



Catabasis Pharmaceuticals

Q3 2018

November 13, 2018

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 single global Phase 3 trial in Duchenne muscular dystrophy, or DMD, to evaluate the efficacy and safety of edasalonexent for registration purposes, 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 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, including our expected target product profile for edasalonexent in DMD; 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 and other factors discussed in the “Risk Factors” section of our Quarterly Report on Form 10-Q for the period ended September 30, 2018, 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.

Recent Accomplishments



- ▶ **Advancing late-stage development of edasalonexent**
 - Phase 3 PolarisDMD trial underway
 - After 72 weeks of treatment, preserved muscle function and sustained disease-modifying effects seen in boys treated with edasalonexent compared to control
- ▶ **Gaining new insights in DMD**
 - MRI T2 correlates with clinical outcome
- ▶ **Building on edasalonexent's potential**
 - Preclinical collaboration established to explore potential benefits of edasalonexent on cardiac function in Duchenne and Becker muscular dystrophies

Edasalonexent Is a Potential Disease Modifying Oral Therapy

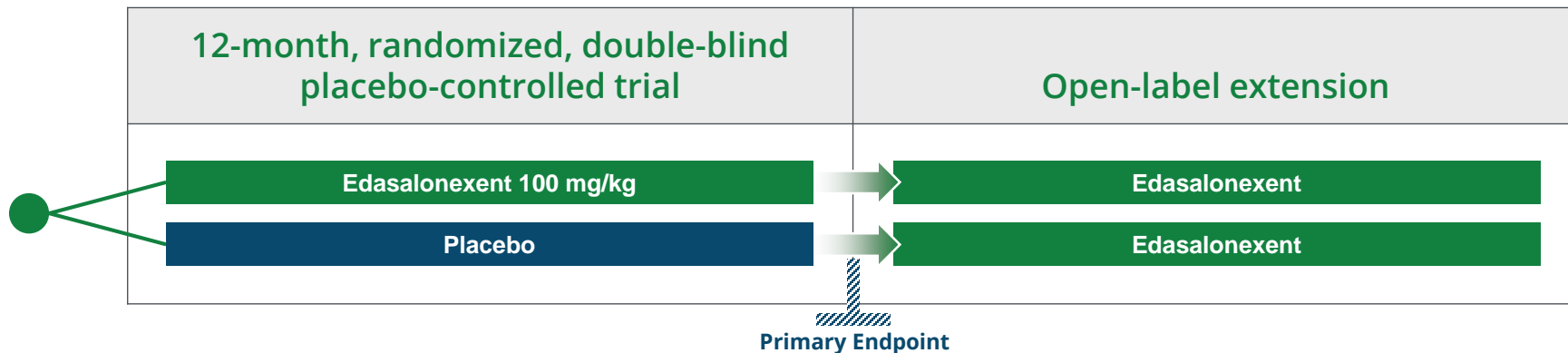
Our Vision for Edasalonexent:

- ▶ For all patients with DMD, regardless of mutation, from time of diagnosis throughout their lifetime
- ▶ Preserve both the skeletal and cardiac muscle disease
- ▶ Enhance the efficacy of dystrophin targeted therapies
- ▶ Favorably differentiated safety and tolerability profile from standard of care



**Developing a
potential **NEW**
Standard of
Care in
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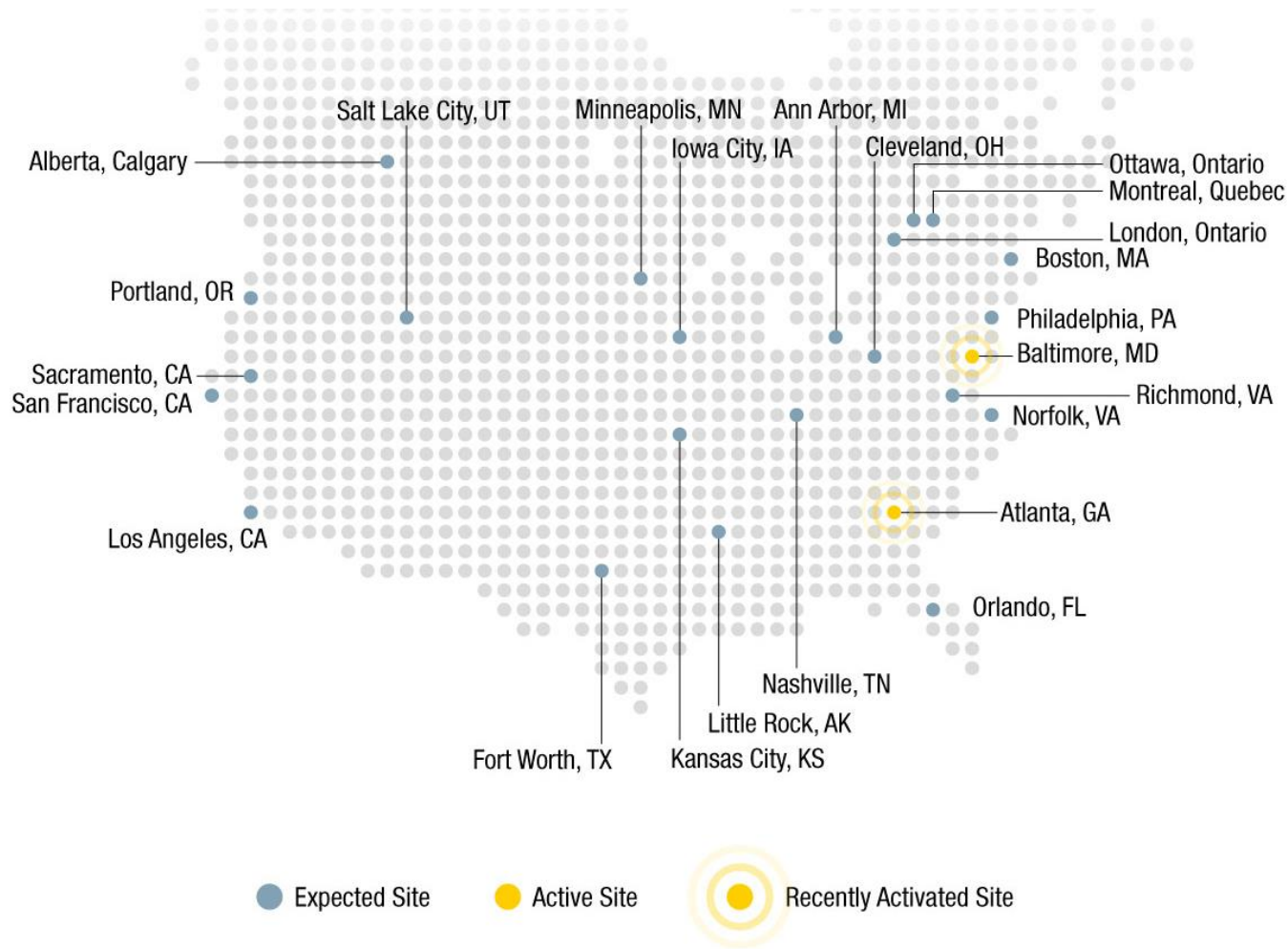
Global Phase 3 PolarisDMD Trial Designed for Registration



Key Phase 3 trial components previously evaluated in MoveDMD trial

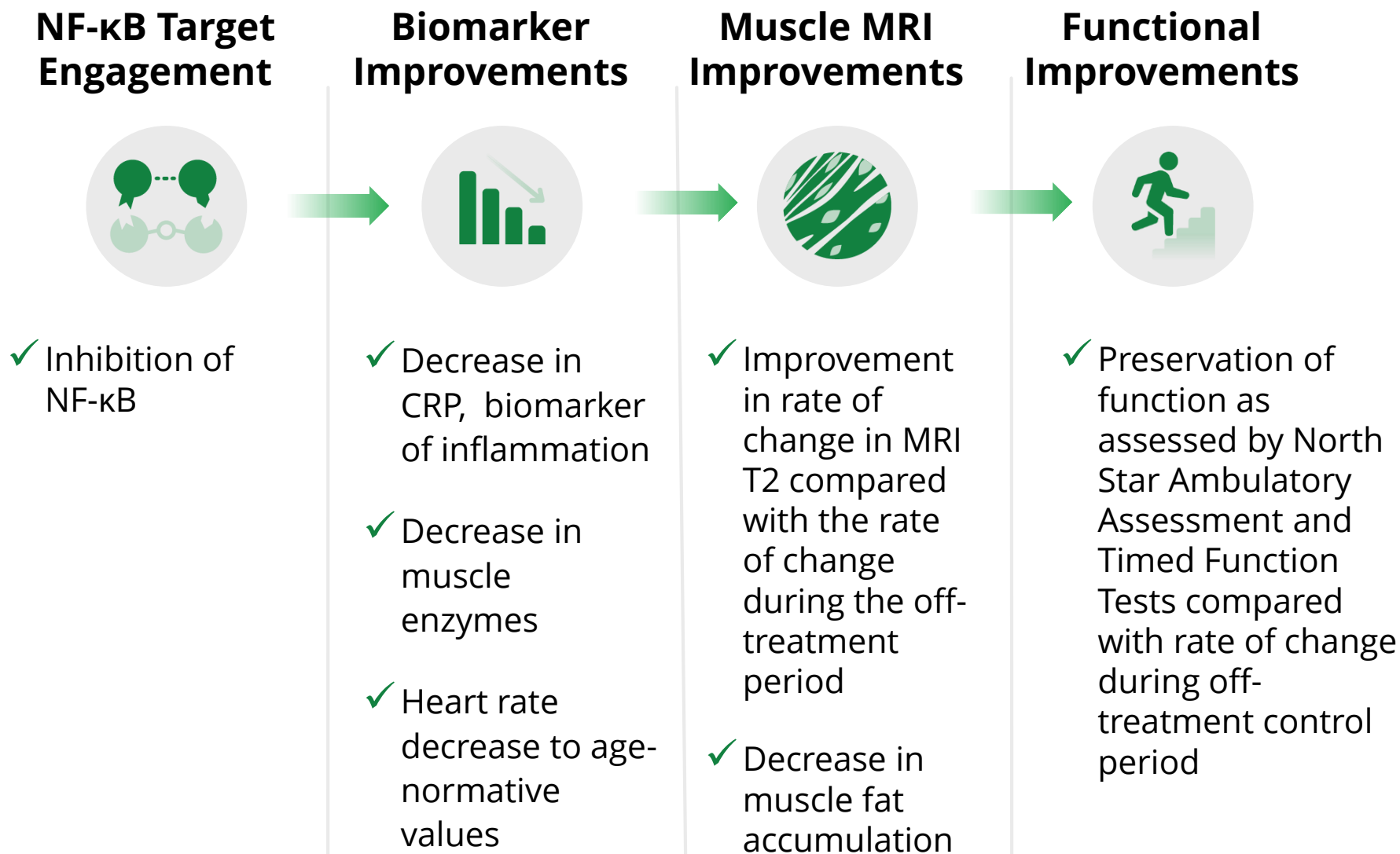
- ▶ **Enrollment:** ~125 boys, 2:1 randomization
- ▶ **Eligibility:** all mutations, age 4 to 7 (up to 8th birthday), off steroids for ≥6 months
- ▶ **Endpoints: consistent with FDA guidance**
 - Primary: Change in North Star Ambulatory Assessment
 - Key secondary: Age-appropriate timed function tests
 - Additional assessments include growth, cardiac and bone measures

Many Clinical Trial Sites in U.S. and Canada to Improve Patient Access



- Sites also anticipated in Europe, Israel and Australia

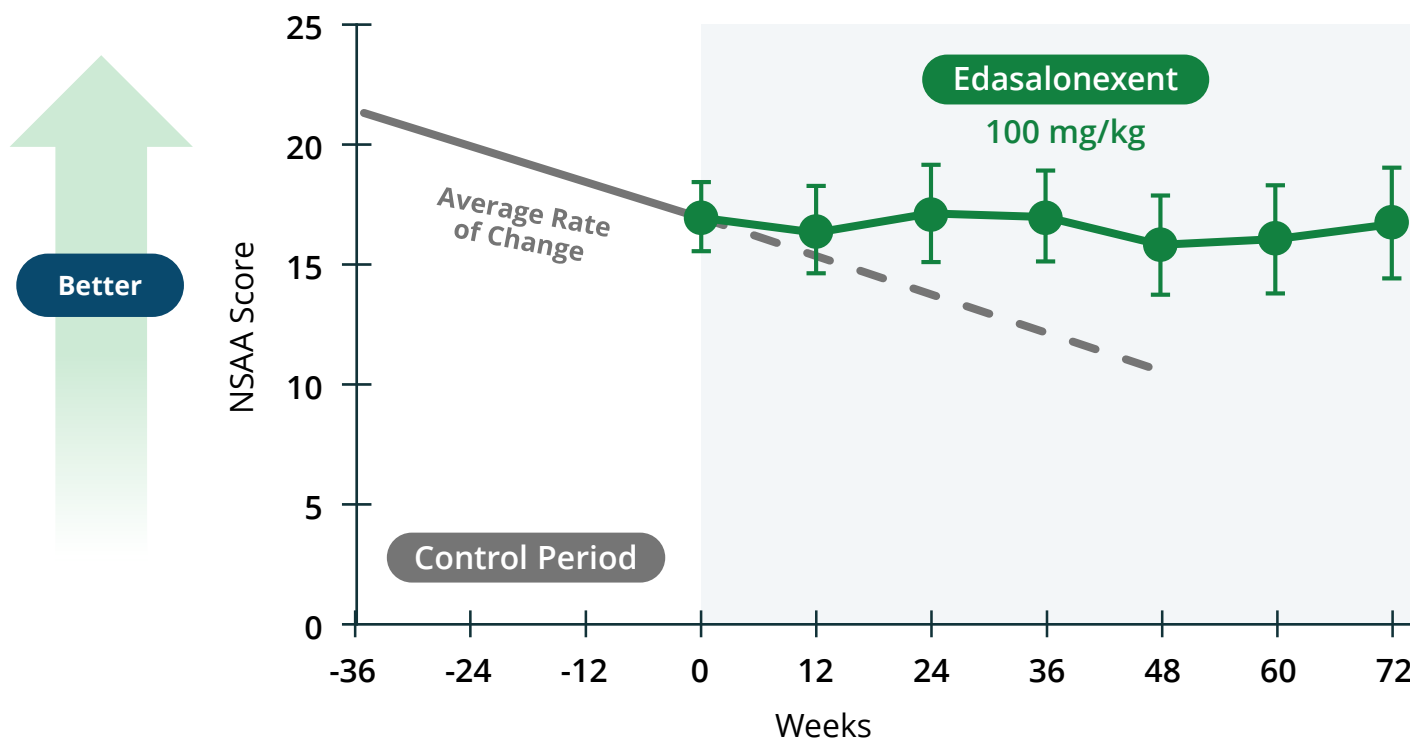
Promising Clinical Results Seen to Date with Edasalonexent



North Star Ambulatory Assessment Score Stabilized with Edasalonexent Treatment

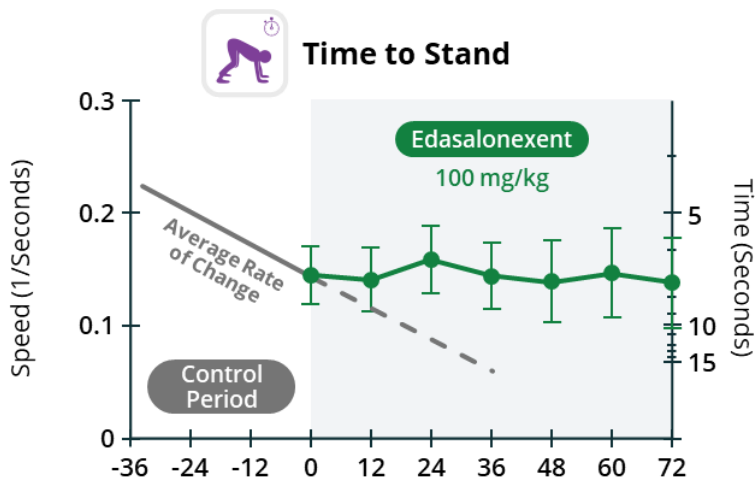
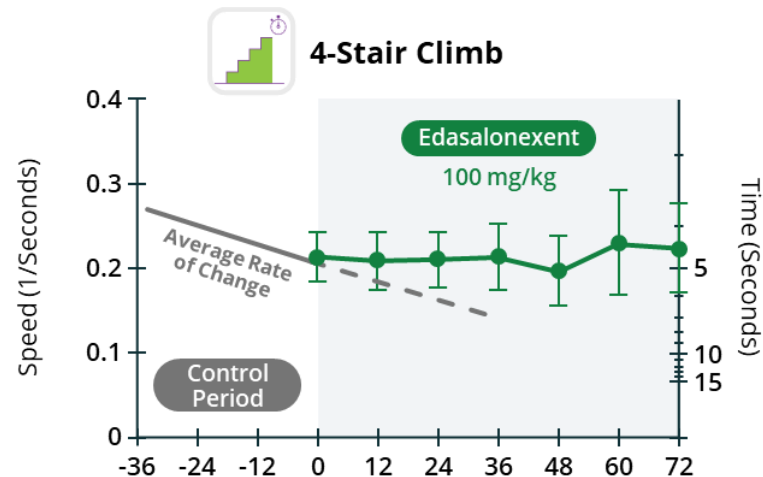
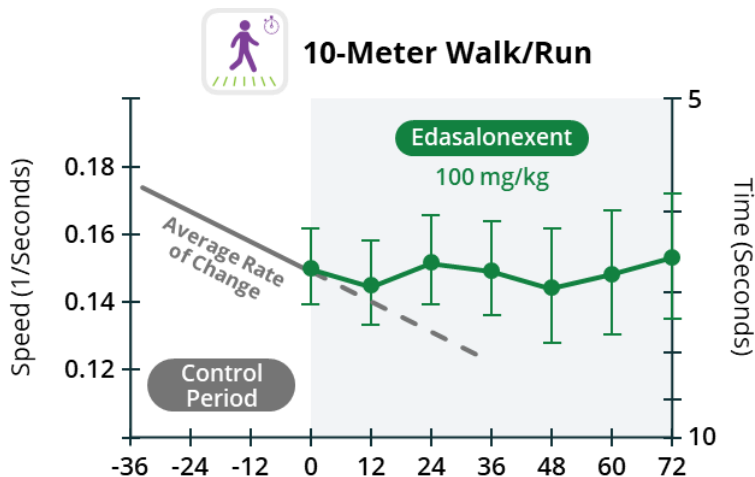


North Star Ambulatory Assessment



All Timed Function Tests Speed Stabilized with Edasalonexent Treatment

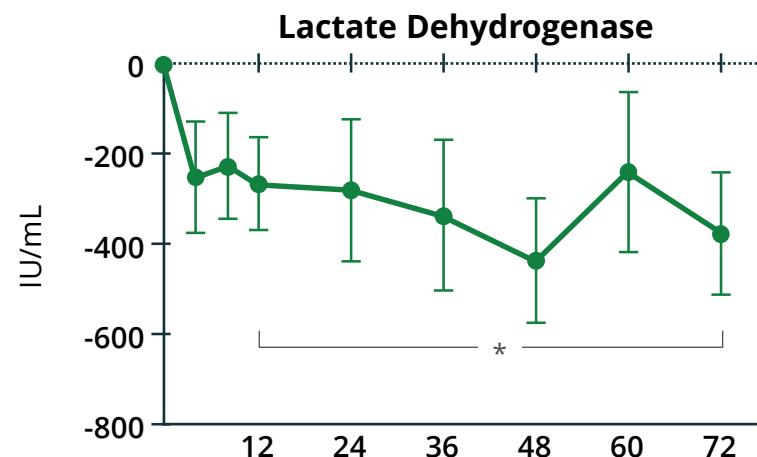
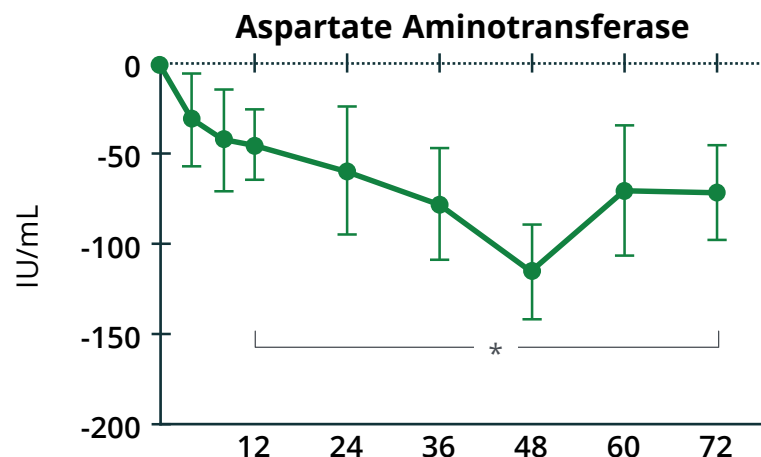
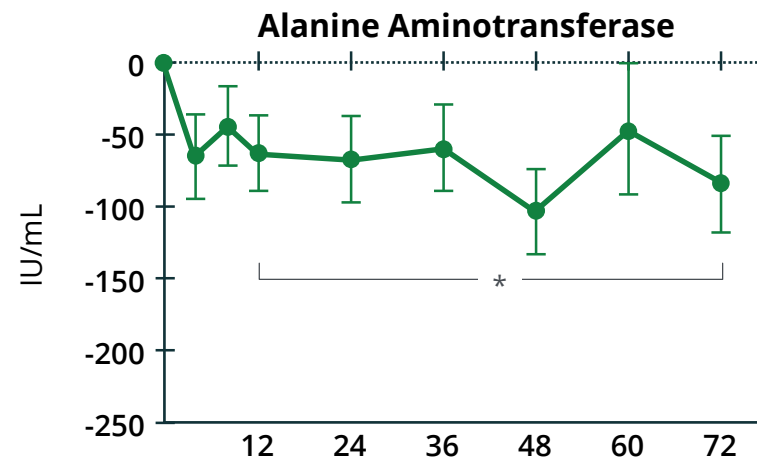
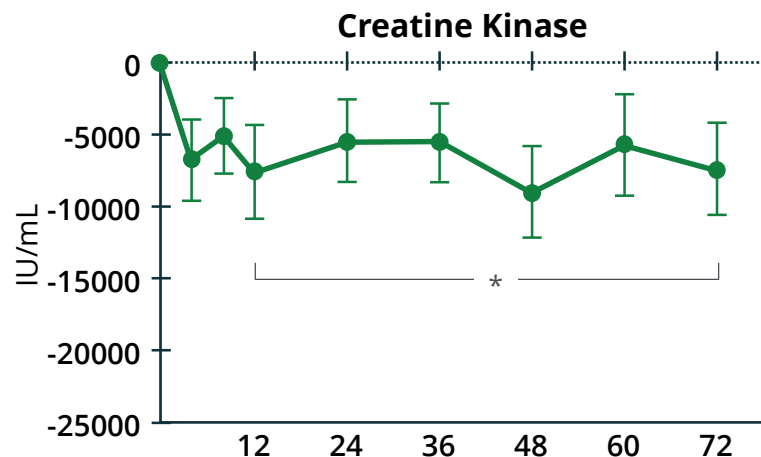
Pre-Specified Analyses



Week	0	12	24	36	48	60	72
N =	16	16	14	13	13	13	12

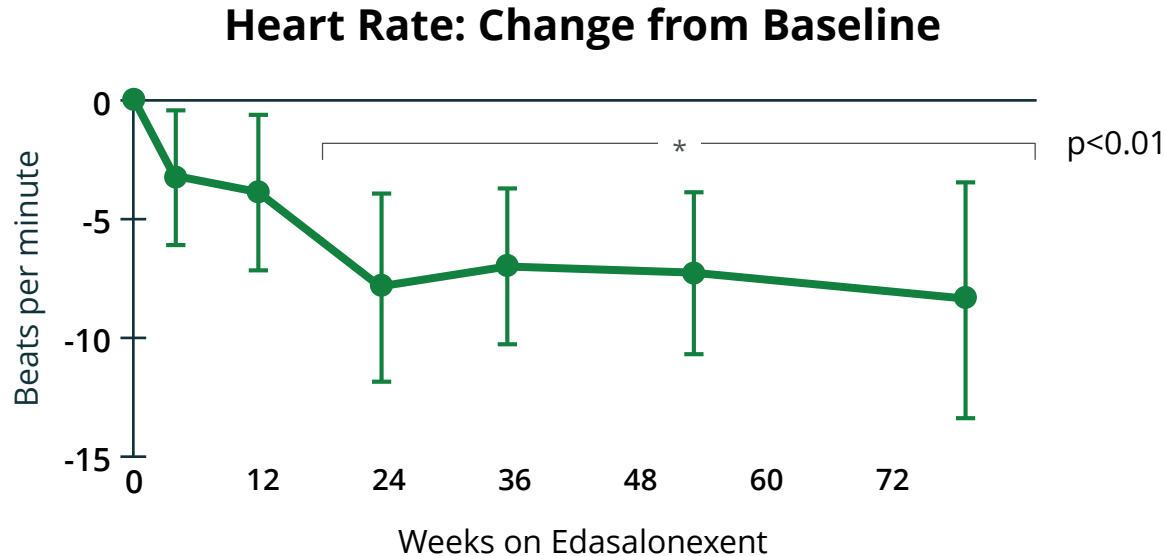
Muscle Enzymes Significantly Decreased on Edasalonexent Treatment

- ▶ 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 100 mg/kg Edasalonexent

Edasalonexent Has Potential to Have Positive Effects on Cardiomyopathy in DMD

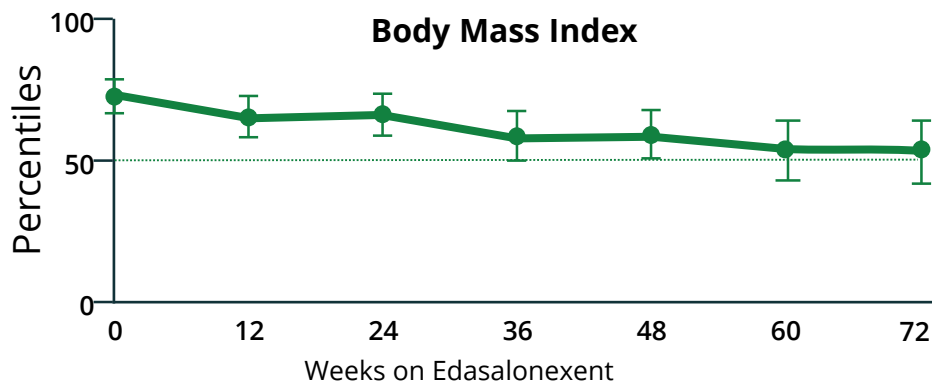


- ▶ Cardiac failure is a leading cause of mortality in DMD
- ▶ In young boys, tachycardia is the first manifestation of cardiac disease in patients with DMD
- ▶ In MoveDMD trial, ECG heart rate significantly decreased from 99 to 93, toward age-normative values of ~92 beats per minute
- ▶ In DMD, fibrosis leads to cardiac dysfunction
 - In *mdx* mouse and GRMD dog, edasalonexent reduced cardiac fibrosis

Edasalonexent Has Been Well Tolerated with No Safety Signals

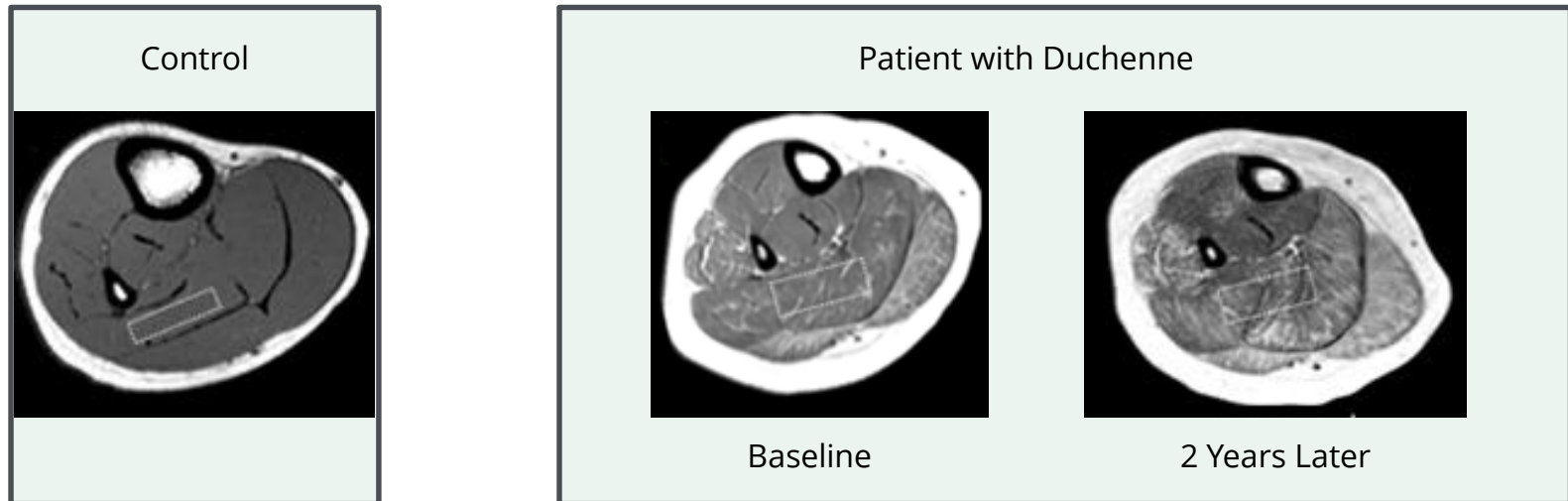
- ▶ No safety signals to date
- ▶ Well tolerated, with majority of adverse events being mild in nature, mostly gastrointestinal
- ▶ No adverse trends in hematology, chemistry, renal or adrenal function, calcium and phosphate
- ▶ Growth: Age-appropriate increases in weight and height
 - Favorably differentiated from typical profile associated with corticosteroid standard of care, which includes weight gain and curtailed growth

Percentiles Compared to CDC Growth Charts



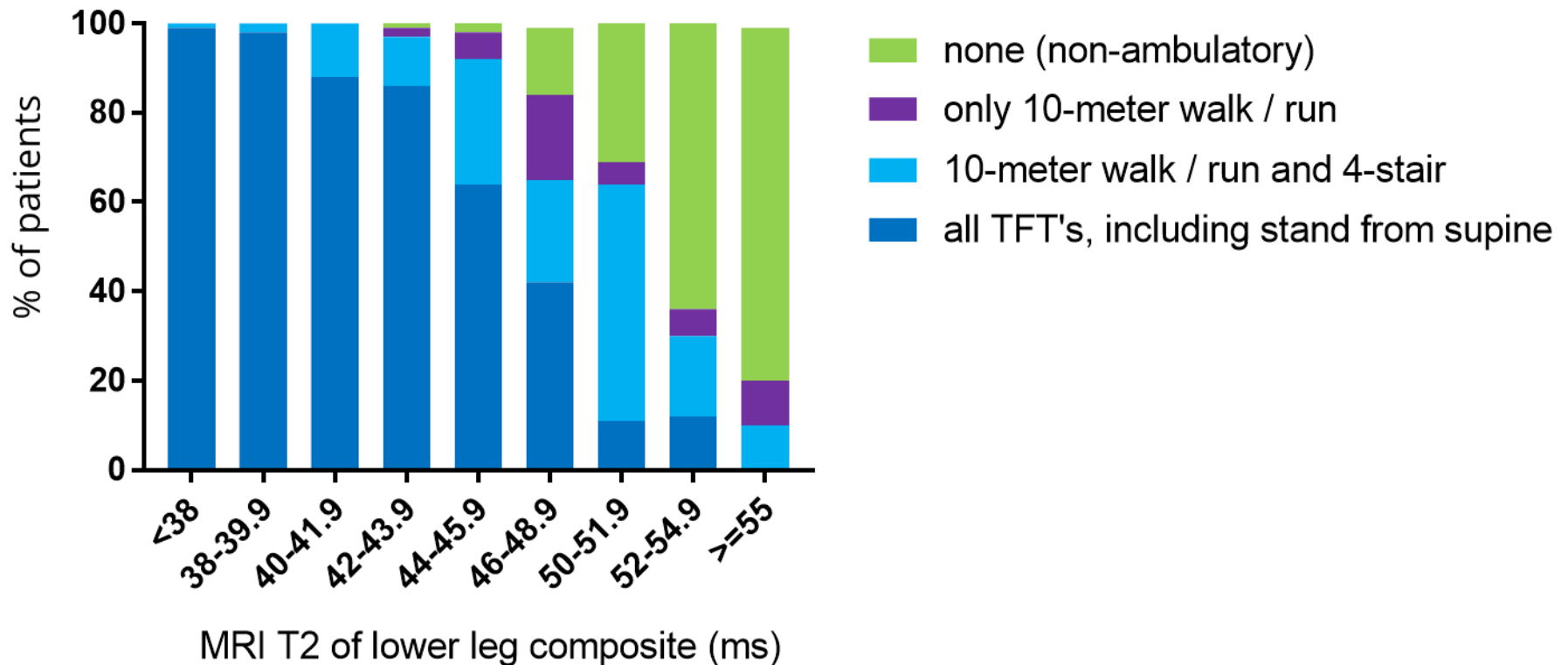
MRI Is a Non-Invasive Approach to Assess Disease Progression in Duchenne

- ▶ **MRI parameters are attractive surrogate endpoints because they are objective and non-effort dependent and correlate with disease progression**



- ▶ **MRI T2 and MRS fat fraction:**
 - Elevated in young boys with DMD and increase with age
 - Changes correlate with speed of functional measures
 - Changes correlate with loss of functional milestones
 - Responsive to therapeutic intervention
 - Predict future loss of function

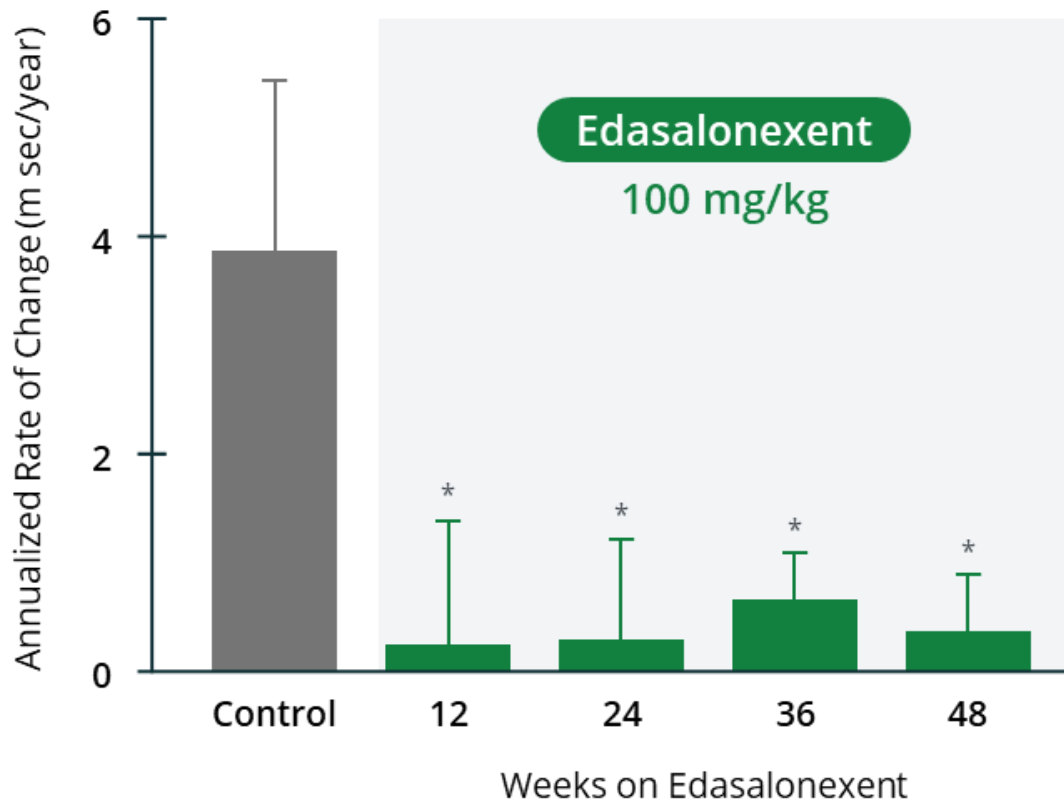
Loss of Functional Milestones Correlates with Lower Leg Composite MRI T2



- ▶ A 2 millisecond increase in MRI T2 was associated with an appreciable change in the ability to perform timed function tests

Significantly Improved Rate of Change of Lower Leg Composite MRI T2 on Edasalonexent Treatment

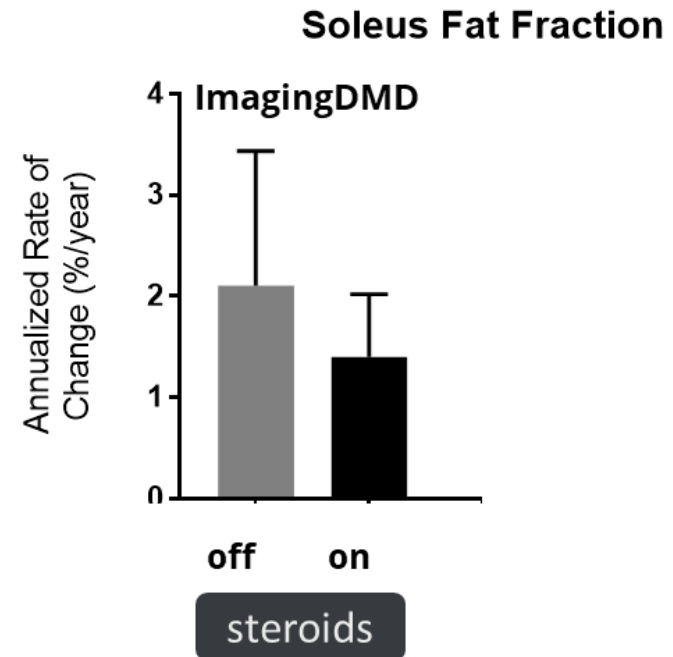
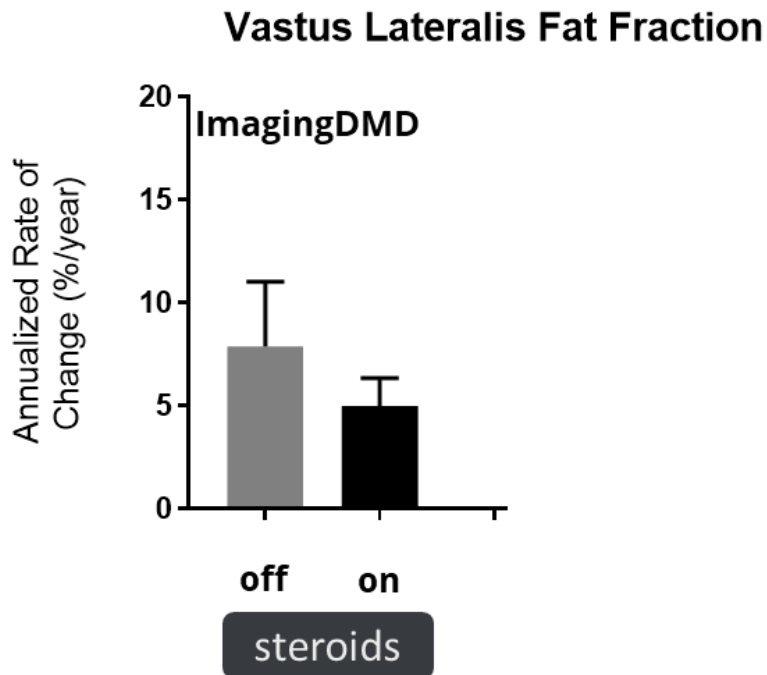
MRI T2: Composite of 5 Lower Leg Muscles



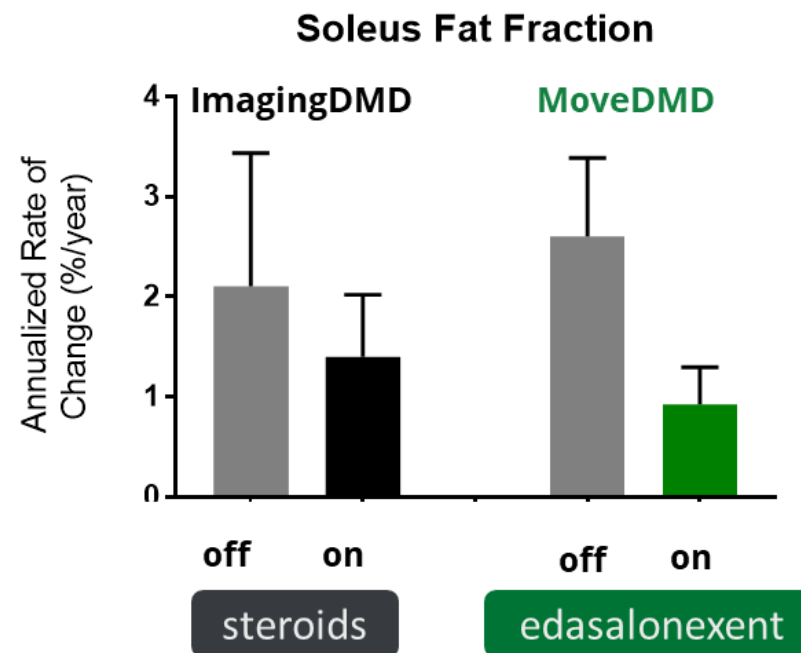
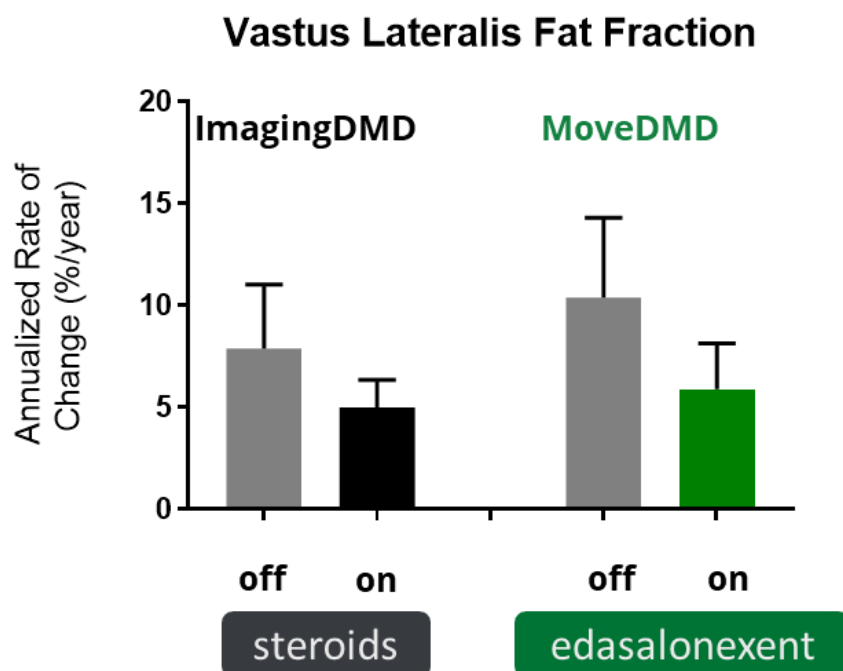
- Stabilization of MRI T2 is consistent with slowing of disease progression also observed in function assessments

Rate of Muscle Fat Accumulation Decreased with Steroid Treatment

► ImagingDMD natural history study



With Edasalonexent Treatment, the Rate of Muscle Fat Accumulation Also Decreased



Preclinical Collaboration to Study Potential Cardiac Benefits of Edasalonexent with UT Southwestern



► Collaboration overview

- Explore the potential benefits of edasalonexent on cardiac function in Duchenne and Becker muscular dystrophies
- Cardiomyopathy is the leading cause of mortality in Duchenne and Becker muscular dystrophies



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Co-Director, UT Southwestern Senator Paul D. Wellstone
Muscular Dystrophy Cooperative Research Center

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