



# **Edasalonexent (CAT-1004) Program**

**Oral small molecule designed to inhibit NF- $\kappa$ B 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 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.

# 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.
- ▶ **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



# Edasalonexent: Potential for Broad Therapeutic Benefit

Activated NF- $\kappa$ B leads to disease progression in DMD

## Skeletal Muscle

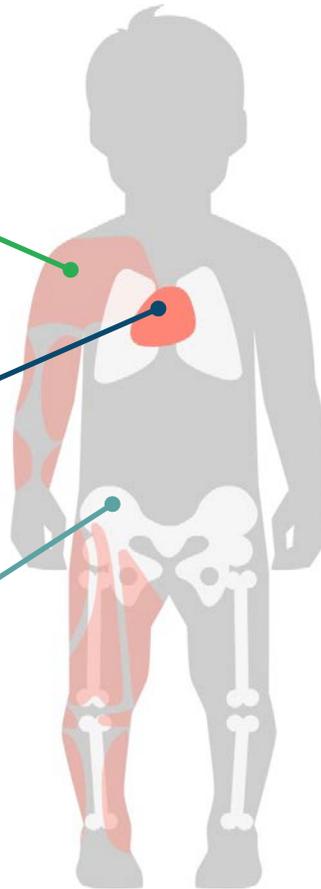
Loss of ambulation, upper limb function, respiratory failure

## Heart

Cardiomyopathy

## Bone

Fractures



Vision for Edasalonexent, and NF- $\kappa$ B inhibitor



**Goal: Improve** skeletal muscle function



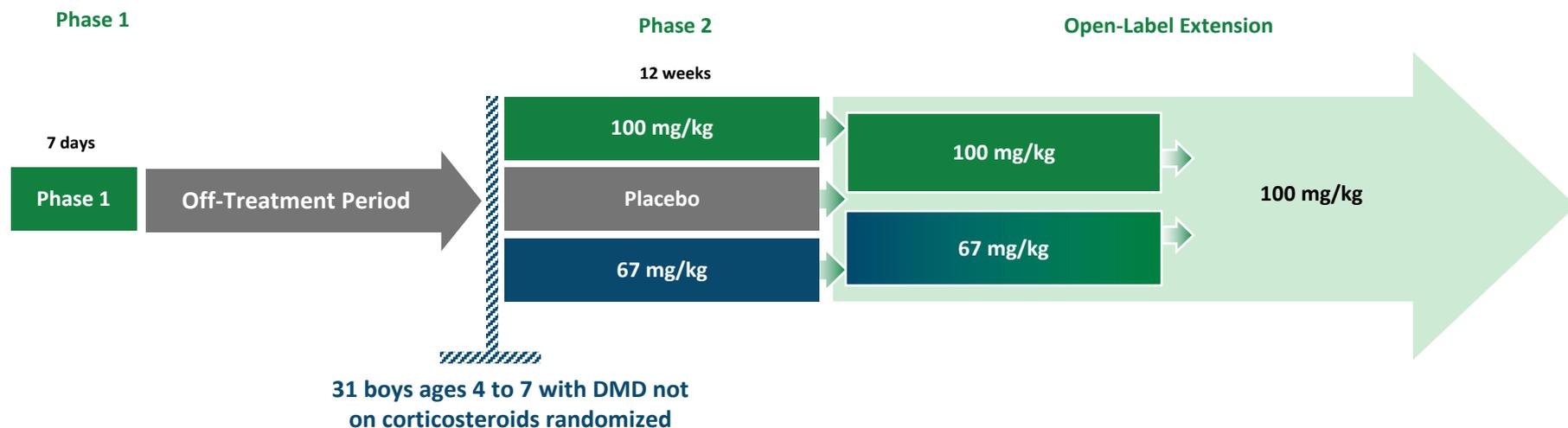
**Goal: Preserve** cardiac function



**Goal: Reduce** risk of fractures

**NF- $\kappa$ B is a key link between loss of dystrophin and disease pathology; plays fundamental role in the initiation and progression of skeletal muscle, respiratory and cardiac disease in DMD**

# MoveDMD Trial Designed to Inform Phase 3



- ▶ **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
  - 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

## 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

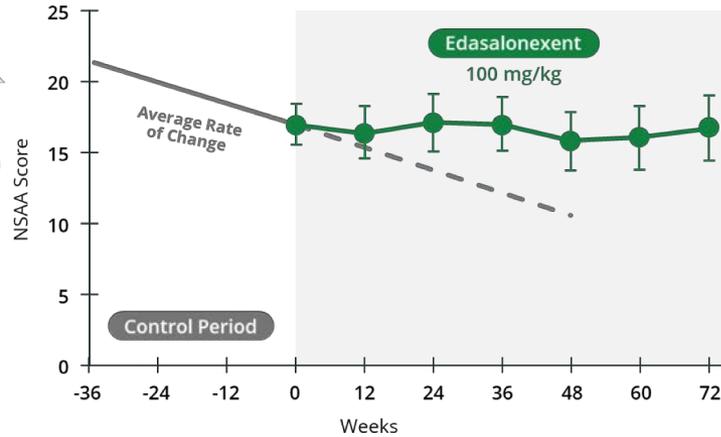


- ✓ Preservation of function as assessed by North Star Ambulatory Assessment and Timed Function Tests compared with rate of change during off-treatment control period

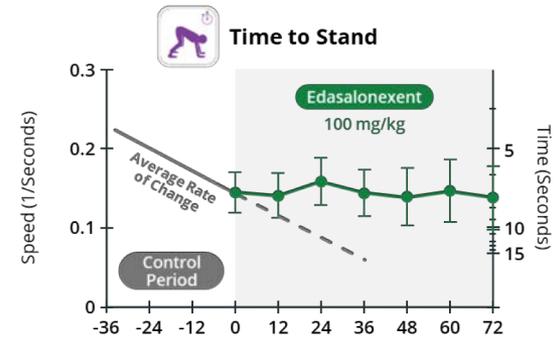
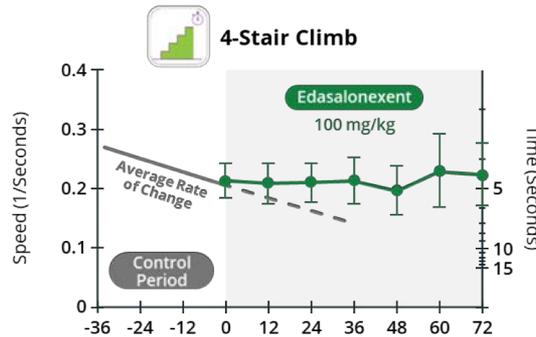
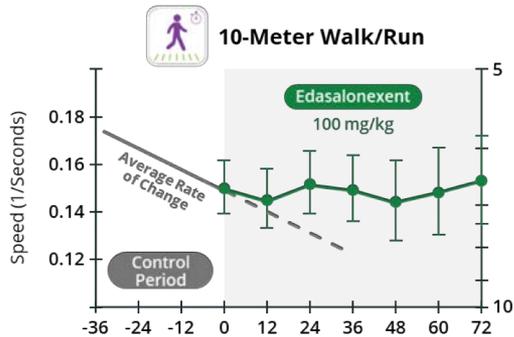
# In the Phase 2 MoveDMD Trial and Open-Label Extension, Edasalonexent Preserved Muscle Function Compared to Off-Treatment Period



Edasalonexent Treatment Stabilized North Star Ambulatory Assessment Score



## Edasalonexent Treatment Stabilized Timed Function Tests



## Safety: Edasalonexent Has Been Well-Tolerated to Date

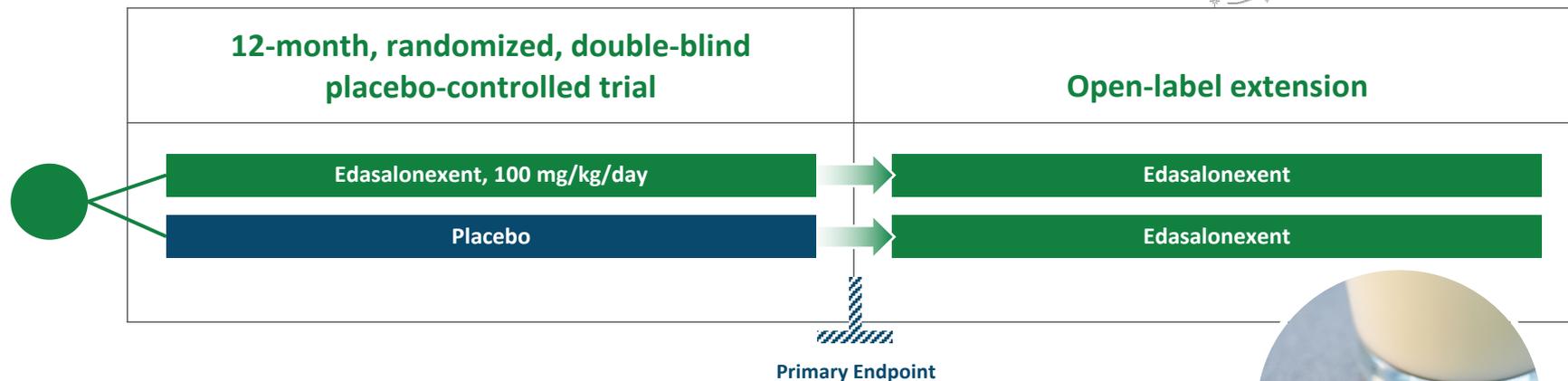
- ▶ 50+ years of patient exposure
  - Majority of adverse events mild in nature
- ▶ Boys on edasalonexent in our Phase 2 MoveDMD and open-label extension trial grew similarly to unaffected boys
  - Height increased by an average of 2.1 inches/year, while weight increased by an average of 2.9 lbs/year, 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.**



# Positive MoveDMD Data Support Phase 3 Registration Trial for Edasalonexent



## ▶ Key enrollment criteria

- Age 4 to 7 (up to 8<sup>th</sup> 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

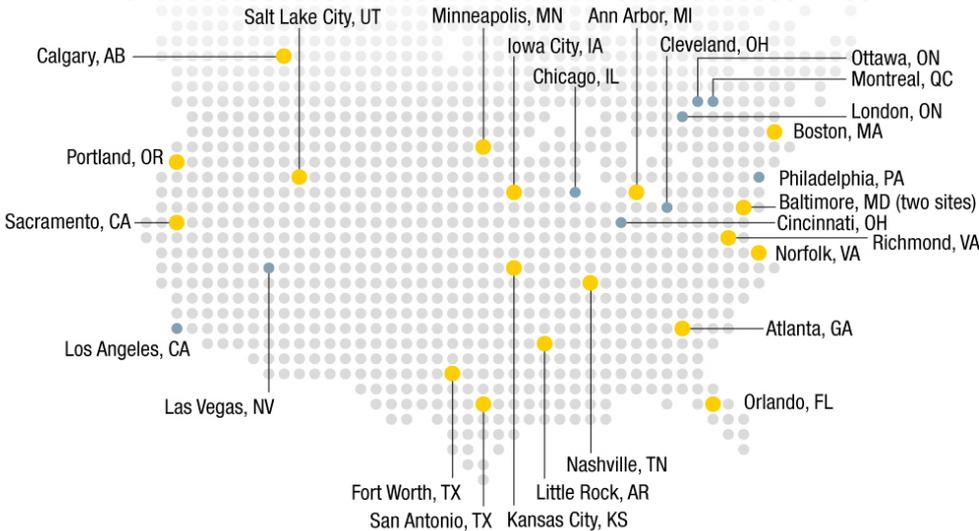
- North Star Ambulatory Assessment, Timed Function Tests, Muscle Strength
- Safety measures
- Assessments of growth, cardiac and bone health
- No biopsy or 6 minute walk test



# Many Clinical Trial Sites to Improve Patient Access, with 22 Open for Enrollment



## North America



● Expected Site    ● Active Site    ● Recently Activated Site



Nearly 40 sites expected globally, many sites active and enrolling patients

# 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](mailto:DMDtrials@catabasis.com)
- ▶ For frequent updates on edasalonexent and PolarisDMD
  - Follow @CatabasisPharma on  book  er and  agram



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