



Edasalonexent (CAT-1004) Program

**Oral NF- κ B inhibitor in development for the treatment of
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 single global Phase 3 PolarisDMD trial in Duchenne muscular dystrophy, or DMD, to evaluate the efficacy and safety of edasalonexent for registration purposes, our plans to continue to evaluate data from the open-label extension of our MoveDMD® clinical trial and from our GalaxyDMD open-label extension 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 Quarterly Report on Form 10-Q for the period ended June 30, 2019, 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

- ▶ 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 the 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



Edasalonexent: Potential for Broad Therapeutic Benefit

Activated NF- κ B leads to disease progression in DMD

Skeletal Muscle

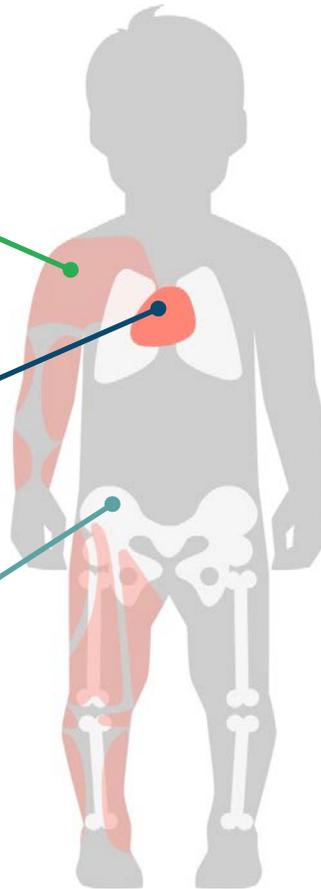
Loss of ambulation, upper limb function, respiratory failure

Heart

Cardiomyopathy

Bone

Fractures



Potential for Edasalonexent, an NF- κ B inhibitor



Goal: Improve skeletal muscle function



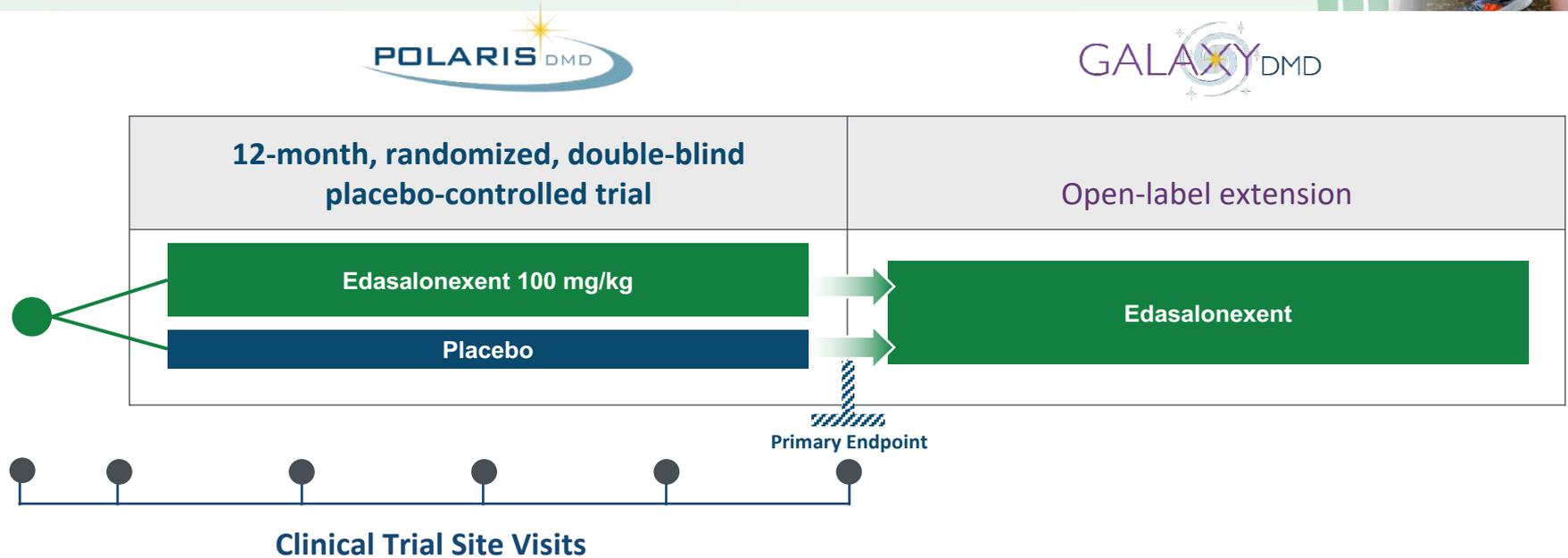
Goal: Preserve cardiac function



Goal: Reduce risk of fractures

In DMD, the loss of dystrophin leads to chronic activation of NF- κ B, which is a key driver of skeletal and cardiac disease progression

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 may receive drug after 12 months through GalaxyDMD
- ▶ Clinical trial site visits and key assessments every 3 months
- ▶ Safety measures including labs every 3 months
- ▶ Trial overseen by Data Safety Monitoring Board
- ▶ Top-line results expected in the second half of 2020

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 a Gel Capsule Taken By Mouth With Food

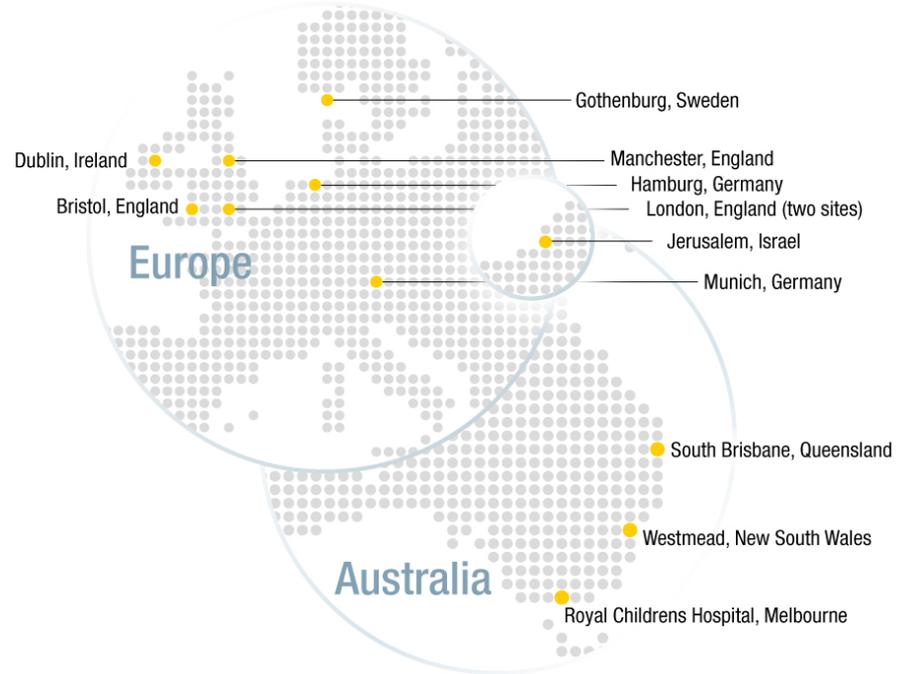
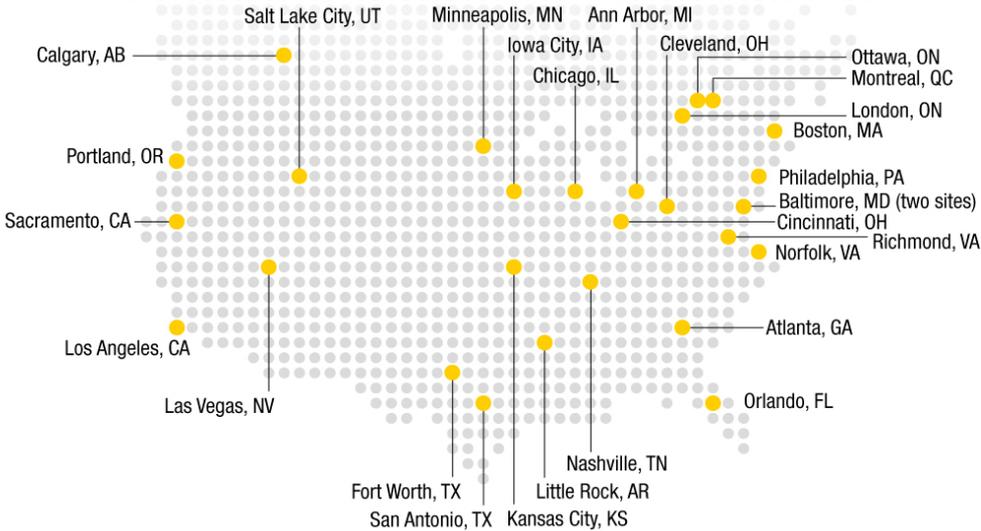
- ▶ **Dose 100 mg/kg/day**
- ▶ **Taken 3 times per day with food**
 - Mid-day dose can be at school or at home after school
- ▶ **2 different small capsule sizes**
 - 100 mg capsules are similar to the size of a tic-tac
 - 250 mg capsules are similar to the size of a jelly bean
- ▶ **Medi-straws provided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



Phase 3 PolarisDMD Clinical Trial Enrolling Globally



North America



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

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



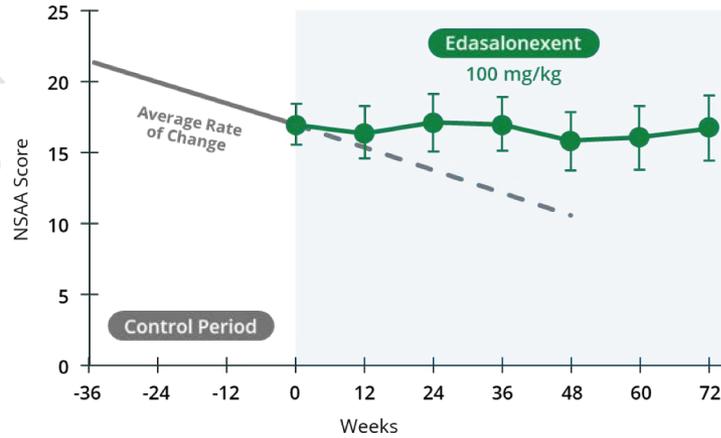
- ✓ Preservation of function as assessed by North Star Ambulatory Assessment and Timed Function Tests compared with rate of change during off-treatment control period

In the Phase 2 MoveDMD Trial and Open-Label Extension:

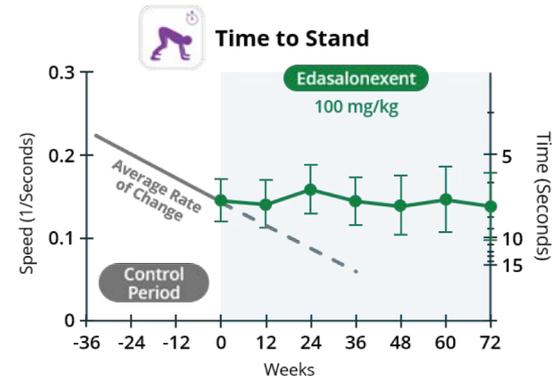
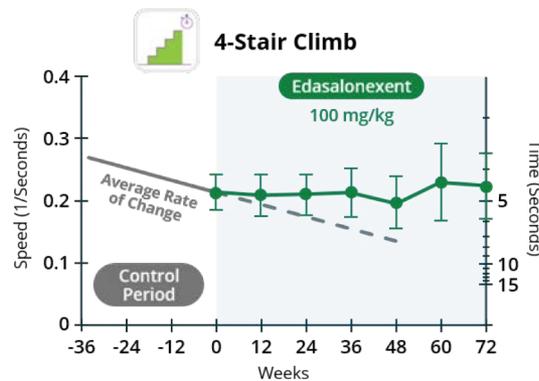
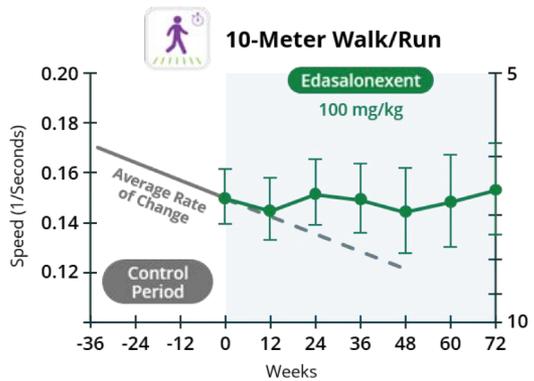
Edasalonexent Preserved Muscle Function Compared to Off-Treatment Control Period



Edasalonexent Treatment Stabilized North Star Ambulatory Assessment Score



Edasalonexent Treatment Stabilized Timed Function Tests



Means ± SEM shown. Includes data of all boys initially started on 100 mg/kg dose (n=16) with 11 boys participating through 72 weeks.

Safety: Edasalonexent Has Been Well-Tolerated to Date, Without Known Side Effects of Steroids



- ▶ 50+ years of patient exposure
 - Majority of adverse events mild in nature
 - Most common treatment-related AE is diarrhea, generally mild and transient
- ▶ Boys on edasalonexent in our Phase 2 MoveDMD and open-label extension trial grew similarly to unaffected boys
 - Height increased by an average of 2.1 inches/year, while weight increased by an average of 2.9 lbs/year, both in line with typical height and weight increases of unaffected boys

Boys are growing taller!
Boys grew **over 2 inches**
per year on average, which
is comparable to the growth
curves of boys not affected
by Duchenne.



Catabasis' Focus on Edasalonexent for Duchenne

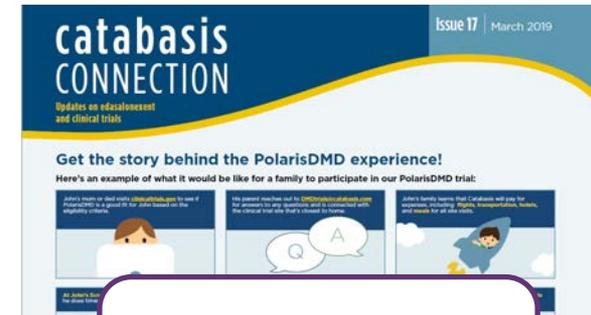


Our goal is for edasalonexent to become a new oral foundational therapy to slow disease progression for all affected by Duchenne as a single agent and potential to be co-administered with other therapies

Catabasis is working to design future clinical trials to expand to other age groups, including those who are non-ambulatory, and Becker muscular dystrophy

Thank You!

- ▶ Patients and families
- ▶ Patient groups
- ▶ ImagingDMD Investigators and Staff
- ▶ For questions regarding the Phase 3 clinical trial:
 - Email Joanne Donovan, M.D., Ph.D. and the Clinical Team: DMDtrials@catabasis.com
- ▶ For frequent updates on edasalonexent and PolarisDMD
 - Follow @CatabasisPharma on  book  er and  agram



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