:

- **Placebo-controlled:** Comparison of change in Part B between edasalonexent and placebo for the 31 boys enrolled

- **Crossover:** Comparison of off-treatment period to edasalonexent treatment period in Part B for the 12 boys who completed Phase 1 and were then randomized to edasalonexent in Phase 2

MoveDMD Phase 2 Demonstrated Numerical Improvements in Multiple Functional Measures

Two Pre-specified Analyses

	Placebo-Controlled			Crossover
	Edasa 67 mg/kg/day (n=10)	Edasa 100 mg/kg/day (n=10)	Edasa Pooled (n=20)	Edasa Pooled (n=12)
10-meter walk/run	+	+	+	+
4-stair Climb	+	+	+	+
Time to stand	-	+	-	+
North Star Ambulatory Assessment (NSAA)	+	+	+	+
Pediatric Outcomes Data Collection Instrument (PODCI)	+	+	+	+*

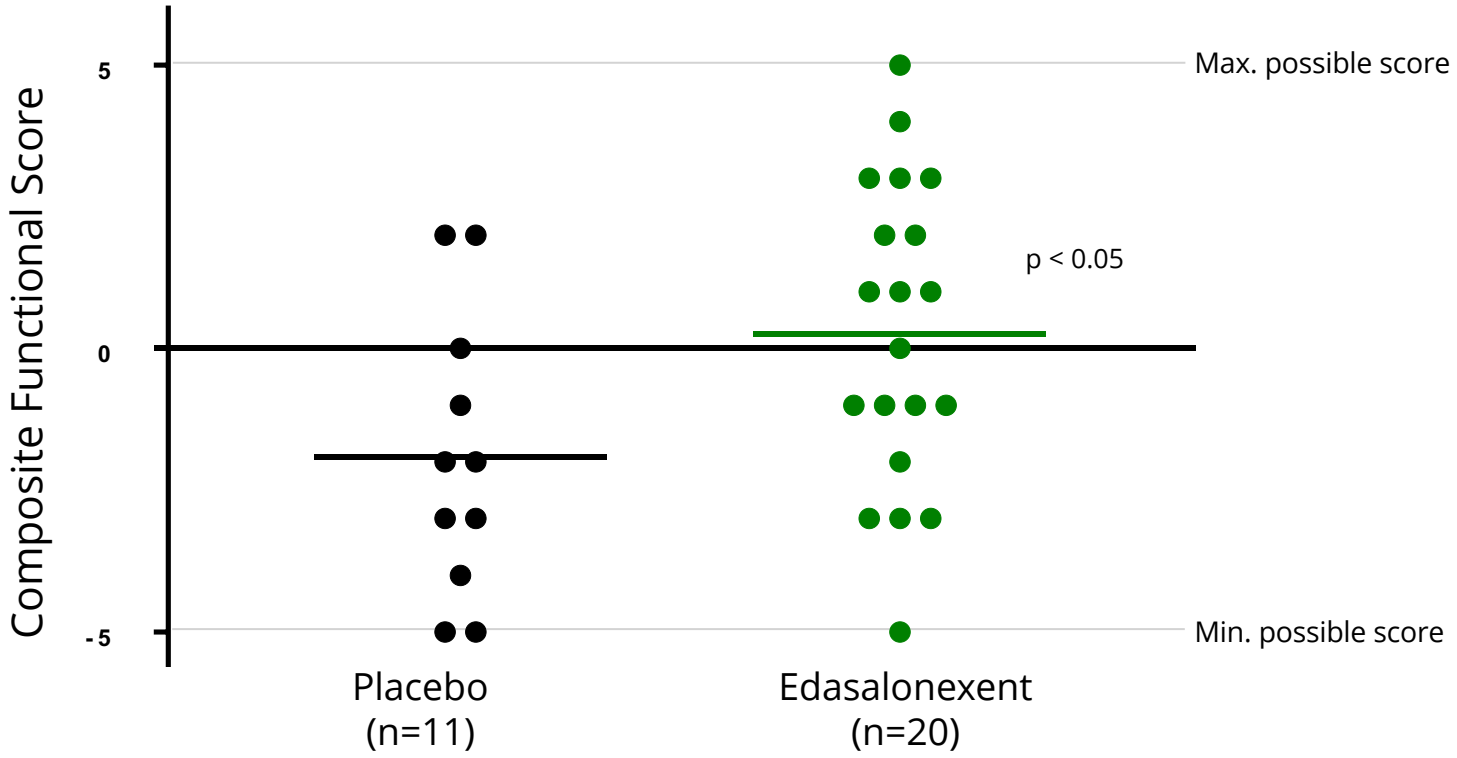
* $p < 0.05$

Placebo-Controlled: Comparison of changes between edasalonexent and placebo for the 31 boys enrolled in Phase 2

Crossover: Comparison of changes during off-treatment period to edasalonexent treatment period for 12 boys who were in both parts of the study

+ indicates numerical improvement with edasalonexent compared to placebo or off-treatment period

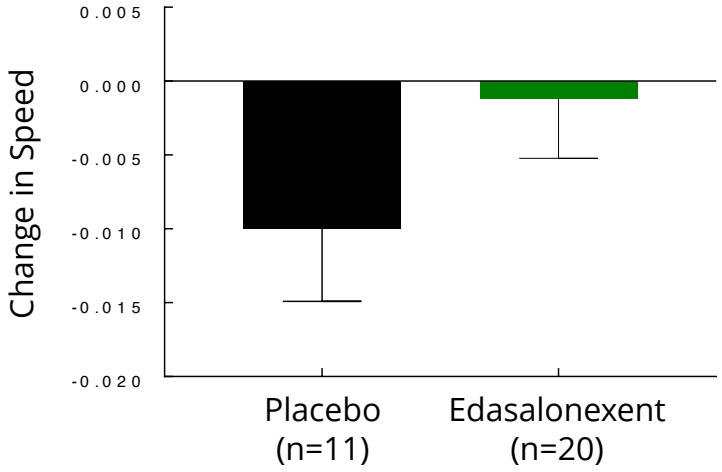
Composite Functional Score: Rate of Decline Slowed with Edasalonexent



- **Individual scores for placebo-controlled analysis of 4-stair climb, 10-meter walk/run, time to stand, NSAA and PODCI were pooled**
  - +1 for improvement, -1 for decline, 0 for no change
- **Post-hoc analysis: average composite scores by individual improved vs. placebo**

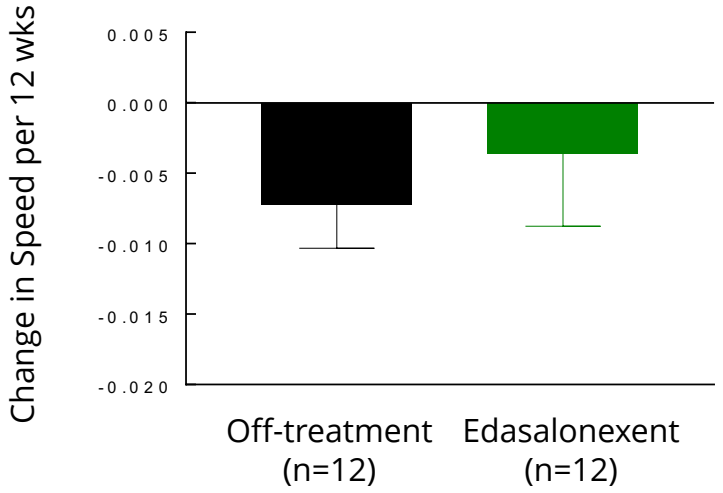
Timed Function Test: 10-meter Walk/Run Speed
Rate of Loss of Function Slowed

Placebo-Controlled



- The change in 10-meter walk/run speed was improved by >80% in edasalonexent group compared to placebo

Crossover

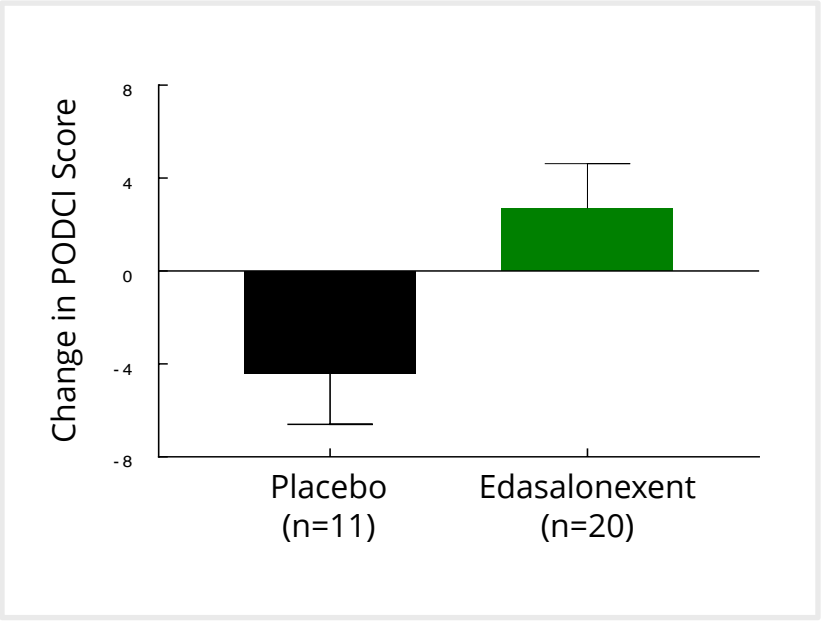


- There was a ~8% decline in speed in the off-treatment period, from a baseline speed of 0.17 tasks/sec (~5.9 seconds)
- The rate of decline in 10-meter walk/run was improved by 50% in edasalonexent crossover period vs. off-treatment period

Error bars in chart denote SEM, change in speed normalized to 12 weeks

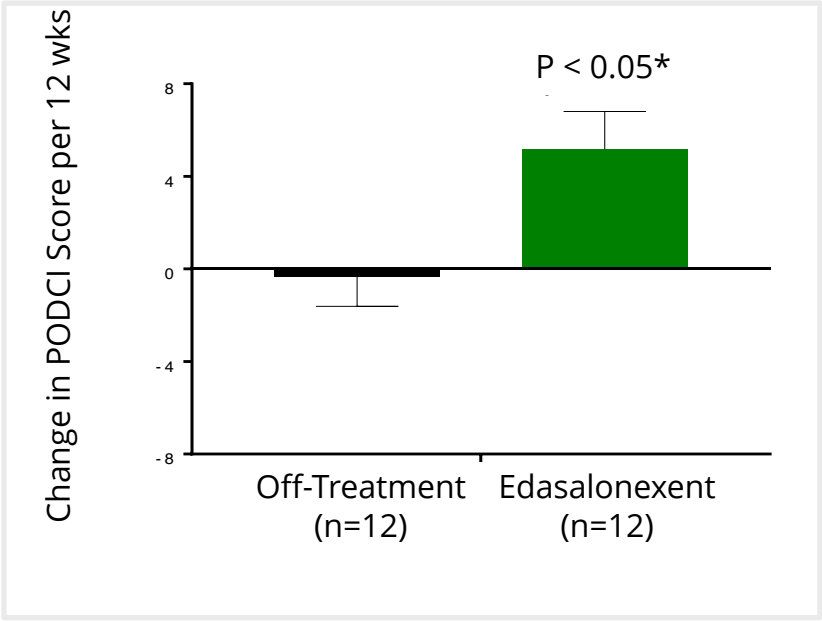
Global Functional Assessment: Pediatric Outcomes Data Collection Instrument (PODCI) Showed Improvements

Placebo-Controlled



- The change in the PODCI was numerically better for edasalonexent than placebo

Crossover



- A decline in the score in PODCI was observed in the off-treatment period while an improvement in score was observed in the edasalonexent crossover period

PODCI is a questionnaire for parents that asks about observations of their son's daily activities, e.g., putting on a coat, walking a block and climbing a flight of stairs. These data illustrate the Transfer and Basic Mobility Scale, which correlates with loss of functional milestones.

Error bars in chart denote SEM, change in score normalized to 12 weeks
* $p < 0.05$ indicates statistically significant

catabasis

MoveDMD Trial Phase 2 Results: Safety

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

Key Conclusions from Phase 2 Data

- **Reduction in rate of functional decline in MoveDMD Phase 2**
  - Reduction in rates of decline in well established timed function tests
  - Improvements in scores on validated global functional assessments
- **No safety signals identified and well-tolerated**
- **Functional improvements observed in Phase 2 provide necessary information for Phase 3 trial design**
- **Expect results from open-label extension of MoveDMD trial in Q3 2017**
- **Expect to announce Phase 3 trial plan in H2 2017**

Acknowledgments

- Patients and families
- Patient groups
- ImagingDMD Staff
- Catabasis team
- Thanks to PPMD and MDA for generous grant support for patient travel