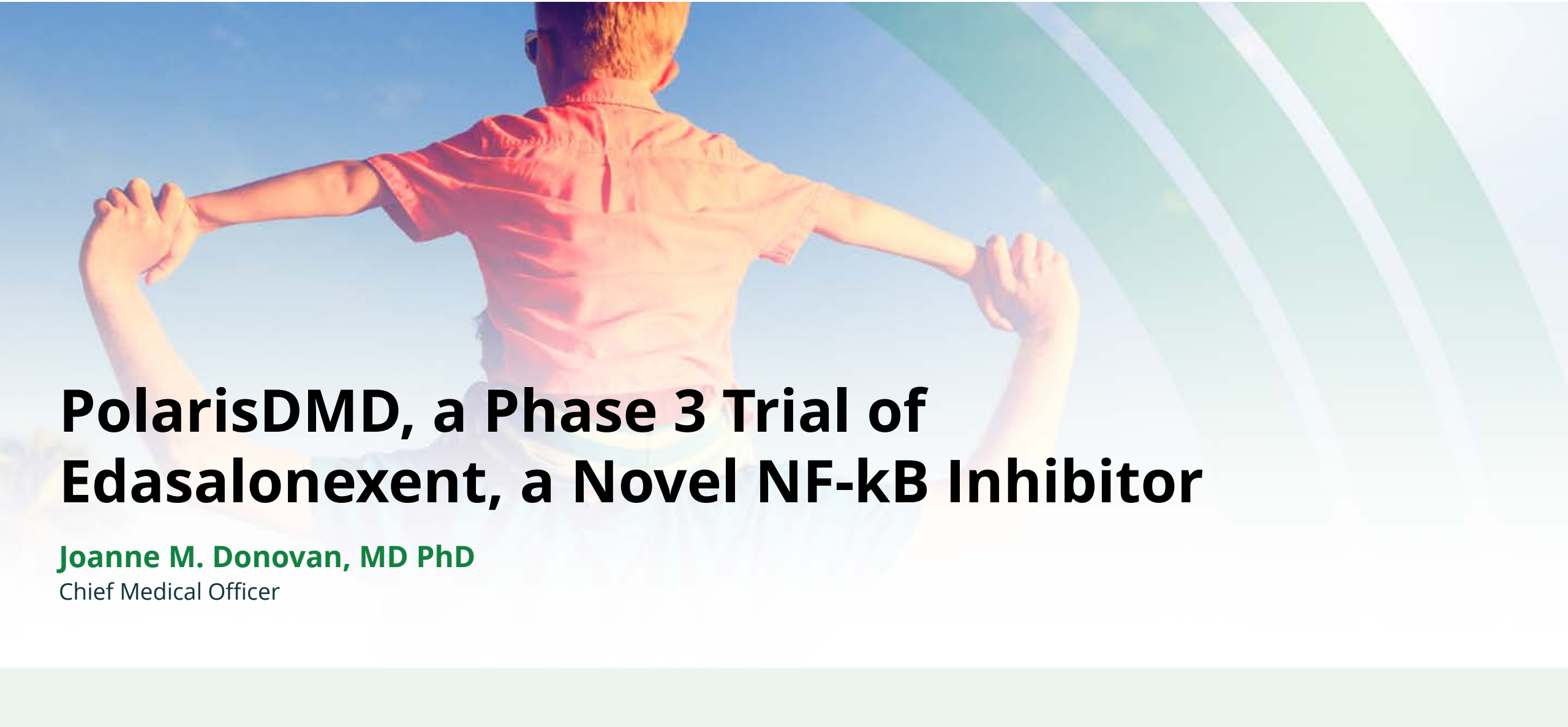
17 February 2019



PolarisDMD, a Phase 3 Trial of Edasalonexent, a Novel NF-kB Inhibitor

Joanne M. Donovan, MD PhD
Chief Medical Officer

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.

Edasalonexent: Potential to Slow Disease Progression for All Those Affected by Duchenne

- Investigational oral disease-modifying agent for all patients with Duchenne, regardless of mutation type: potential for new standard of care
- Edasalonexent substantially slowed disease progression compared to control
- Potential treatment alone and also exploring potential to combine with dystrophin-targeted therapies

POLARIS DMD

NF-kB Inhibition Provides Potential for Broad Therapeutic Benefit in Muscular Dystrophy

Activated NF-kB leads to disease progression in DMD

Skeletal Muscle

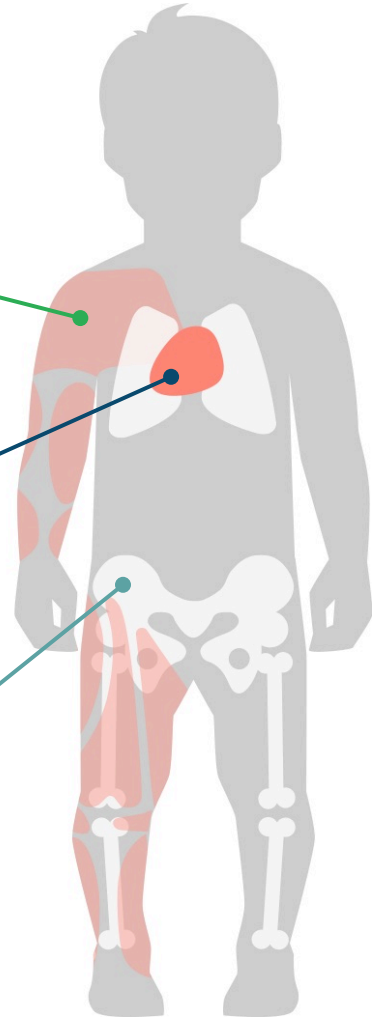
Loss of ambulation, upper limb function, respiratory failure

Heart

Cardiomyopathy

Bone

Fractures



Vision for edasalonexent, an NF-kB inhibitor

Improve
skeletal muscle function

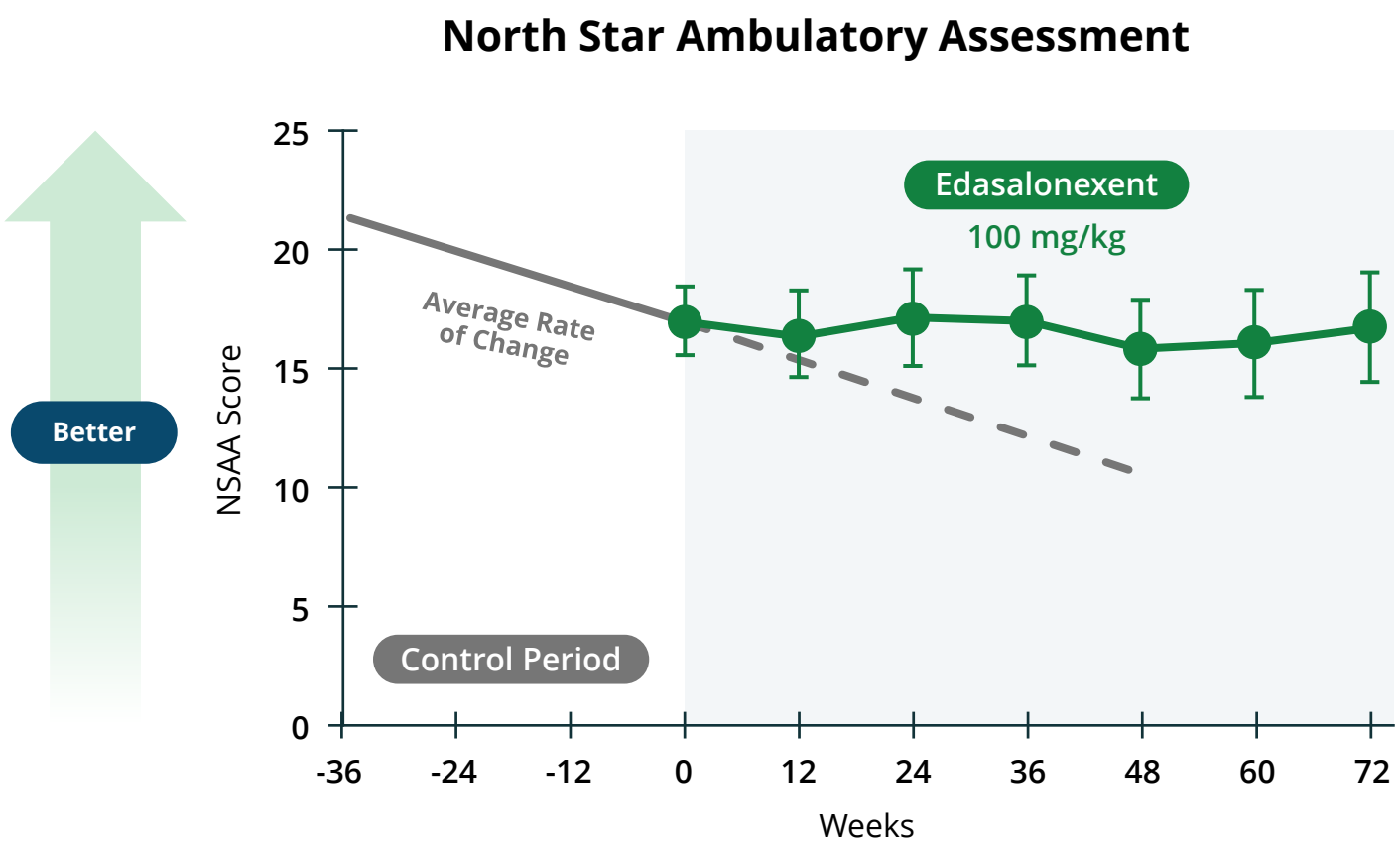
Improve
cardiac function

Reduce
risk of fractures

Promising Clinical Results Seen to Date with Edasalonexent in MoveDMD Trial

NF-κB Target Engagement	Biomarker Improvements	Muscle MRI Improvements	Functional Improvements
✓ Inhibition of NF-κB **Population:** **4 to 7 year old boys not on steroids**	✓ Decrease in CRP, biomarker of inflammation ✓ Decrease in muscle enzymes ✓ Heart rate decrease to age-normative values	✓ Improvement in rate of change in MRI T2 compared with the rate of change during the off-treatment period ✓ Decrease in muscle fat accumulation	✓ Preservation of function as assessed by North Star Ambulatory Assessment and Timed Function Tests compared with rate of change during off-treatment control period

North Star Ambulatory Assessment Score Stabilized with Edasalonexent Treatment



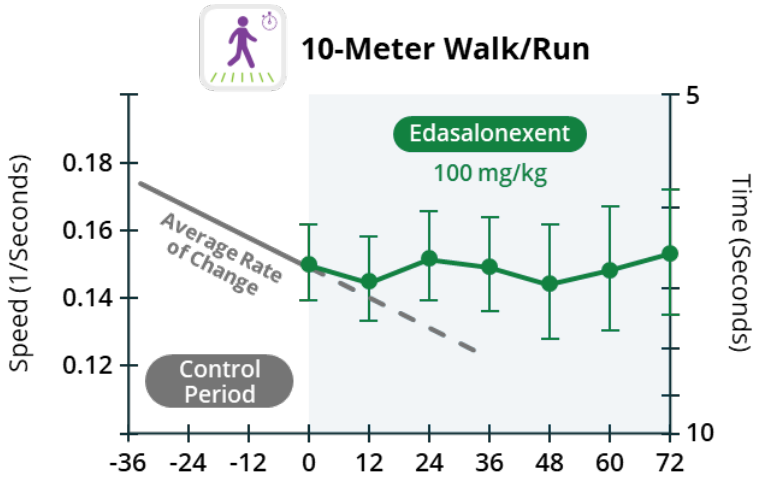
- **Disease progression on edasalonexent improved compared with average rate of change during off-treatment control period**

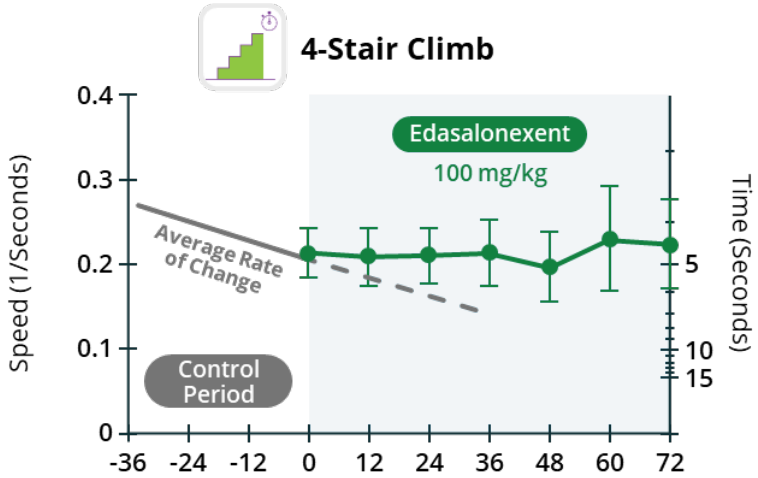
Means ± SEM shown
Includes data of all boys initially started on 100 mg/kg dose (n=16)

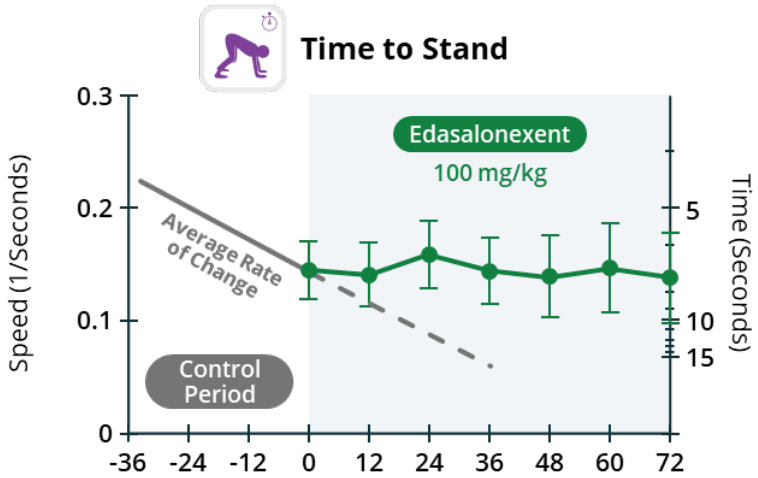
All Timed Function Tests Speed Stabilized with Edasalonexent Treatment

Pre-Specified Analyses

Better







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

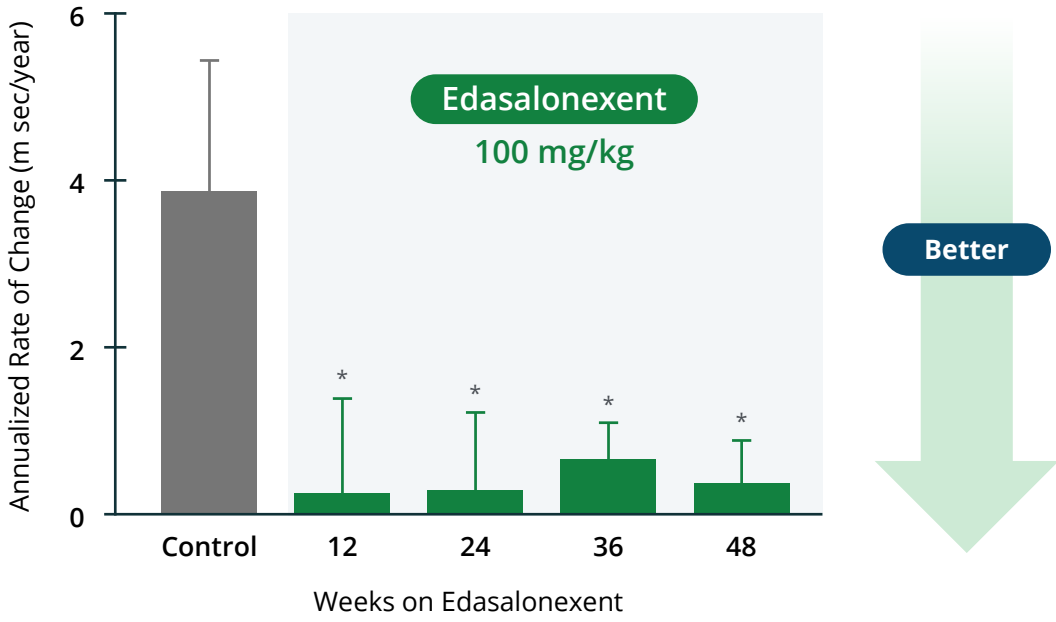
Week	0	12	24	36	48	60	72
N =	**16**	**16**	**14**	**13**	**13**	**13**	**12**

Means ± SEM shown
Includes data of all boys initially started on 100 mg/kg dose (n=16)

Edasalonexent Significantly Improved Rate of Change of MRI T2

- Following 48 weeks of edasalonexent, the rate of increase in fat fraction of the soleus and vastus lateralis was also substantially decreased as compared to the rate of increase during the off-treatment control period

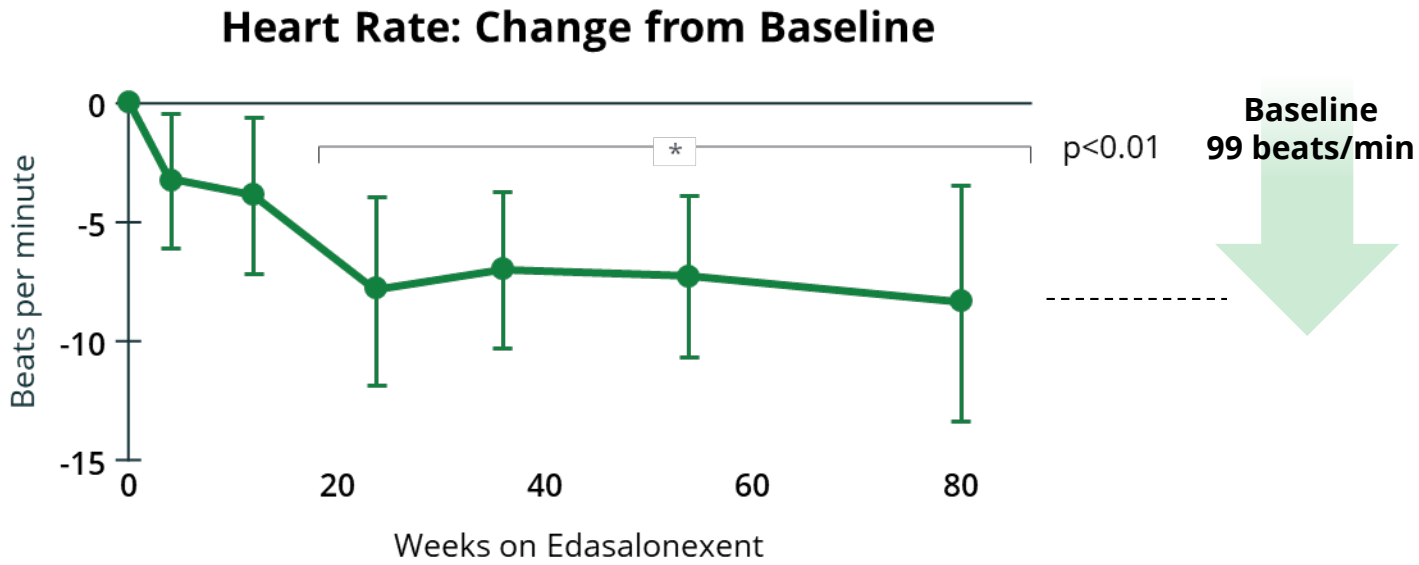
MRI T2: Composite of 5 Lower Leg Muscles



Means ± SEM shown
* p<0.05 for mixed model comparison with off-treatment period
Willcocks, et al, 2016, *Ann Neurol.*

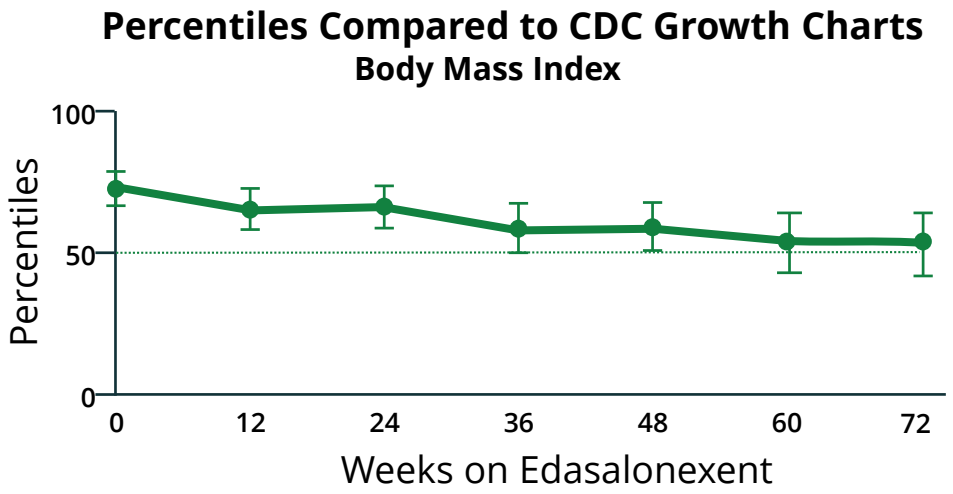
Edasalonexent Significantly Improved Biomarkers

- Significantly improved CRP and all muscle enzymes, including CK
- Boys affected by Duchenne have elevated heart rates and edasalonexent treatment decreased heart rate towards age-normative values

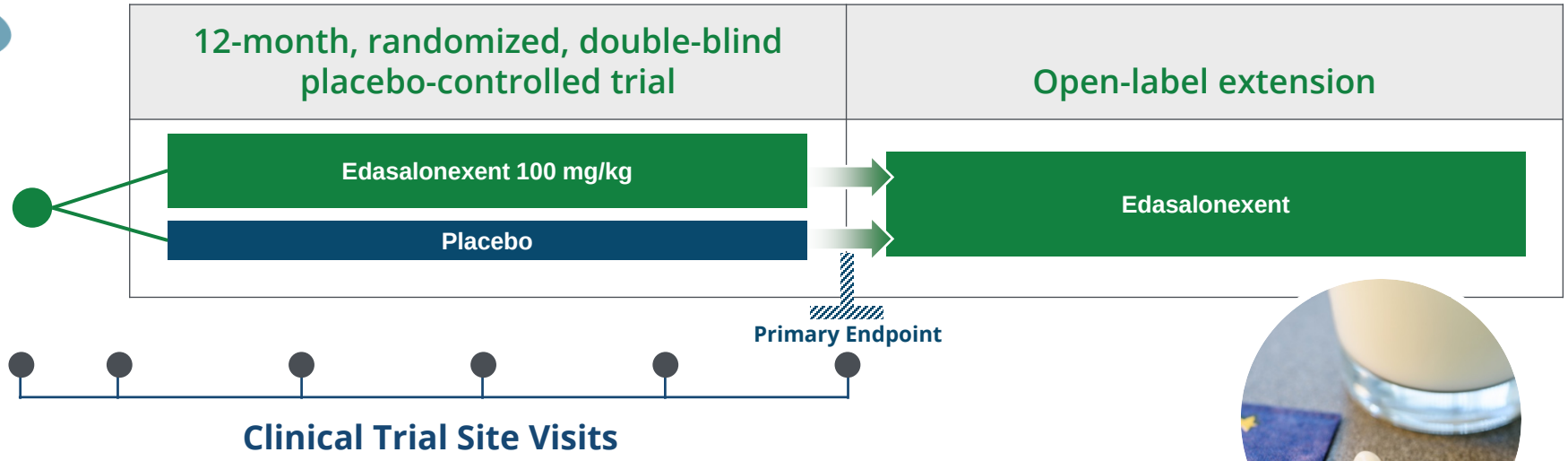


Edasalonexent Is Well Tolerated, with No Safety Signals or Steroid-Associated Side Effects

- No safety signals in 50+ years of patient exposure
- Well tolerated, with majority of adverse events mild in nature
- Boys on edasalonexent grow similarly to unaffected boys
  - Height increased by an average of 5.3 cm/year, while weight increased by an average of 1.3 kg/year, so BMI decreased
  - Favorably differentiated from excess weight gain and curtailed growth seen with corticosteroid standard of care

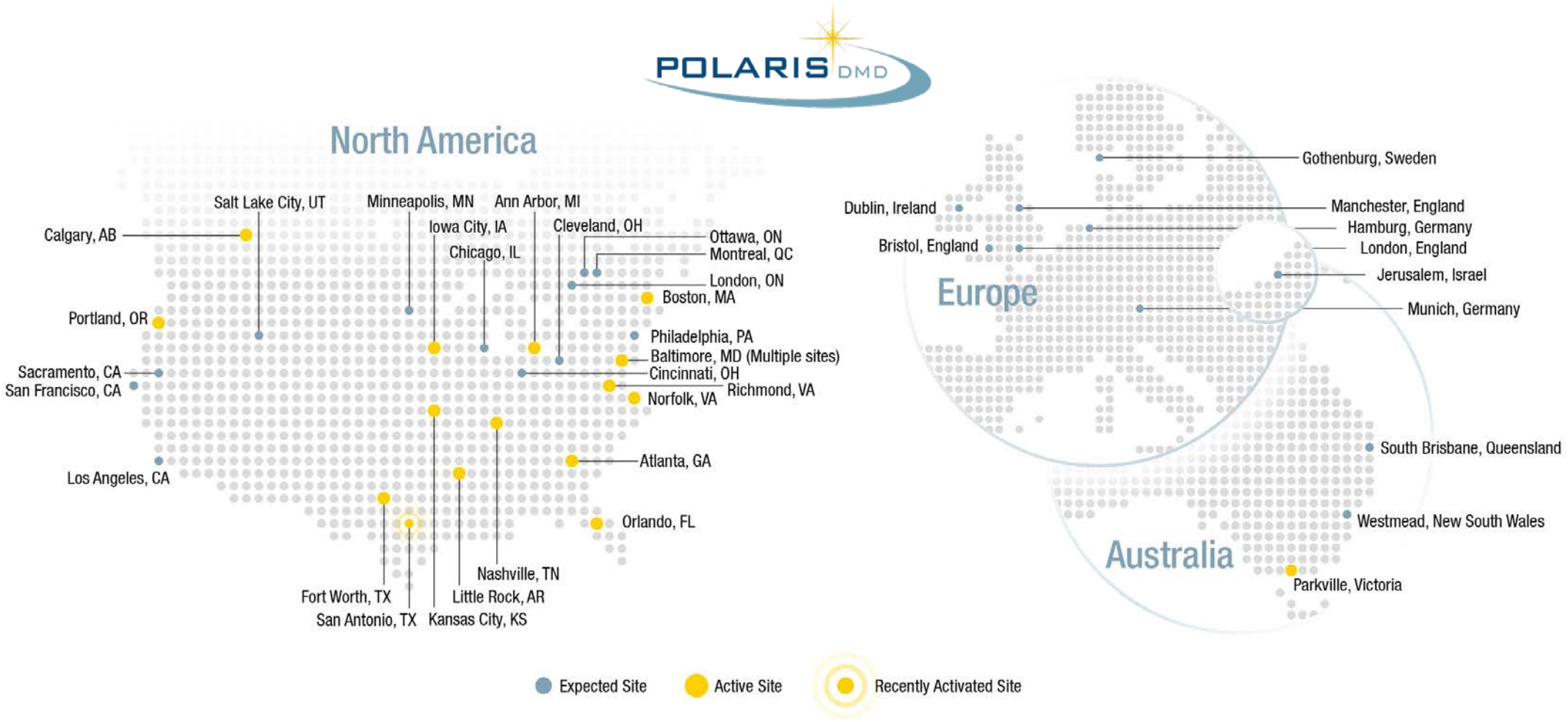


Design of Phase 3 PolarisDMD Trial



- **Key enrollment criteria**
  - Age 4 to 7 (up to 8th 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 or 6 minute walk test

PolarisDMD Global Clinical Trial Sites



Nearly 40 sites globally, many sites active and enrolling patients

Edasalonexent Is a Potential Disease Modifying Oral Therapy

Our Vision for Edasalonexent:

- For all patients, regardless of mutation, from time of diagnosis throughout their lifetime
- Address 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 Duchenne

Grazie Mille!

- **Patients and families**
- **Patient groups**
- **University of Florida**
  - Krista Vandenborne 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
- **Oregon Health & Science University**
  - Erika L. Finanger, MD
  - Bill Rooney, PhD
- **Children's Hospital of Philadelphia**
  - Gihan I. Tennekoon, MD
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Duchenne Parent Project onlus