Edasalonexent (CAT-1004) Program

Oral small molecule designed to inhibit NF-κB 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 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.

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 is to become an oral new foundational therapy to slow disease progression for all people affected by Duchenne at all ages as a single agent an in combination with other therapies
- **PolarisDMD, a Phase 3 clinical trial of edasalonexent, is enrolling boys with Duchenne in the U.S.**

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 foundational therapy
- Edasalonexent substantially slowed disease progression compared to control
- Potential treatment alone and also exploring potential to combine with dystrophin-targeted therapies

POLARIS DMD

Edasalonexent: Potential for Broad Therapeutic Benefit

NF-kB in DMD

Skeletal Muscle

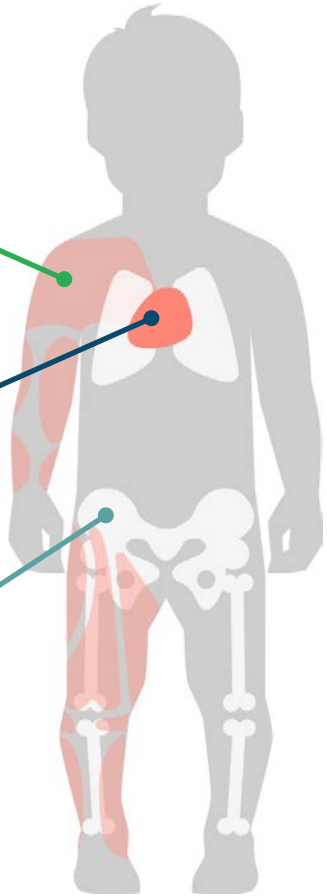
Loss of ambulation, upper limb function, respiratory failure

Heart

Cardiomyopathy

Bone

Fractures



Target Product Profile for Edasalonexent

Improve
skeletal muscle function

Improve
cardiac function

Reduce
risk of fractures

NF-kB 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

Promising Clinical Results Seen to Date with Edasalonexent

NF-κB Target Engagement

- ✓ Inhibition of 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

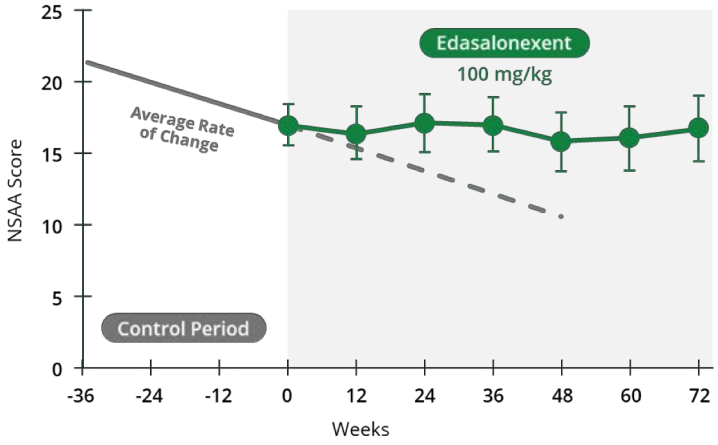
Functional Improvements

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

Edasalonexent Preserved Muscle Function Compared to Off-Treatment Period

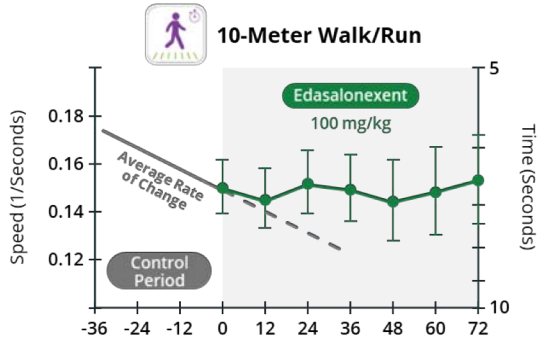
Edasalonexent Treatment Stabilized North Star Ambulatory Assessment Score

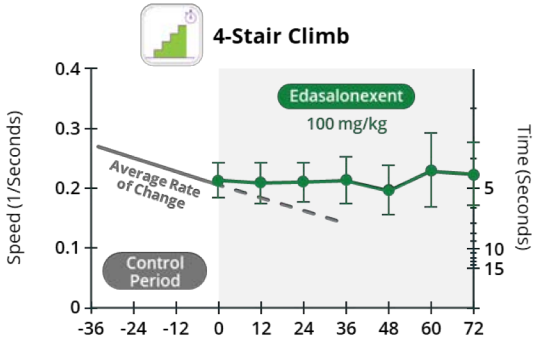
Better

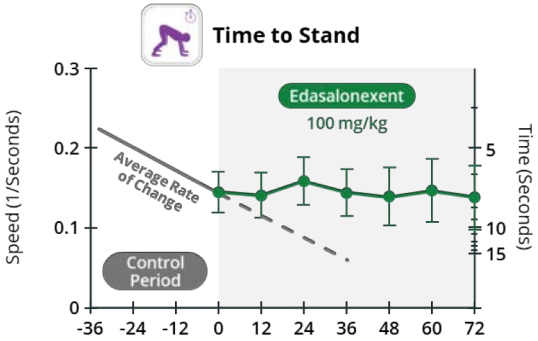


Edasalonexent Treatment Stabilized Timed Function Tests

Better





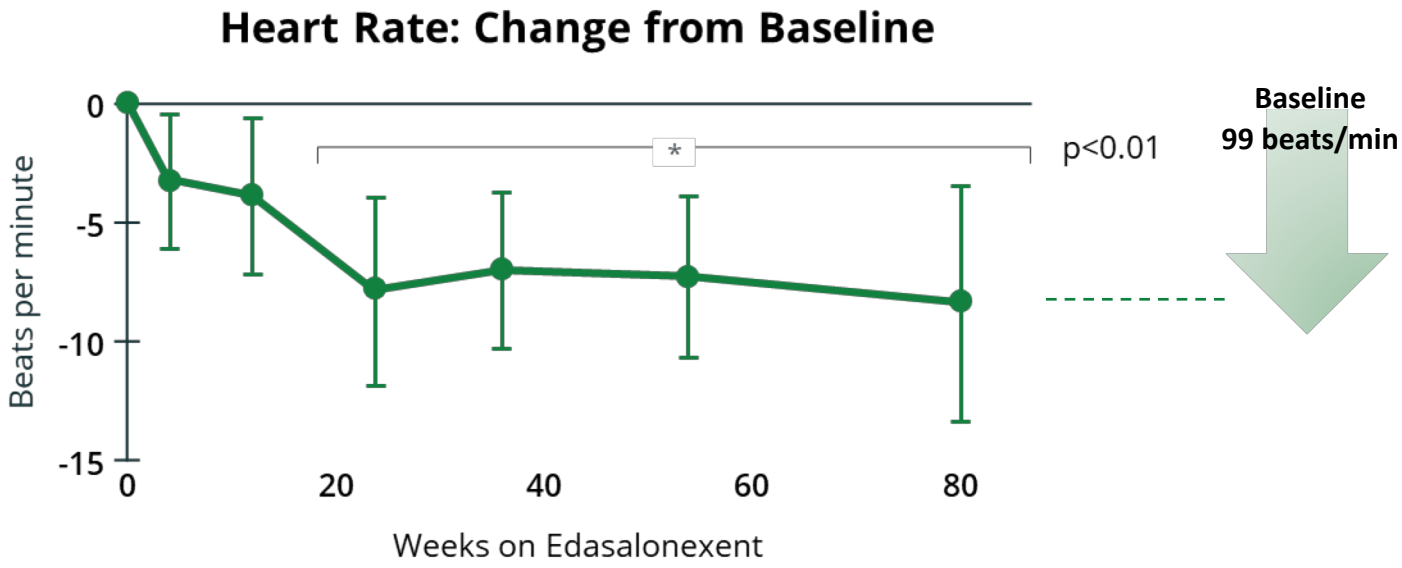


catabasis

Means ± SEM shown

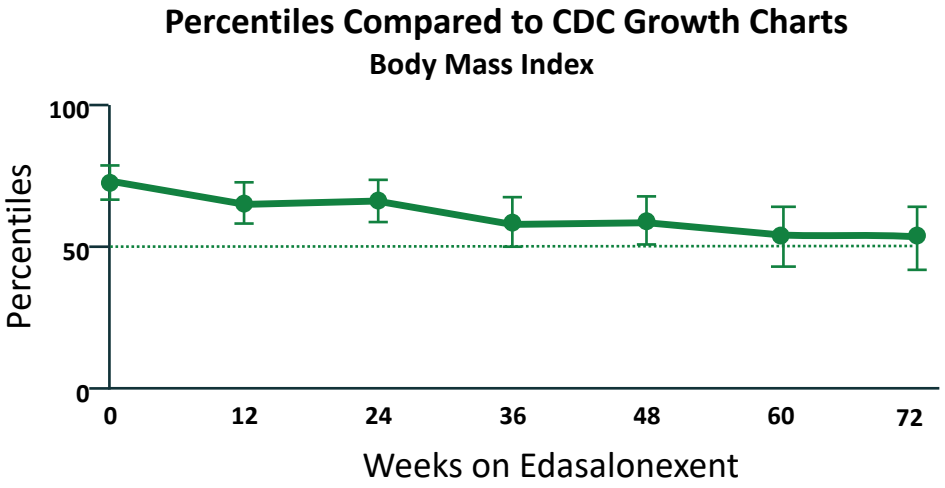
Edasalonexent Significantly Improved Biomarkers

- 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

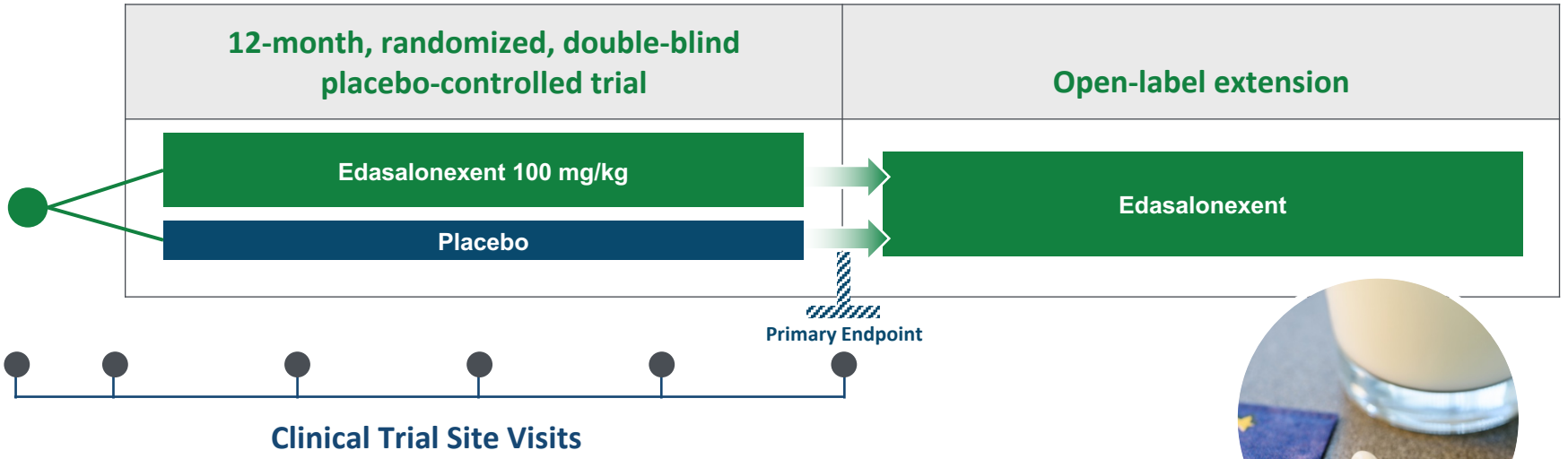


Edasalonexent Is Well Tolerated, with No Safety Signals or Steroid-Associated Side Effects

- No safety signals in 50+ years of patient exposure
- Well tolerated, with majority of adverse events mild in nature
- Boys on edasalonexent grow similar to unaffected boys
  - Favorably differentiated from weight gain and curtailed growth seen with corticosteroid standard of care



Design of Phase 3 PolarisDMD Trial

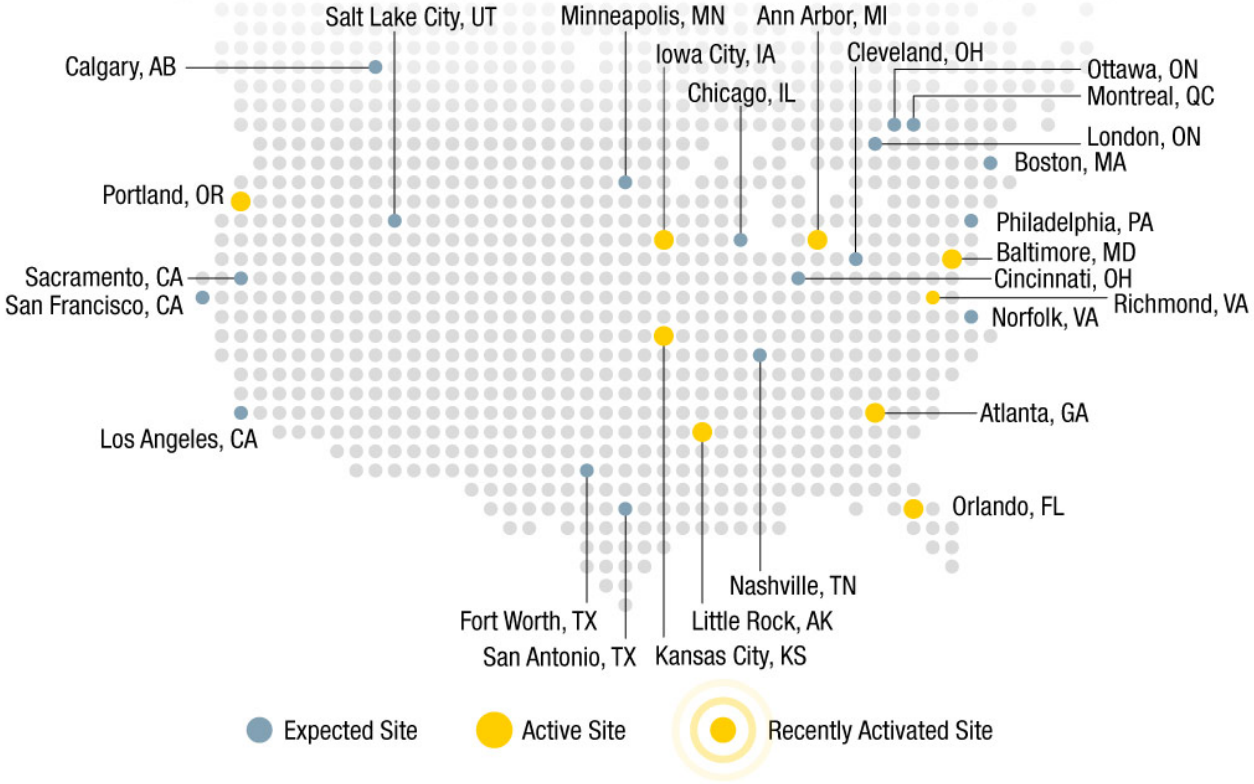


- **Key enrollment criteria**
  - Age 4 to 7 (up to 8th birthday)
  - Able to complete timed function tests
  - Not on corticosteroids for at least 6 months
  - Not on other investigational therapies for at least 1 month, can be on stable eteplirsen
- **Visits / key assessments every 3 months**
  - North Star Ambulatory Assessment, Timed Function Tests, Muscle Strength
  - Safety measures
  - Assessments of growth, cardiac and bone health
  - No biopsy or 6 minute walk test

POLARIS DMD

Many Clinical Trial Sites to Improve Patient Access

- **Many sites active and enrolling patients**
- Additional sites will be active in the coming weeks
- Sites also expected in Europe, Australia and Israel



POLARIS DMD

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

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** on our website at **www.catabasis.com** and **clinicaltrials.gov** NCT03703882

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

Parent Project Muscular Dystrophy

And **thank you** PPMD!