



Edasalonexent (CAT-1004)

**An NF- κ B Inhibitor in Development for Patients with
Duchenne Muscular Dystrophy**

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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, including statements about future clinical trial plans including, among other things, statements about our plans to commence a single global Phase 3 trial in Duchenne muscular dystrophy, or DMD, in the first half of 2018 to evaluate the efficacy and safety of edasalonexent for registration purposes, our plans to report top-line results from this trial in 2020 and our plans to continue to evaluate data from the open-label extension of our MoveDMD® clinical trial of edasalonexent for the treatment of DMD. 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 the Company’s product candidates, including the final trial design of our planned Phase 3 trial in DMD; availability and timing of results from preclinical studies and clinical trials, including the availability of top-line results from our planned Phase 3 trial in DMD in 2020; 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, including our expected target product profile for edasalonexent in DMD; our ability to obtain financing on acceptable terms and in a timely manner to fund our planned Phase 3 trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes; availability of funding sufficient for the Company’s foreseeable and unforeseeable operating expenses and capital expenditure requirements; other matters that could affect the availability or commercial potential of the Company’s product candidates; and general economic and market conditions and other factors discussed in the “Risk Factors” section of the Company’s Quarterly Report on Form 10-Q for the period ended September 30, 2017, which is on file with the Securities and Exchange Commission, and in other filings that the Company 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.

Catabasis' Focus on Edasalonexent

- ▶ Catabasis is a biotech company in Cambridge, MA whose mission is to bring hope and life-changing therapies to patients and their families.
- ▶ Our goal is for edasalonexent to become a foundational oral therapy to slow the rate of progression for all people affected by Duchenne at all ages as stand alone agent and in combination with other therapies.

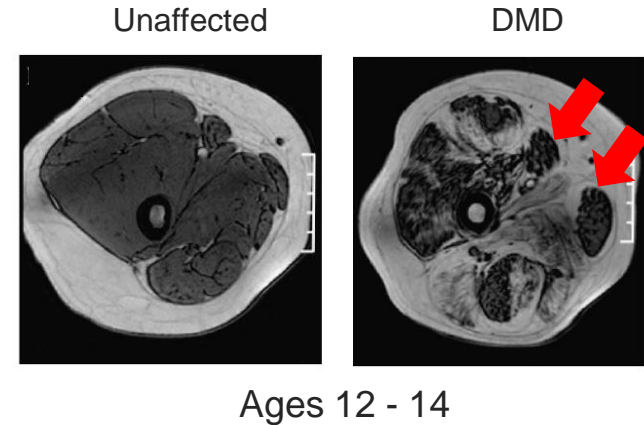


- ▶ **Results of the MoveDMD clinical trial support edasalonexent's advancement into Phase 3 clinical trials, planned to start in the first half of 2018.**

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

▶ Why Edasalonexent?

- In Duchenne, lack of dystrophin and mechanical stress activate NF- κ B 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
- Pre-clinical models support positive effects on skeletal, respiratory and cardiac muscle
- Phase 1 trials in adults showed no safety signals and that edasalonexent targets NF- κ B



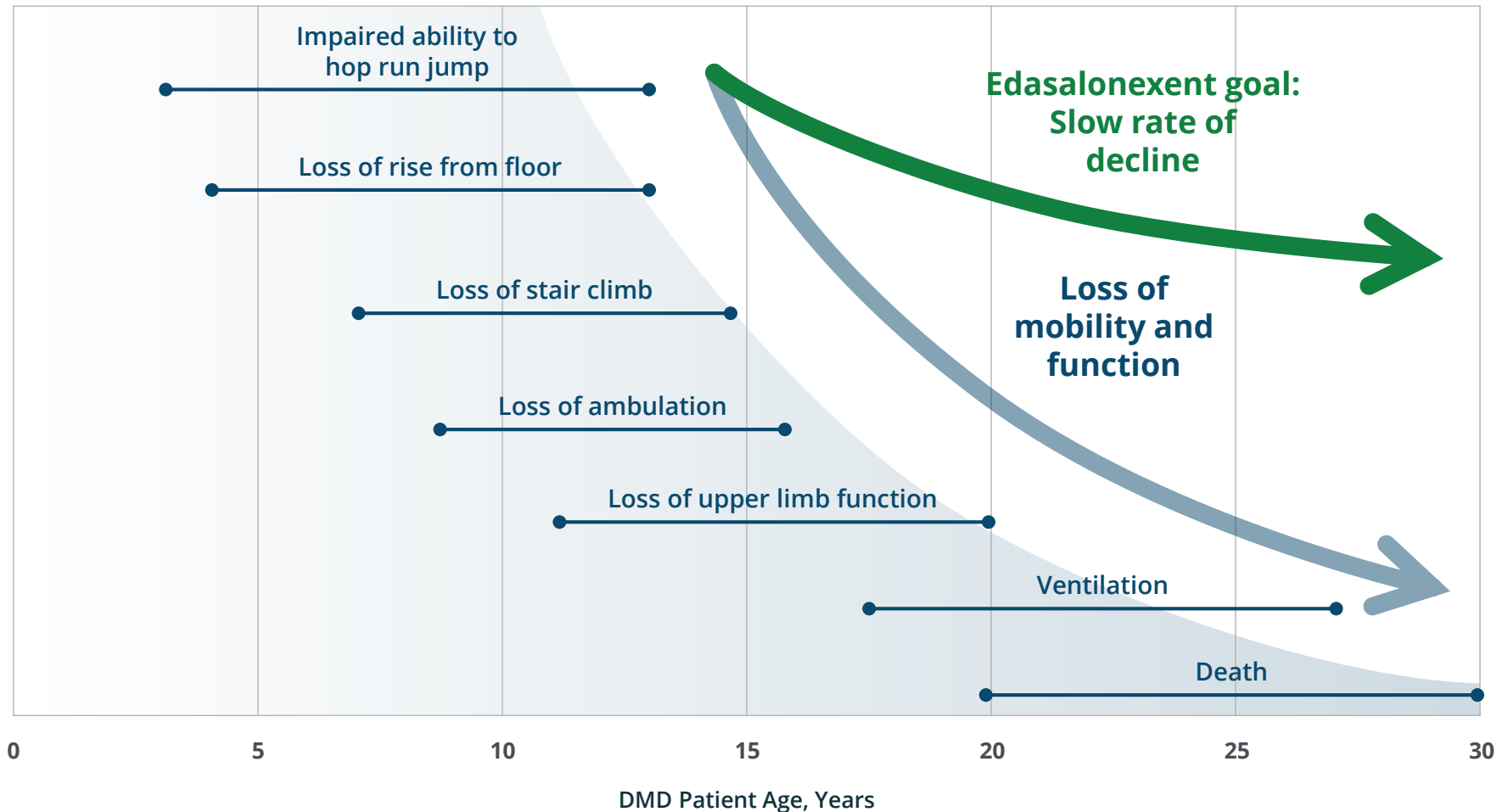
▶ Catabasis has been conducting the MoveDMD trial to understand the effects of edasalonexent in young boys with Duchenne



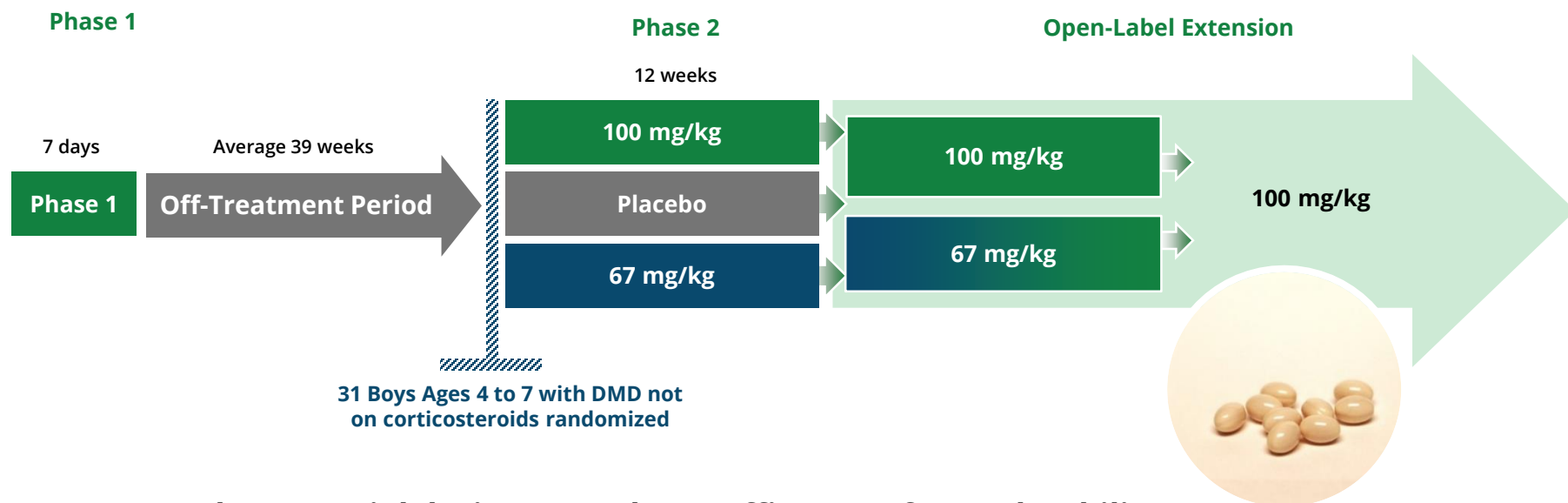
DMD Is Characterized by a Predictable Cascade of Discrete Losses of Function and Mobility Milestones



Typical DMD Disease Progression



MoveDMD Trial Designed to Inform Phase 3

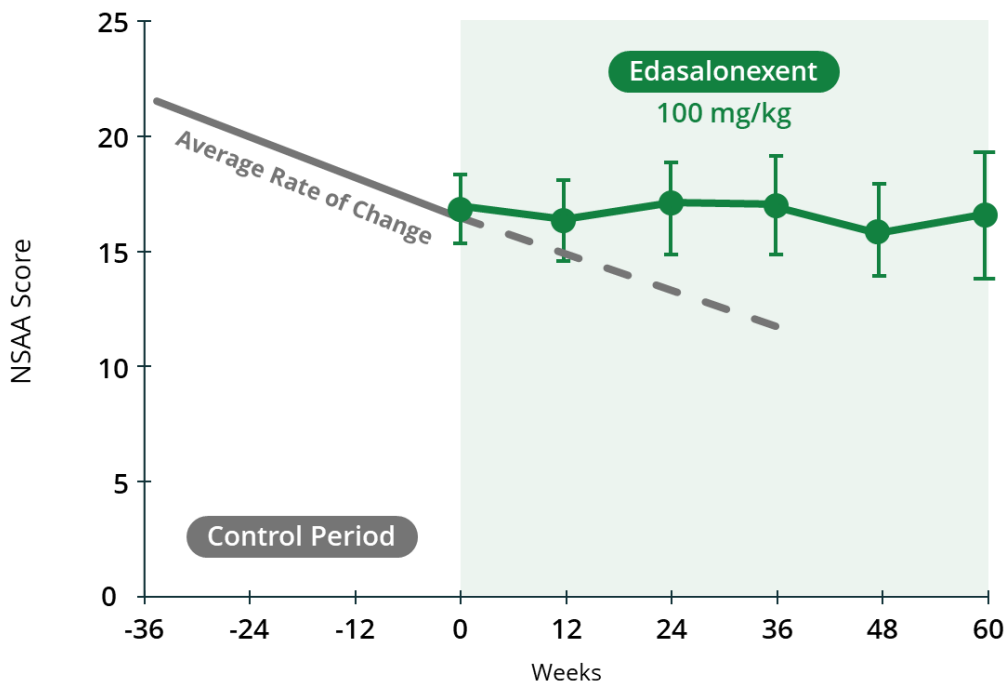
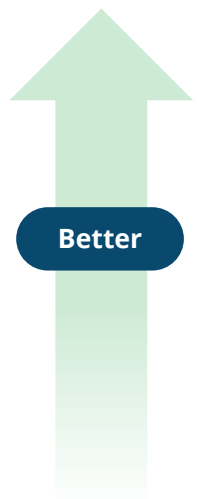


- ▶ **Integrated 3-part trial design to evaluate efficacy, safety, tolerability**
 - Assessments included North Star Ambulatory Assessment, age-appropriate timed function tests, MRI
- ▶ **Off-treatment control period measurements between Phase 1 and Phase 2**
 - Provided internal control for planned analyses
- ▶ **Phase 2 showed favorable trends towards the slowing of disease progression after 12 weeks with no safety issues**
- ▶ **Open-label extension enabled assessment of safety and efficacy following longer term treatment**

North Star Ambulatory Assessment Score Stabilized with Edasalonexent Treatment



North Star Ambulatory Assessment

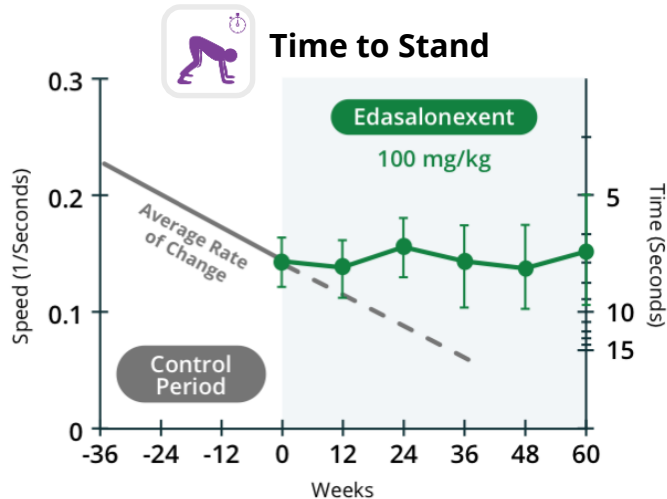
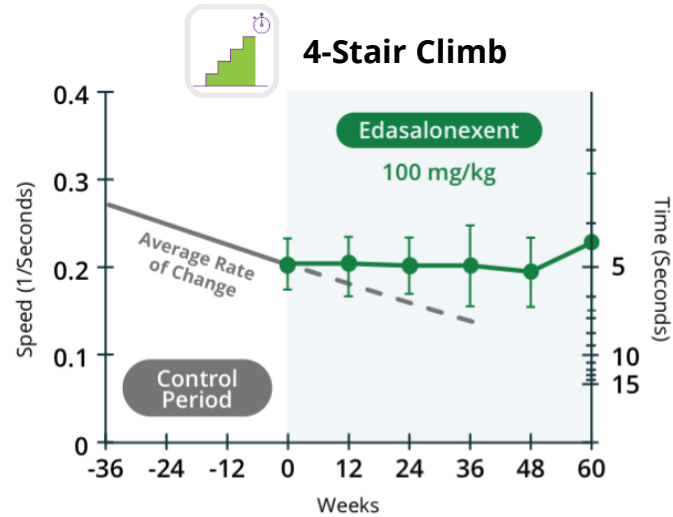
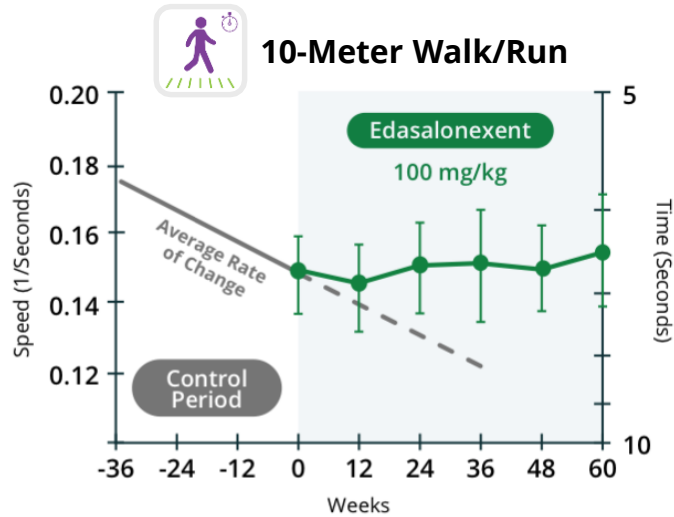


► Disease progression on edasalonexent improved compared with rate of change during off-treatment control period

All Timed Function Tests Speed Stabilized with Edasalonexent Treatment



Pre-Specified Analyses



- ▶ Disease progression on edasalonexent improved compared with rate of change during off-treatment control period

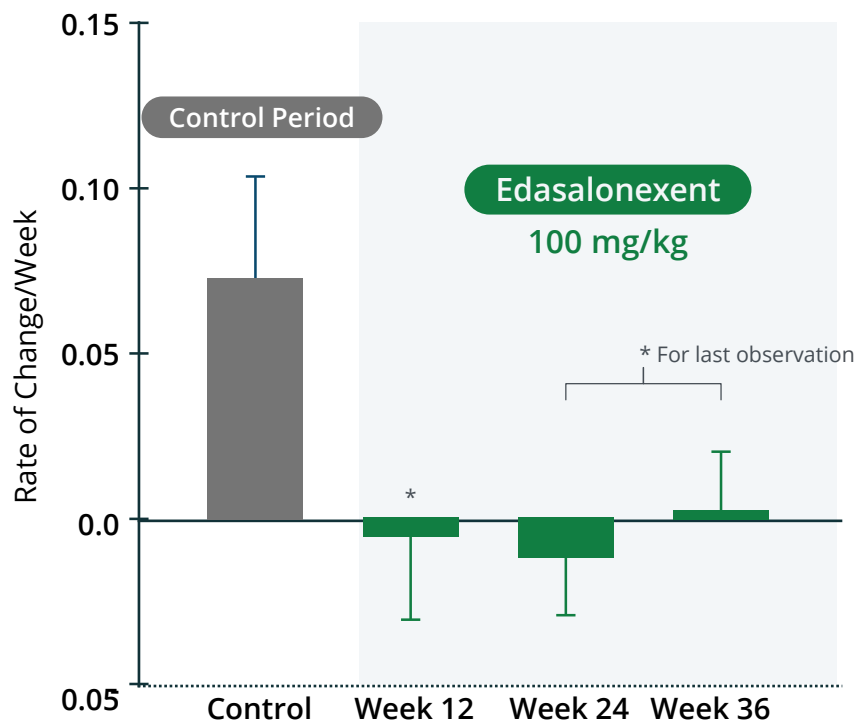


Edasalonexent Significantly Improved Rate of Change of MRI T2 Compared with Pre-Specified Control Period

- ▶ MRI T2 increases over time in DMD and is highly correlated with worsening timed function tests^ϕ
- ▶ Rate of change in boys on active treatment improved significantly compared to their own rate of change during the off-treatment control period
- ▶ 12-week Phase 2 MRI T2 primary endpoint for treated boys compared to boys in the placebo group was directionally positive although not statistically significant



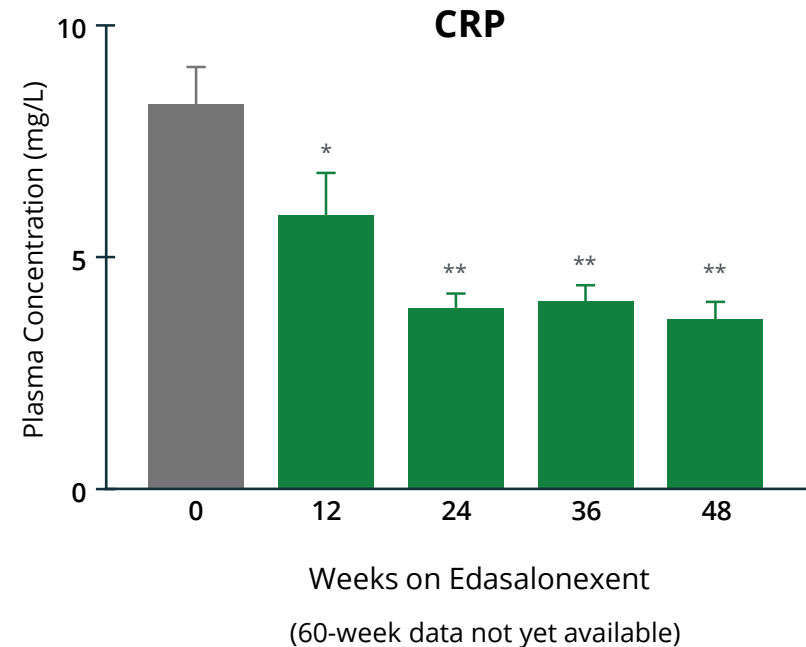
MRI T2 Rates of Change



Edasalonexent Significantly Reduced Plasma C-Reactive Protein Compared with Pretreatment Baseline

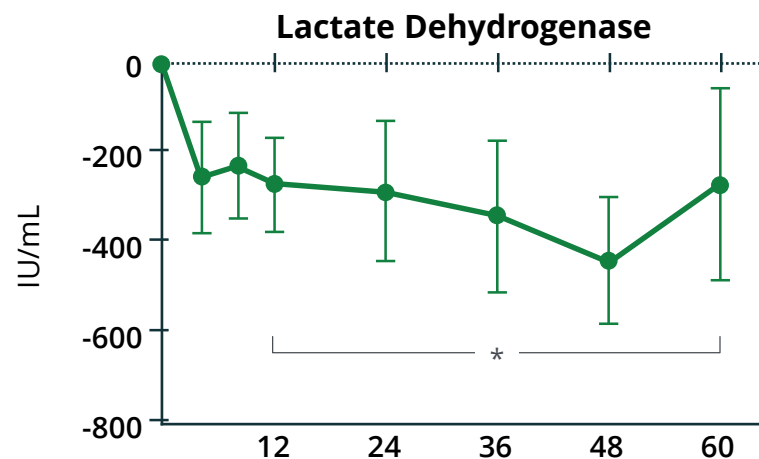
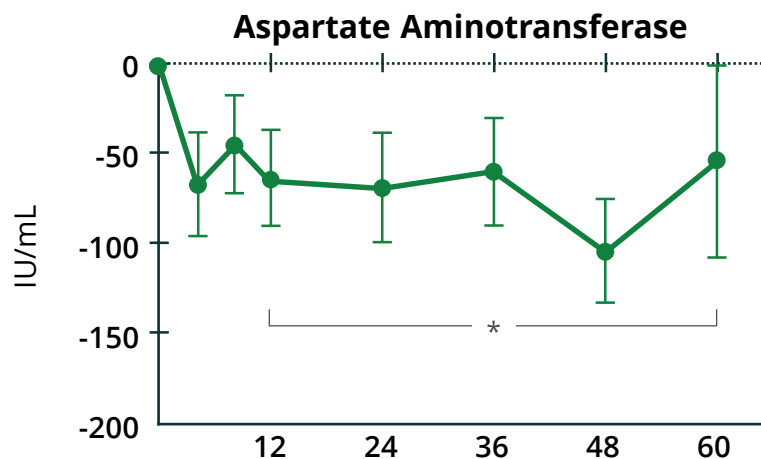
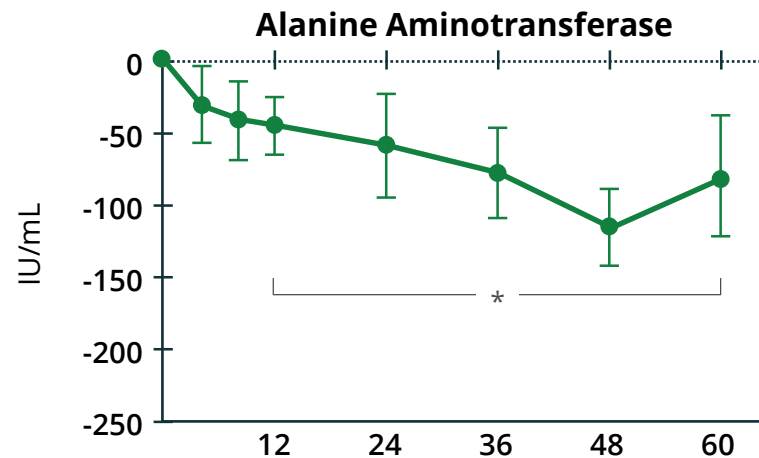
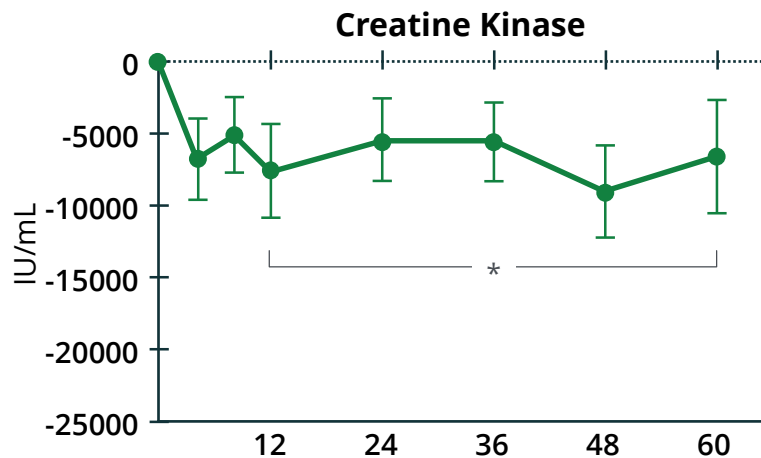


- ▶ C-reactive protein (CRP) is a well-characterized blood test marker that provides a global assessment of inflammation
- ▶ CRP is elevated in DMD
 - CRP approximately 3-fold higher in boys affected by DMD compared to unaffected boys†
- ▶ In MoveDMD, CRP significantly decreased from baseline through 48 weeks of 100 mg/kg edasalonexent
 - No change in CRP following 12 weeks of placebo



Muscle Enzymes Significantly Decreased from Baseline on Edasalonexent

- ▶ Plasma muscle enzymes are elevated 10 to 100 fold in DMD, indicative of leakage from damaged myocytes
- ▶ Decrease is consistent with positive impact on muscle health and supportive of an edasalonexent benefit



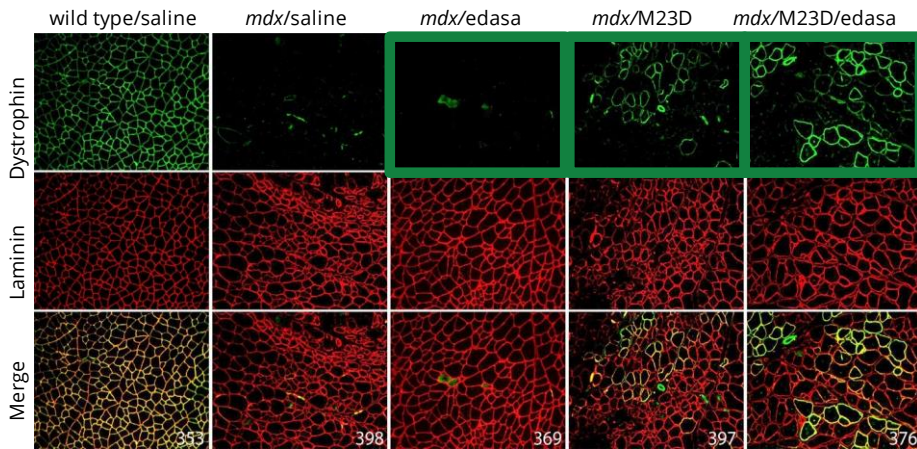
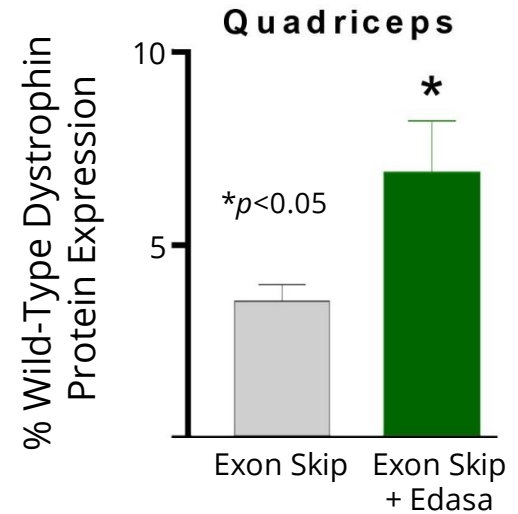
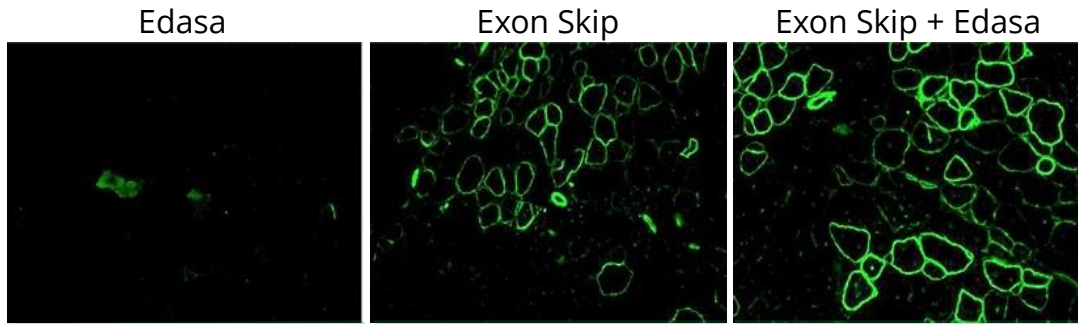
Weeks on Edasalonexent

Edasalonexent: Well Tolerated without Safety Signals



- ▶ **No safety signals to date**
- ▶ **Well tolerated, with most adverse events mild in nature, mostly gastrointestinal**
 - Most common treatment-related adverse event was mild and transient diarrhea
 - No serious treatment-related adverse events or dose reductions
- ▶ **No adverse trends in laboratory tests**
 - Decreases in all 4 muscle enzymes
- ▶ **Growth: Age appropriate weight, height and BMI changes through 60 weeks of treatment**
- ▶ **Heart rate decreased toward normal values at this age**

Edasalonexent Increases Dystrophin Expression in Combination with Exon-Skipping in *mdx* Mice

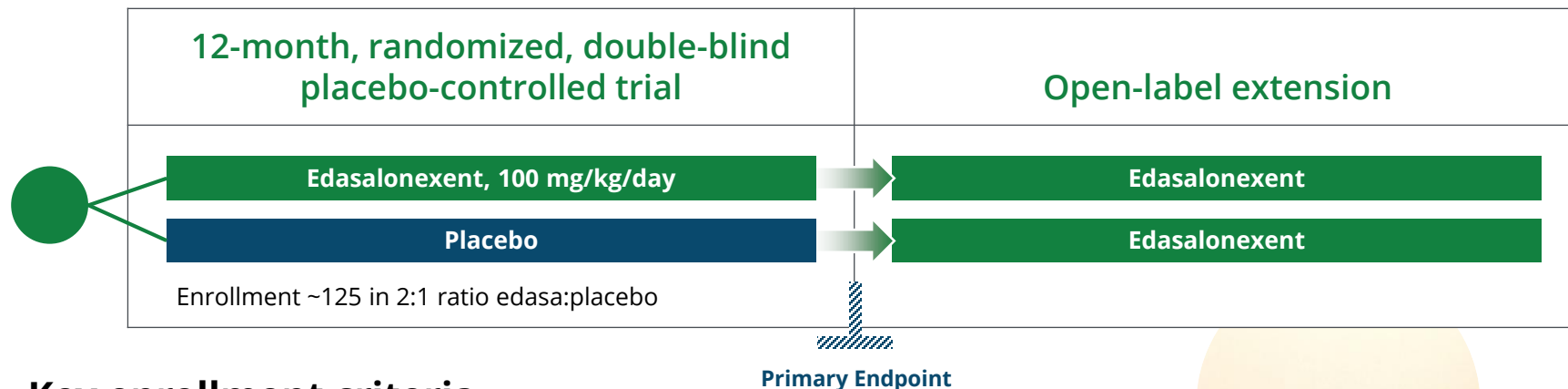


M23D: exon skipping specific for *mdx*

- ▶ Activated NF- κ B increases the expression of several microRNAs that suppress dystrophin production
- ▶ Inhibiting NF- κ B may enhance dystrophin expression in combination with dystrophin-targeted therapies in DMD

Boys amenable to eteplirsen are currently receiving combination treatment in MoveDMD trial

Positive MoveDMD Data Support Planned Global Phase 3 Registration Trial for Edasalonexent



▶ Key enrollment criteria

- Age 4 to 7th birthday
- Able to complete timed function tests
- Not on corticosteroids for at least 6 months
- Not on other investigational therapies for at least 1 month, can be on stable eteplirsen

▶ Visits / key assessments every 3 months

- North Star Ambulatory Assessment, Timed Function Tests, Muscle Strength
- Safety measures
- Assessments of growth, cardiac and bone health
- No biopsy, 6 minute walk test or MRI

▶ Locations: US, Canada, Europe and Australia – specific sites to be determined

Edasalonexent: Potential to Slow Disease Progression for All Boys with DMD

- ▶ Investigational oral disease-modifying agent for all patients with DMD, regardless of mutation type
- ▶ Edasalonexent substantially slowed DMD disease progression through 60 weeks in MoveDMD trial
- ▶ Phase 3 expected to start in first half of 2018
- ▶ Potential as monotherapy and also exploring potential to combine with dystrophin-targeted and other therapies



Grazie mille!

- ▶ **Patients and families**
- ▶ **Patient groups**
- ▶ **ImagingDMD Investigators and Staff**
 - University of Florida
 - Krista Vandeborne PT PhD
 - H. Lee Sweeney, PhD.
 - Rebecca J. Willcocks, Ph.D.
 - Sean C Forbes, PhD.
 - William T. Triplett, BSc
 - Glenn Walters, Ph.D.
 - Nemours Childrens Hospital
 - Richard S. Finkel MD (presenting author)
 - Oregon Health & Science University
 - Erika L. Finanger, MD
 - Bill Rooney, PhD
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Duchenne
Parent
Project
o n l u s

