Edasalonexent (CAT-1004)

Oral small molecule designed to inhibit NF-κB for the treatment of Duchenne muscular dystrophy

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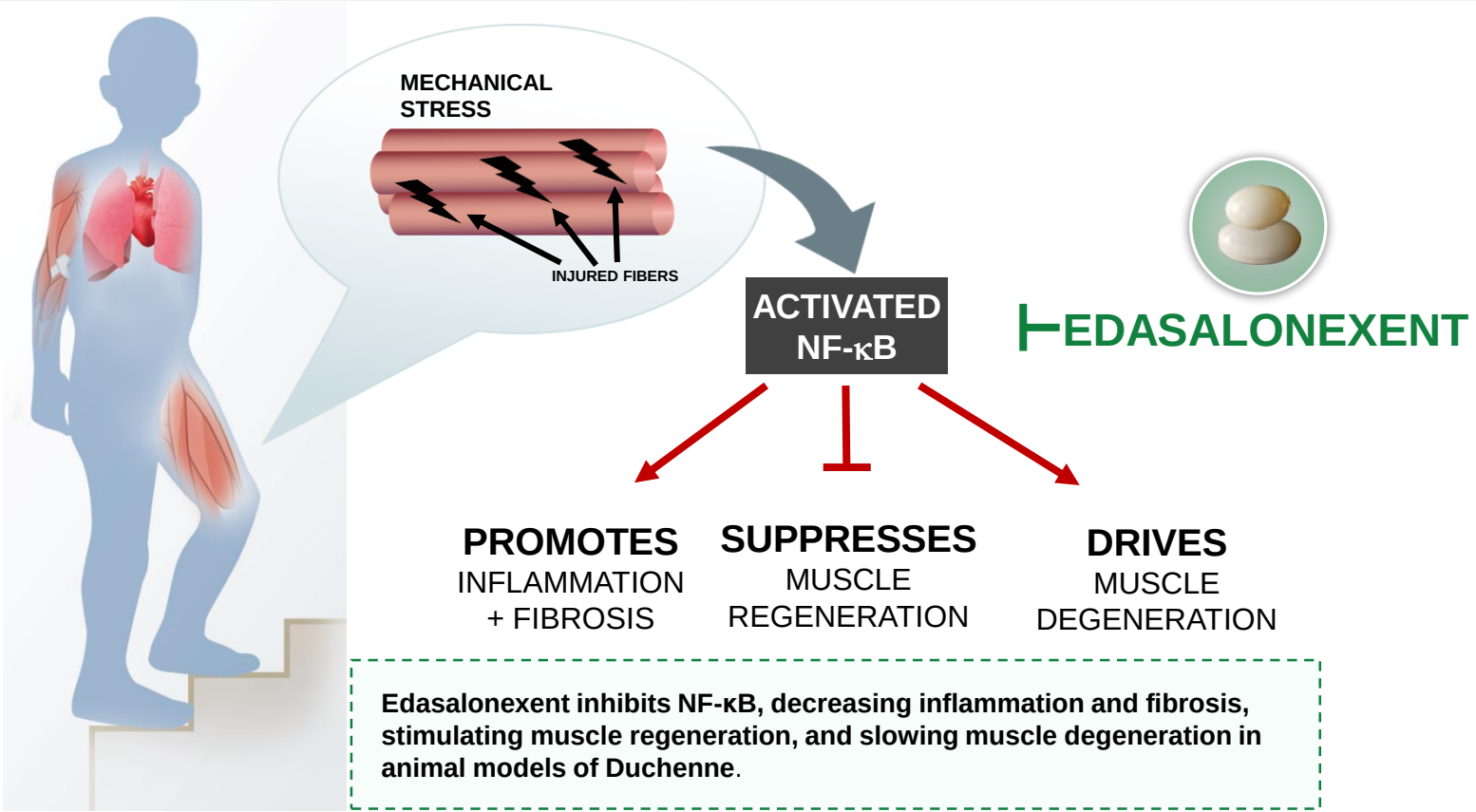
CMO, Catabasis Pharmaceuticals
June 29, 2018

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 plans to commence a 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, including the final trial design of our planned Phase 3 trial in DMD; 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; 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 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 March 31, 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 Inhibits NF-κB and Slows Muscle Degeneration and Stimulates Muscle Regeneration



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

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

Edasalonexent: Translation from Target Engagement to Functional Improvements in Duchenne

NF-κB Target Engagement

Phase 1 Normal Healthy Volunteers

- Decrease in activated NF-κB
- Decrease in NF-κB gene expression

MoveDMD Phase 1

- Decrease in NF-κB gene expression

Biomarker Improvements

MoveDMD Phase 2 / OLE

- Decrease in C-reactive protein
- Decrease in muscle enzymes

Muscle Improvements

MoveDMD Phase 2 / OLE

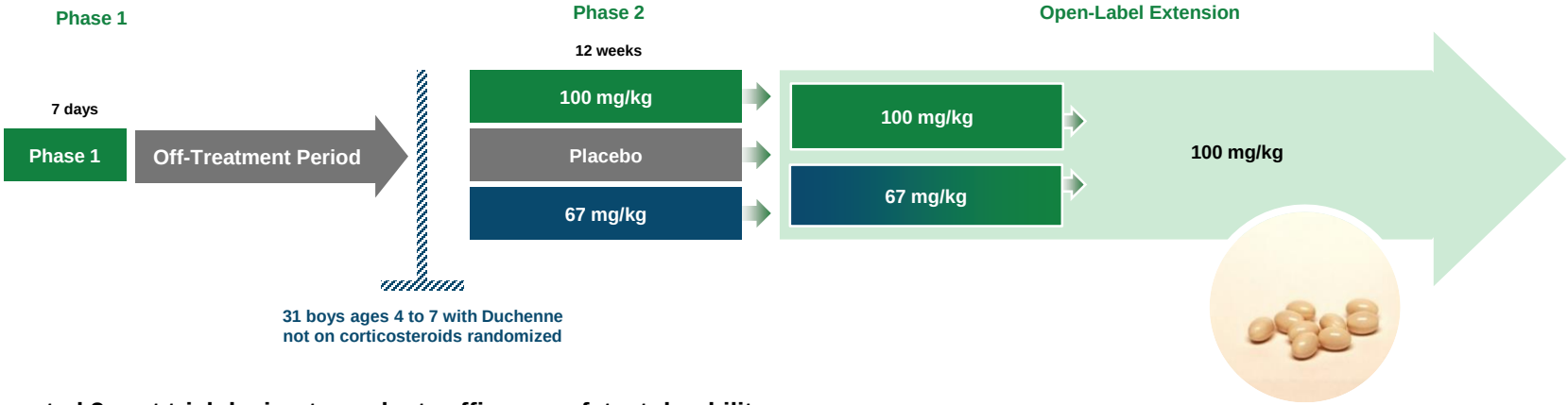
- Improvement in rate of change in MRI T2 compared to control
- Decrease in soleus and vastus lateralis fat accumulation compared to control

Functional Improvements

MoveDMD Phase 2 / OLE

- Slowing of decline in function as assessed by NSAA and Timed Function Tests compared to control

MoveDMD Trial Design



- **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 pre-specified MoveDMD analyses
  - To confirm consistency of patient off-treatment control period disease progression with available natural history data
- **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**

MoveDMD Trial Endpoints: Multiple Measures of Physical Function and Biomarkers

Assessments of Physical Function*

North Star Ambulatory Assessment

17 assessments, each scored 0-2. Maximum score: 34

NSAA Score	Perform (2)	Perform with difficulty (1)	Unable to perform (0)
	Patient with Function		Complete Loss of Function
Most Difficult Lost Early	Hop right leg		
	Hop left leg		
	Stand on heels		
	Rise from floor		
	Run		
	Jump		
	Lift head		
	Descend box step right		
	Descend box step left		
	Climb box step right		
	Climb box step left		
	Stand on one leg right		
	Stand on one leg left		
	Get to sitting		
	Rise from chair		
Least Difficult Lost Late	Walk		
	Stand		

3 Timed Function Tests

Time to Stand

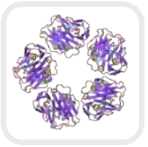
4-Stair Climb

10-Meter Walk/Run

Non-Effort Based Assessments*

MRI T2 and Fat Fraction

Muscle Enzymes

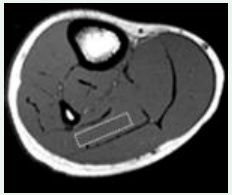


C-Reactive Protein

**Assessed before initiation of active treatment and every 12 weeks during open-label extension*

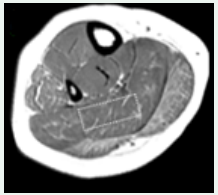
MRI is a Non-Invasive Approach to Assess Disease Progression in Duchenne

Control

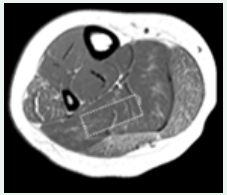


Patient with Duchenne

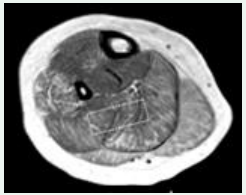
Courtesy of ImagingDMD



Baseline



1 Year Later



2 Years Later

- **MoveDMD incorporated both Magnetic Resonance Imaging (MRI) and Magnetic Resonance Spectroscopy (MRS)**
- **MRI T2 measures both inflammation and fat content**
  - MRI T2 is elevated from a young age and increases with age as fat increases
  - Changes in MRI T2 correlate with changes in function[Φ] and loss of functional milestones
- **MRS Fat Fraction measures fat content**
  - Changes in MRS Fat Fraction correlate with changes in function[Φ] and loss of functional milestones

[Φ] Willcocks et al, 2016, Ann. Neurol., Willcocks et al, 2014, Ann. Neurol

Changes in Fat Fraction on Edasalonexent Consistent with Slowing of Disease Progression

MRS Fat Fraction
Change from Baseline

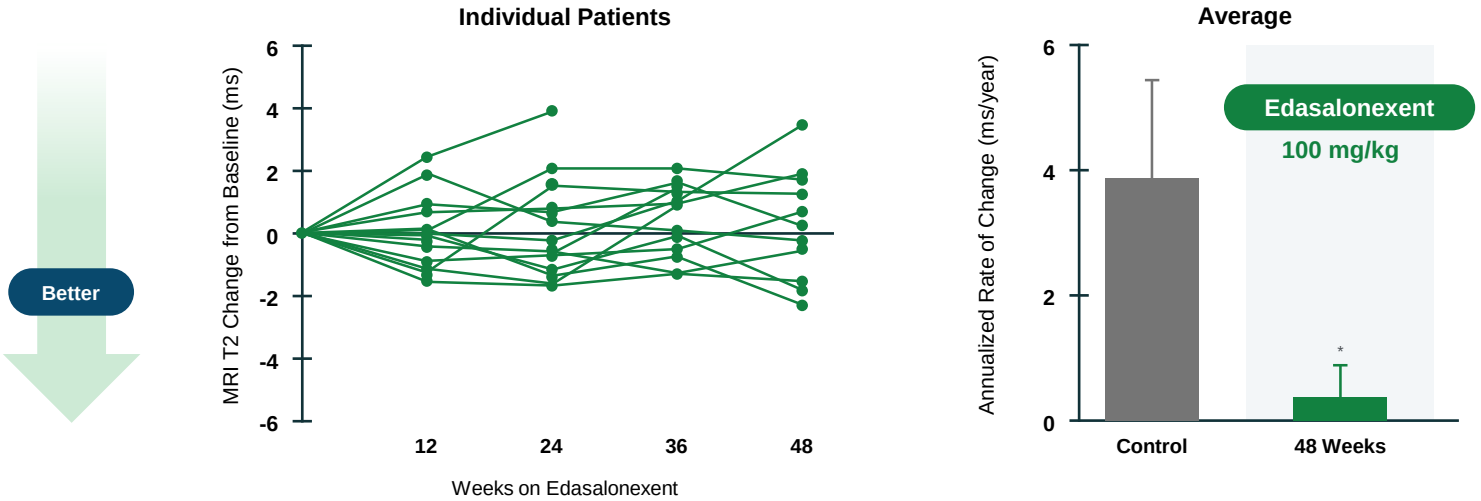
Muscle	MoveDMD Off-Treatment Control Period Annualized Rate	MoveDMD 48 weeks on Edasalonexent	ImagingDMD_Natural History Study* 1 Year Change
Soleus (calf)	2.6%	0.85%	3%
Vastus lateralis (thigh)	10.4%	5.9%	7%

- Rate of increase in Fat Fraction of the soleus and vastus lateralis was substantially decreased as compared to the off-treatment control period following 48 weeks of edasalonexent
- Increases in Fat Fraction correlate with declines in function and predict future loss of functional milestones*
- In the ImagingDMD natural history study, boys were largely on steroids

*Willcocks et al, 2016, Ann. Neurol., Willcocks et al, 2014, Ann. Neurol

Edasalonexent Significantly Improved Rate of Change of MRI T2

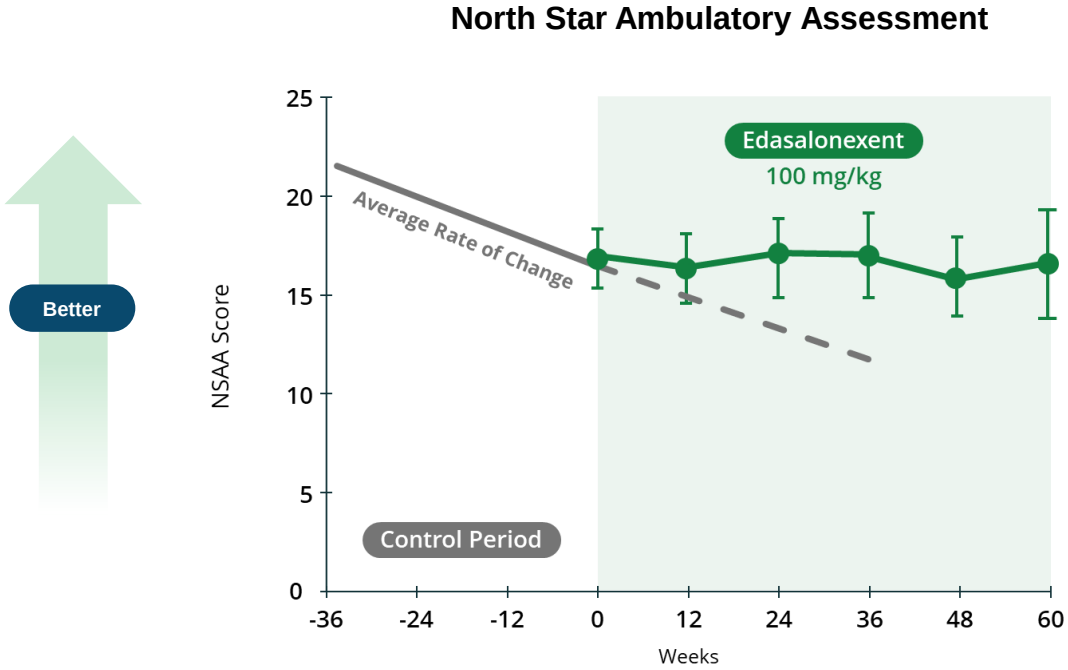
MRI T2: Composite of 5 Lower Leg Muscles



- On edasalonexent, the rate of change for the MRI T2 of lower leg muscles improved significantly compared to the rate of change during the off-treatment control period Φ
- Stabilization of MRI T2 is consistent with slowing of disease progression also observed in functional assessments

Means ± SEM shown; * p<0.05 for repeated measure mixed model comparison with off-treatment period; Φ p<0.05 for 12, 24, 36 and 48 weeks

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

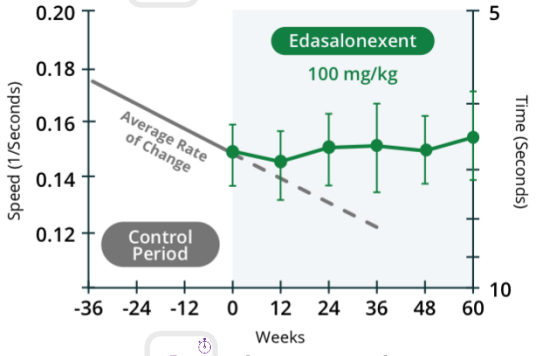
Means ± SEM shown

All Timed Function Test Speeds Stabilized with Edasalonexent Treatment

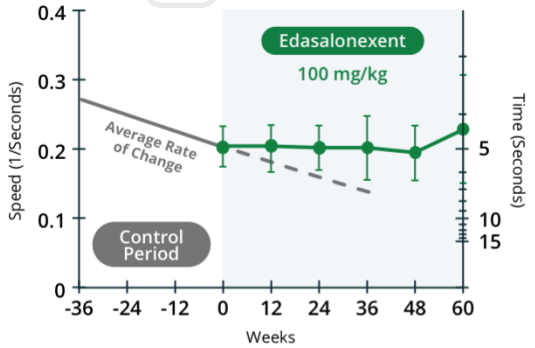
Pre-Specified Analyses

Better

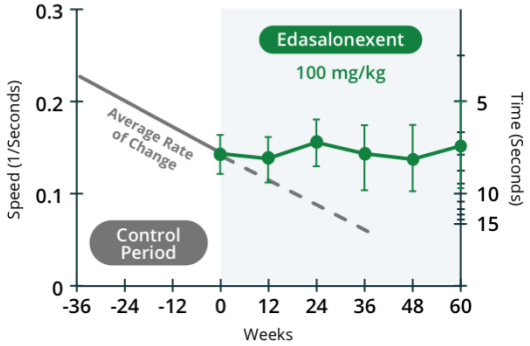
10-Meter Walk/Run



4-Stair Climb



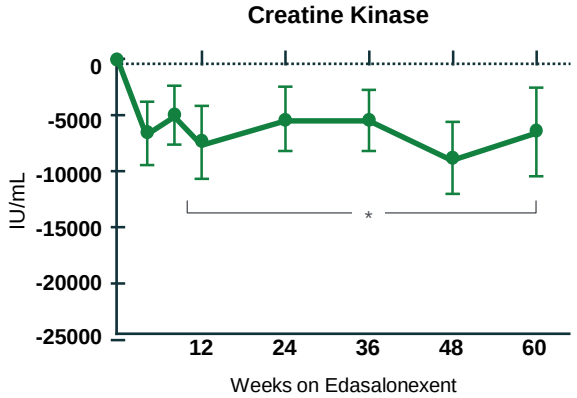
Time to Stand



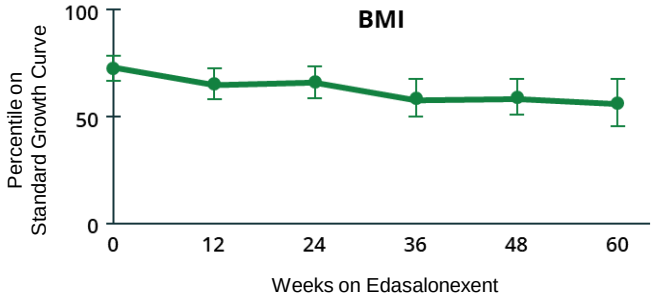
- Disease progression on edasalonexent improved compared with average rate of change during off-treatment control period

Means ± SEM shown

Edasalonexent: Well Tolerated Without Safety Signals



* p<0.05 for change from baseline after 12 weeks



- No safety signals in MoveDMD trial 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
- Heart rate decreased toward normal values at this age

Summary: Edasalonexent Substantially Slowed Predicted Disease Progression in MoveDMD Study

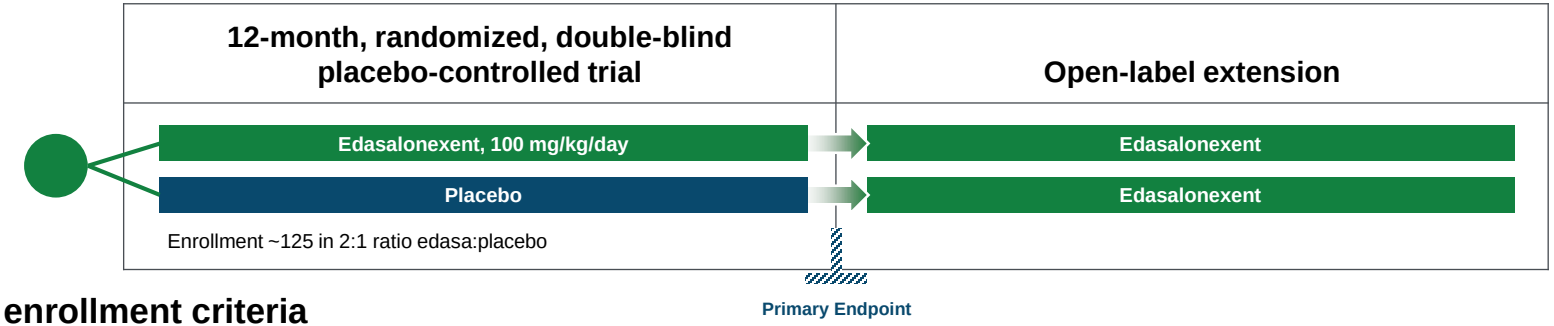
- **Clinically meaningful slowing of disease progression on edasalonexent over more than 1 year compared to off-treatment control period**
  - North Star Ambulatory Assessment stabilized
  - All timed function tests stabilized (10-meter walk/run, 4-stair climb and time to stand)

- **MRI measures support positive edasalonexent treatment effects over 48 weeks**
  - Muscle MRI T2 significantly improved during edasalonexent treatment versus off-treatment control period progression
  - Increases in Fat Fraction decreased compared to the off-treatment control period and to that expected for natural history on corticosteroids

- **No safety signal and well tolerated over more than 1 year**
  - Height, weight and BMI growth patterns continued to be similar to unaffected boys

- **Supportive of Phase 3 clinical trial**

Positive MoveDMD Data Support
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 or 6 minute walk test
- **Expected Locations: US, Canada, Europe, Israel and Australia**

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
- Edasalonexent substantially slowed disease progression compared to control
- Preparing for Phase 3 clinical trial, POLARIS DMD
- Potential as monotherapy and also exploring potential to combine with dystrophin-targeted and other therapies

POLARIS DMD

Thank You

- **Patients and families**
- **Patient groups**
- **Nemours Children's Hospital**
  - Richard Finkel, MD
- **ImagingDMD & University of Florida**
  - Krista Vandenborne, PT PhD
  - H. Lee Sweeney, PhD
  - Rebecca J. Willcocks, PhD
  - Glenn Walter, PhD
  - Sean C. Forbes, PhD
  - William T. Triplett, BSc
- **Oregon Health Sciences University**
  - Erika L. Finanger, MD
  - William Rooney, PhD
- **The Children's Hospital of Philadelphia**
  - Gihan I. Tennekoon, MD
  - Sabrina W. Yum, MD
- **University of California**
  - Perry Shieh, MD PhD
- **Catabasis Pharmaceuticals**
  - Maria Mancini, MHP
  - Angelika Fretzen, PhD
  - Pradeep Bista, PhD
  - Andrew Nichols, PhD
  - James MacDougall, PhD

- **For Questions email: DMDTrials@catabasis.com**

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