Edasalonexent in Development for the Treatment of Duchenne: Information on Three Clinical Trials

Erika Finanger, M.D., ***Associate Professor of Pediatrics, Division of Neurology, School of Medicine at Oregon Health & Science University and Principal Investigator for the PolarisDMD, GalaxyDMD and MoveDMD trials***

Joanne Donovan, M.D., Ph.D., ***Chief Medical Officer, Catabasis***

Maria Mancini, MHP, ***Vice President, Clinical Operations, Catabasis***

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 PolarisDMD 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, and our plans to combine edasalonexent treatment with other DMD treatments such as gene therapy and other dystrophin-targeted approaches. 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 Annual Report on Form 10-K for the period ended December 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.

Disclosures

- Joanne Donovan M.D., Ph.D. and Maria Mancini, MHP are employees of Catabasis
- Dr. Erika Finanger, M.D. has received research grants from Catabasis, and has served on a Catabasis SAB
- Edasalonexent is an investigational drug that is not yet approved in any territory

Today's Webinar Will Include

An update on the currently enrolling global Phase 3 PolarisDMD trial

An introduction to the new open-label extension GalaxyDMD trial

Results from the Phase 2 MoveDMD trial and open-label extension

Q&A

Catabasis' Focus on Edasalonexent for Duchenne

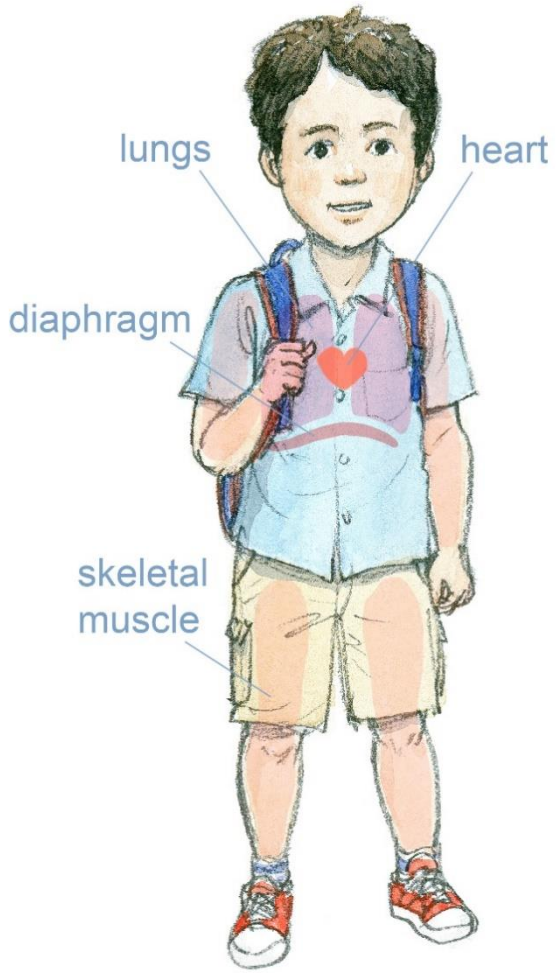
- Catabasis is a biotech company in Cambridge, MA whose mission is to bring hope and life-changing therapies to patients and their families.
- Our goal is for edasalonexent to become an oral new foundational therapy to slow disease progression for all people affected by Duchenne at all ages as a single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

POLARIS DMD

- **PolarisDMD, a Phase 3 clinical trial of edasalonexent, is enrolling boys with Duchenne**

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

- Being developed as a new oral foundational therapy for all patients with Duchenne, regardless of mutation type
- Being developed for treatment alone as well as with dystrophin-targeted therapies
- In Phase 2 MoveDMD trial and open-label extension, edasalonexent substantially slowed disease progression compared to off-treatment control period
- Edasalonexent is an investigational agent not currently approved in any territory



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

Activated NF-kB leads to disease progression in DMD

Skeletal Muscle

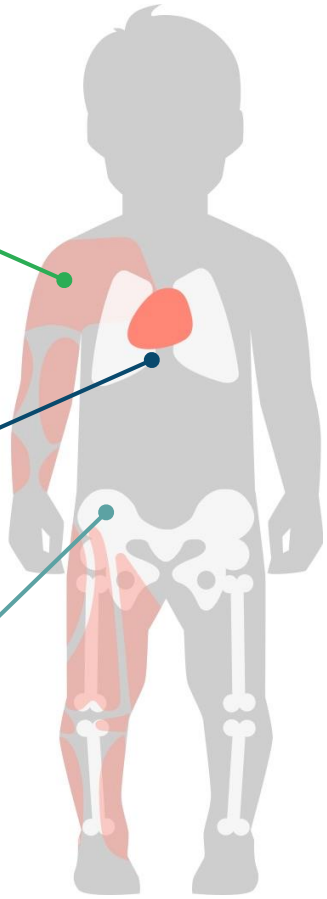
Loss of ambulation, upper limb function, respiratory failure

Heart

Cardiomyopathy

Bone

Fractures



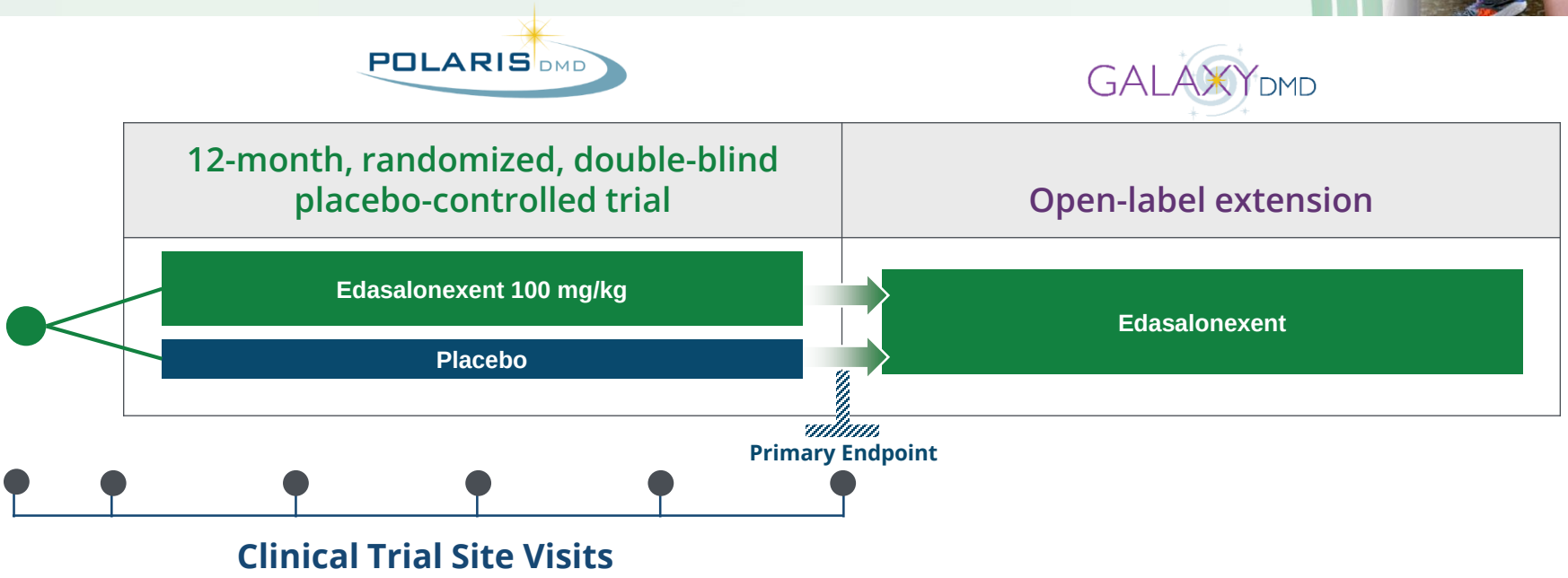
Vision for edasalonexent, an NF-kB inhibitor

Goal: Improve skeletal muscle function

Goal: Preserve cardiac function

Goal: Reduce risk of fractures

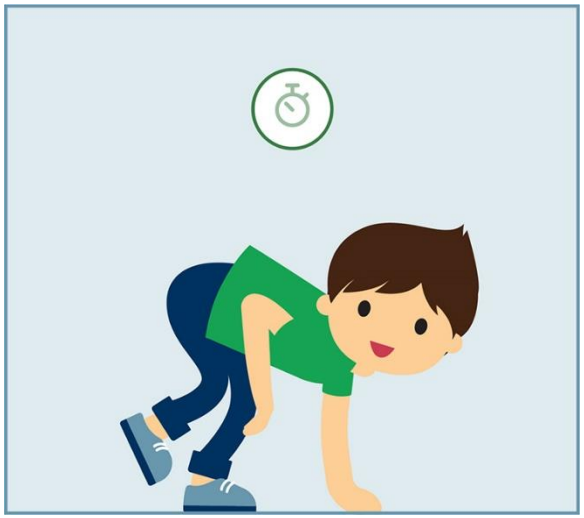
What to Expect When Participating in the Phase 3 PolarisDMD Trial

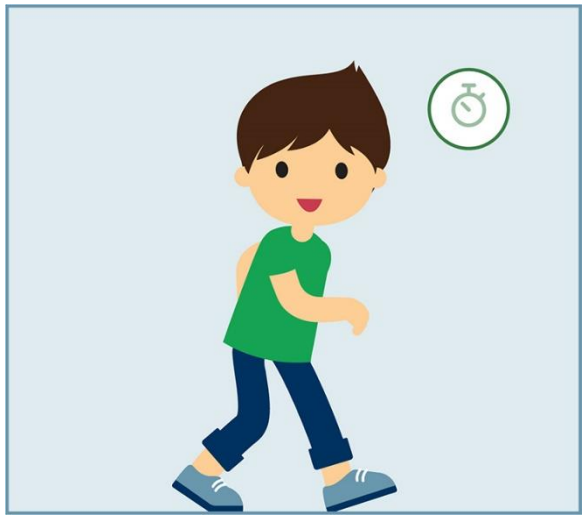


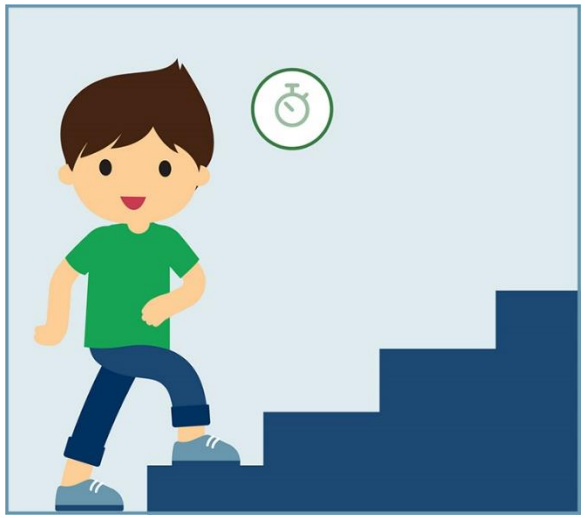
- Enrolling ~125 boys ages 4 to 7 (up to 8th birthday)
  - Not on corticosteroids for at least 6 months
- 2:1 randomization, 67% of boys receive drug initially, all boys can receive drug after 12 months
- Clinical trial site visits and key assessments every 3 months
- Safety measures including labs every 3 months
- Trial overseen by Data Safety Monitoring Board

How We Designed PolarisDMD

- **Designed the trial with input from advocacy organizations, families, physicians**
  - Understand the burden of clinical trial participation
- **NSAA and additional endpoints are measures that reflect every day life**
  - Standing up from the ground, walking, climbing stairs







PolarisDMD Trial Patient Eligibility

MAIN INCLUSION CRITERIA

- Genetic diagnosis and symptoms of Duchenne
- Able to stand from lying down in ≤ 10 seconds
- Able to swallow placebo capsules
- Boys from their 4th birthday to their 8th birthday
- Able to perform 10 meter walk/run and 4 stair climb tests
- Followed by a medical professional who coordinates Duchenne care

PolarisDMD Trial Patient Eligibility

MAIN EXCLUSION CRITERIA

- Treated with oral corticosteroids within 24 weeks; inhaled steroids or steroid creams are ok
- Within ~4 weeks: other investigational drugs or ongoing participation in any other therapeutic clinical trial
- Within 4 weeks prior to Day 1: on other specific medications listed on clinicaltrials.gov
- Within 3 months prior to Day 1: human growth hormone

EXONDYS 51® is OK if on it for at least 24 weeks

Key Assessments Performed During Clinic Visits

Primary endpoint: North Star Ambulatory Assessment

Assessment measures— from most to least difficult

Hop right leg	Climb box step right
Hop left leg	Climb box step left
Stand on heels	Stand on one leg right
Rise from floor	Stand on one leg left
Run	Get to sitting
Jump	Rise from chair
Lift head	Walk
Descend box step right	Stand
Descend box step left	

How measures are scored

2 Can perform

1 Can perform with difficulty

0 Unable to perform

Key secondary endpoints: Timed Function Tests

10-meter walk/run

4-stair climb

Time to rise from supine

Additional Assessments Will Include Growth, Cardiac and Bone Health Measures

Growth

- Monitoring height and weight to assess how boys are growing relative to their expected growth curves

Heart

- Monitoring with an easy to wear at-home small adhesive device at baseline, 6 and 12 months
- Will be analyzed for changes in heart rate as well as heart rate variability

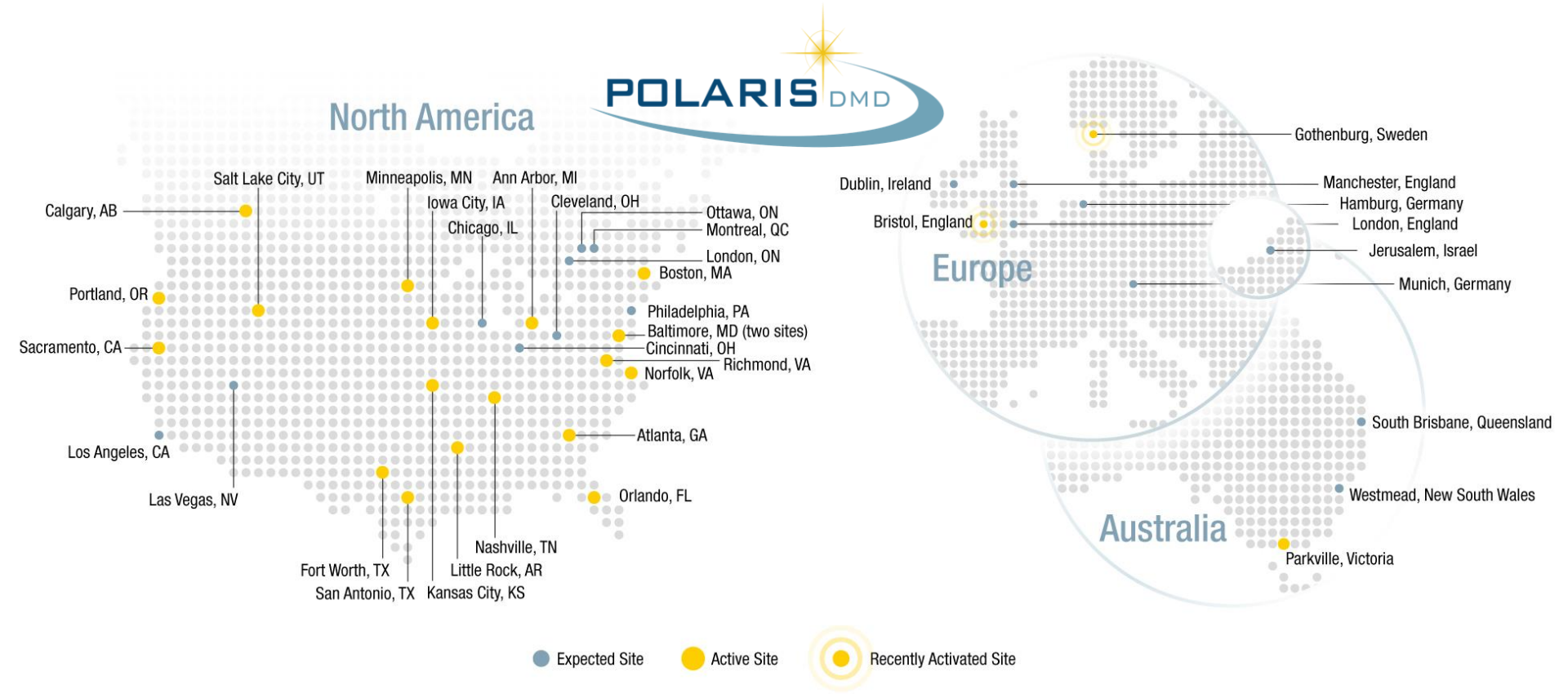
Bone

- X-rays of the spine at baseline and after one year of treatment
- Bone mineral density by DXA at baseline and after one year of treatment

In the PolarisDMD Trial Edasalonexent Is Taken as a Gel Capsule

- **Dose 100 mg/kg/day**
- **Taken 3 times per day with food**
  - Mid-day dose can be at school or at home after school
- **2 different small capsule sizes**
  - 100 mg capsules are similar to the size of a tic-tac
  - 250 mg capsules are similar to the size of a jelly bean
- **Medi-straws provided to facilitate capsule swallowing**

Clinical Trial Sites Across US, Canada, Australia, Israel and Europe, Many Open for Enrollment



Many clinical trial sites active and enrolling patients, site activations will be updated

PolarisDMD Designed to Minimize Burden of Trial Participation

John's mom or dad visits clinicaltrials.gov to see if PolarisDMD is a good fit for John based on the eligibility criteria.

His parent reaches out to DMDtrials@catabasis.com for answers to any questions and is connected with the clinical trial site that's closest to home.

Q A

John's family learns that Catabasis will pay for expenses, including flights, transportation, hotels, and meals for all site visits.

At John's Screening visit, he does timed tests like...

Standing up from the ground

Walking 10 meters

Climbing four stairs

There are no muscle biopsies or MRIs!

John meets the entry criteria during the Screening visit and returns for the **Baseline visit** where he is enrolled in the PolarisDMD trial. John starts on study drug that day (**2 boys receive edasalonexent for each boy that receives placebo**).

POLARIS DMD

John and his family return to the clinical trial site **once every three months** during the trial. John and his family talk with the site in between visits to share how they are doing in the trial.

At home, **John takes 2 or 3 capsules with food** 3 times a day throughout the trial. After completing the PolarisDMD trial, John would be eligible to participate in the GalaxyDMD study (to receive edasalonexent in an open-label extension).

An Option for 4-7 Year Old Boys Not on Steroids: Phase 3 PolarisDMD Trial

- **Reasons to consider this trial**
  - Edasalonexent substantially slowed disease progression compared to off-treatment control period in the MoveDMD study
  - Edasalonexent has been well tolerated in clinical trials to date
    - Not a steroid
    - Age-appropriate growth
  - PolarisDMD trial designed to minimize impact on families
    - Clinical trial site visits every 3 months
    - Costs for travel to the trial sites provided
  - Edasalonexent is given by mouth with food

POLARIS DMD

Launching New GalaxyDMD Trial for Boys Receiving Open-Label Edasalonexent

- **GalaxyDMD is planned to enroll boys from MoveDMD open-label extension and provide opportunity for open-label edasalonexent after completing 1-year PolarisDMD trial**
- **Ongoing monitoring with patient visits every 6 months**
  - Assessments of muscle function:
    - North Star Ambulatory Assessment
    - Timed Function Tests
  - Long term safety including growth and bone health
- **GalaxyDMD will provide continued long-term safety data**

GalaxyDMD Focuses on Long-Term Safety and Allows Sibling Participation

- **Primary focus is evaluation of long-term safety in boys as they get older**
  - Site visits every 6 months
- **Participants from MoveDMD trial transitioning to GalaxyDMD**
  - MoveDMD boys are in the process of transitioning to GalaxyDMD
  - These boys have received edasalonexent for 2+ years, average age close to 9
- **Once boys from MoveDMD and PolarisDMD enter GalaxyDMD, there will also be an opportunity for their brothers to join**
  - Age 4-10 (up to 11th birthday) and meet inclusion criteria
  - May be eligible receiving approved exon-skipping therapies

GALAXY DMD

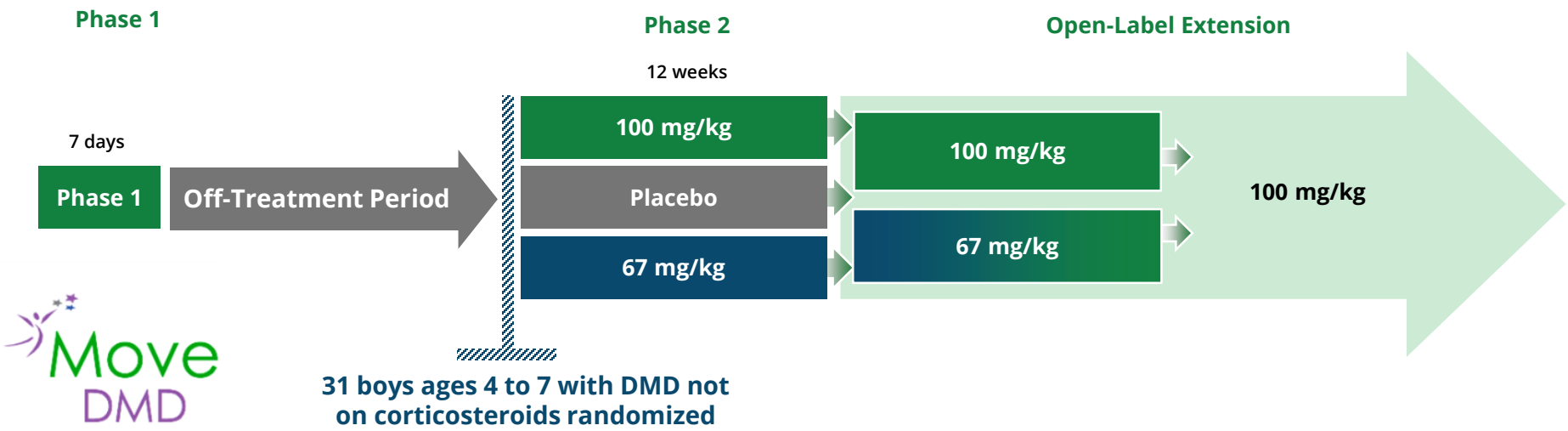
GalaxyDMD Inclusion and Exclusion Criteria

- **For boys who have been in the MoveDMD or PolarisDMD study:**
  - Inclusion: completion of the MoveDMD or PolarisDMD study

- **For siblings of boys who have completed the MoveDMD or PolarisDMD study:**
  - Inclusion: Genetic diagnosis of Duchenne, age from 4-10 (up to 11th birthday)
  - Exclusion: Use of investigational drug or growth hormone, on corticosteroids during previous 24 weeks

GALAXY DMD

Phase 2 MoveDMD Trial Designed to Inform Phase 3



- **Integrated multi-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
  - Compared off-treatment control period disease progression with available natural history data
- **Open-label extension enabled assessment of safety and efficacy following longer term treatment**

Promising Clinical Trial Results Seen to Date with Edasalonexent

NF-κB Target Engagement

- ✓ Inhibition of NF-κB
  - Changes in the activity of genes in white blood cells that are regulated by NF-κB

Move DMD

Biomarker Improvements

- ✓ Decrease in CRP, biomarker of inflammation
- ✓ Decrease in muscle enzymes
- ✓ Heart rate decrease to age-normative values

Muscle MRI Improvements

- ✓ Improvement in rate of change in MRI T2 compared with the rate of change during the off-treatment period
- ✓ Decrease in muscle fat accumulation

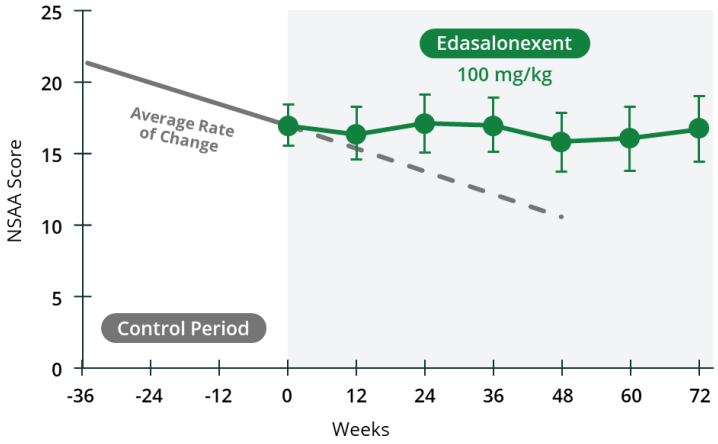
Functional Improvements

- ✓ Preservation of function as assessed by North Star Ambulatory Assessment and Timed Function Tests compared with rate of change during off-treatment control period

In the Phase 2 MoveDMD Trial and Open-Label Extension Edasalonexent Preserved Muscle Function Compared to Off-Treatment Period

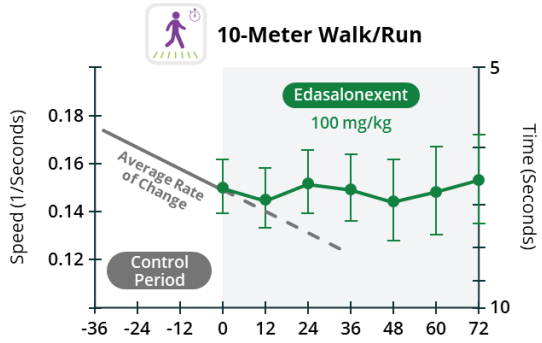
Edasalonexent Treatment Stabilized North Star Ambulatory Assessment Score

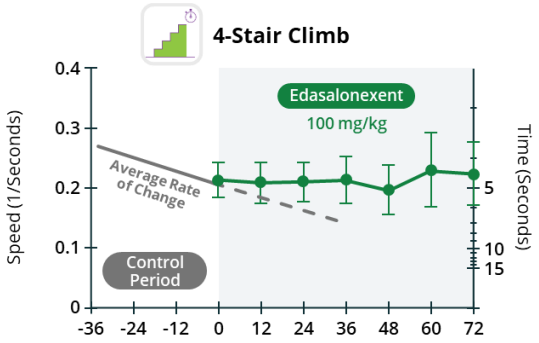
Better

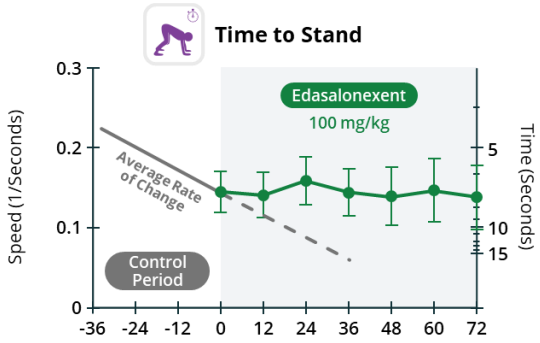


Edasalonexent Treatment Stabilized Timed Function Tests

Better





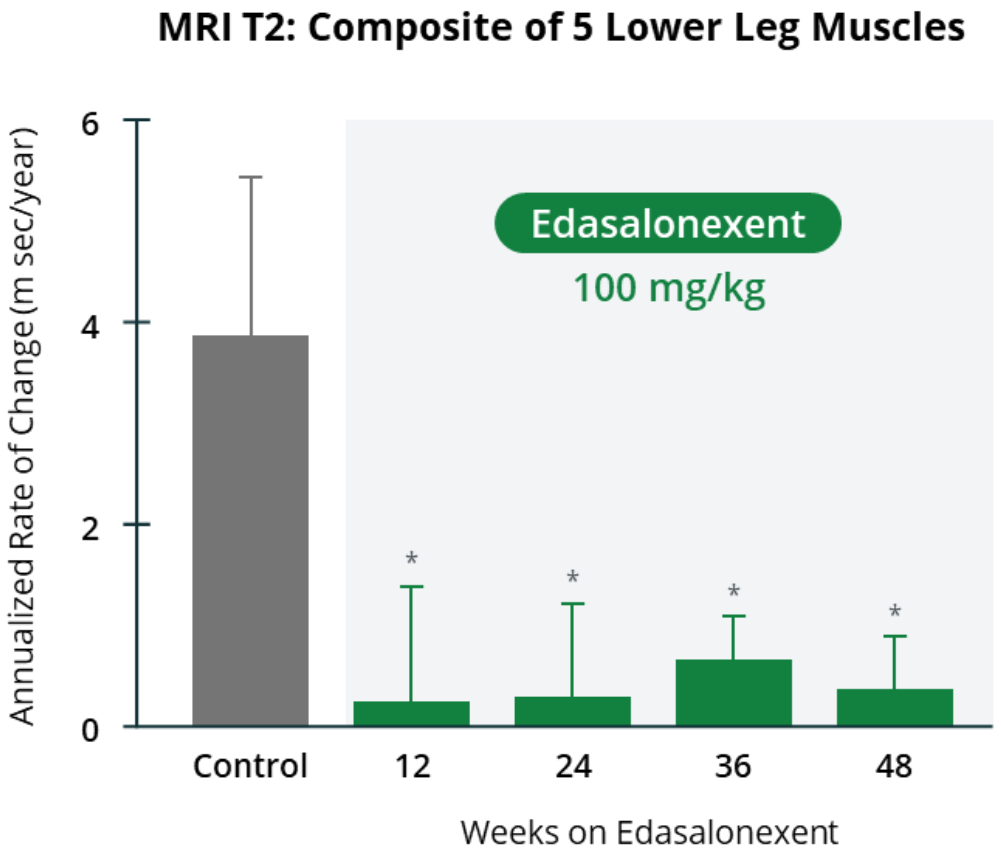


catabasis

Means ± SEM shown

Edasalonexent Improved Rate of Change of MRI T2 Compared to Rate of Change in Off-Treatment Control Period

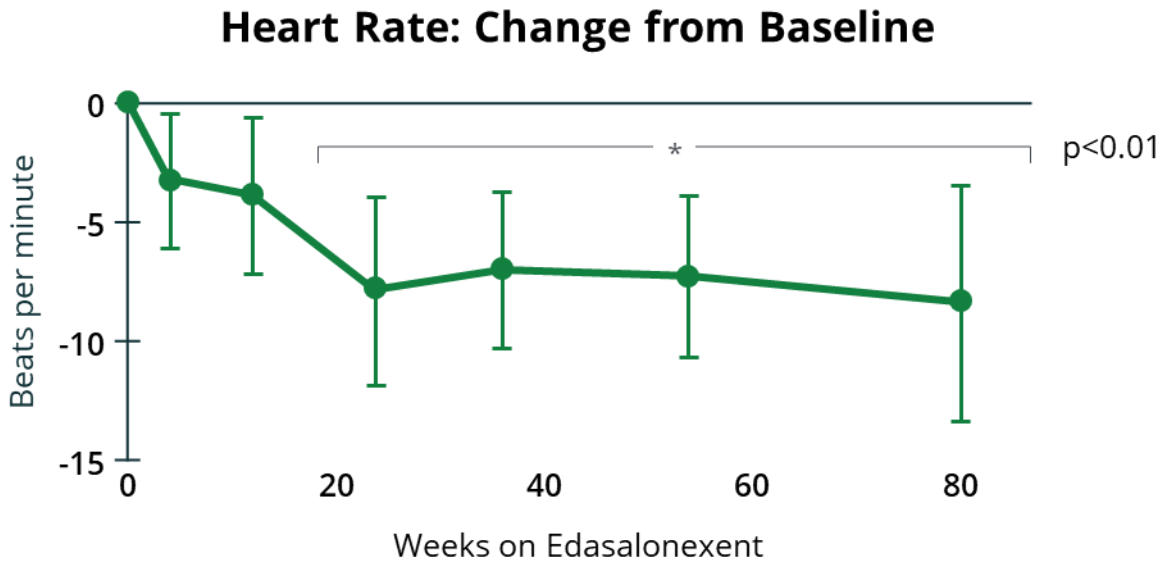
- MRI is a non-invasive approach to assess disease progression in DMD



Means ± SEM shown; * p<0.05 for mixed model comparison with off-treatment period

Edasalonexent Improved Biomarkers

- 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

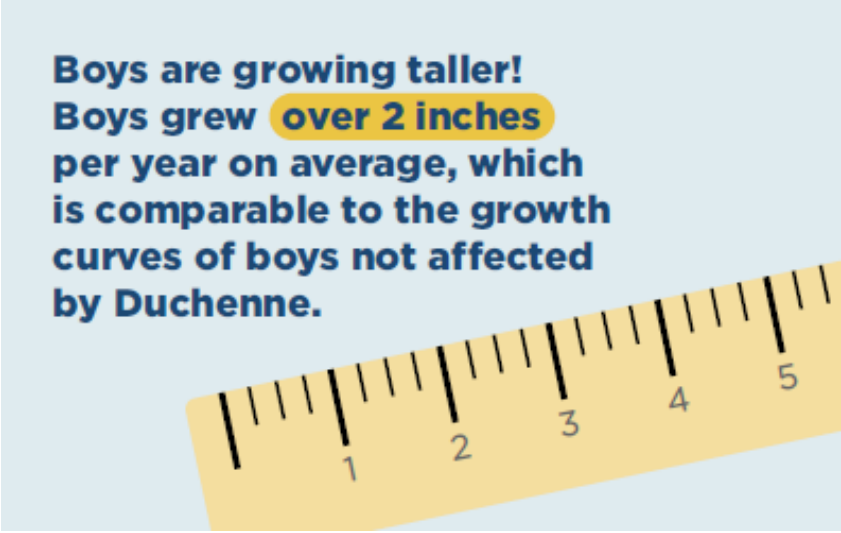


Baseline
99 beats/min

Safety:
Edasalonexent Has Been Well Tolerated To Date

- **50+ years of patient exposure**
  - Majority of adverse events observed were mild in nature

- **Boys on edasalonexent in our Phase 2 clinical trial and open-label extension grew similarly to unaffected boys**
  - Height increased by an average of 2.1 inches/year
  - Weight increased by an average of 2.9 pounds/year
  - Both increases in line with typical height and weight increases of unaffected boys

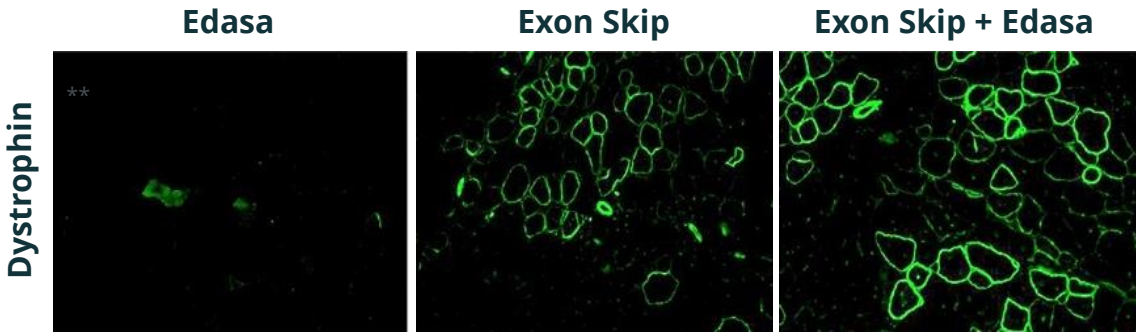


We Plan to Investigate the Potential for Combination Treatment in DMD

Edasalonexent increased dystrophin expression in combination with exon-skipping in *mdx* mice

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

- In eligible boys in the MoveDMD open-label extension, combination treatment of edasalonexent and EXONDYS 51® (exon skipping) was well tolerated.
- Boys on EXONDYS 51 are eligible for Phase 3 PolarisDMD and GalaxyDMD trials



Our Mission Is to Bring Hope and Life-Changing Therapies to Patients and Their Families

Catabasis Team

- Our goal is for edasalonexent to become a new oral foundational therapy to slow disease progression for those affected by Duchenne at all ages, regardless of mutation type, as a single agent and in combination with other therapies.

Learn More and Contact Us with Any Questions

- **Email** our clinical team at **DMDtrials@catabasis.com**

- **Follow us** on social media for frequent updates **@CatabasisPharma**

- **Learn more** about PolarisDMD on our website at **www.catabasis.com** and **clinicaltrials.gov** NCT03703882

- **Sign up** to receive our Newsletter and information updates on our website

Cure Duchenne™ And **thank you** CureDuchenne!