



Edasalonexent (CAT-1004) Program

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

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Officer

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Chief Medical

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.

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

Catabasis Team



- ▶ Our focus is edasalonexent and muscular dystrophy
- ▶ Our goal is for edasalonexent to preserve muscle function for everyone affected by Duchenne

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

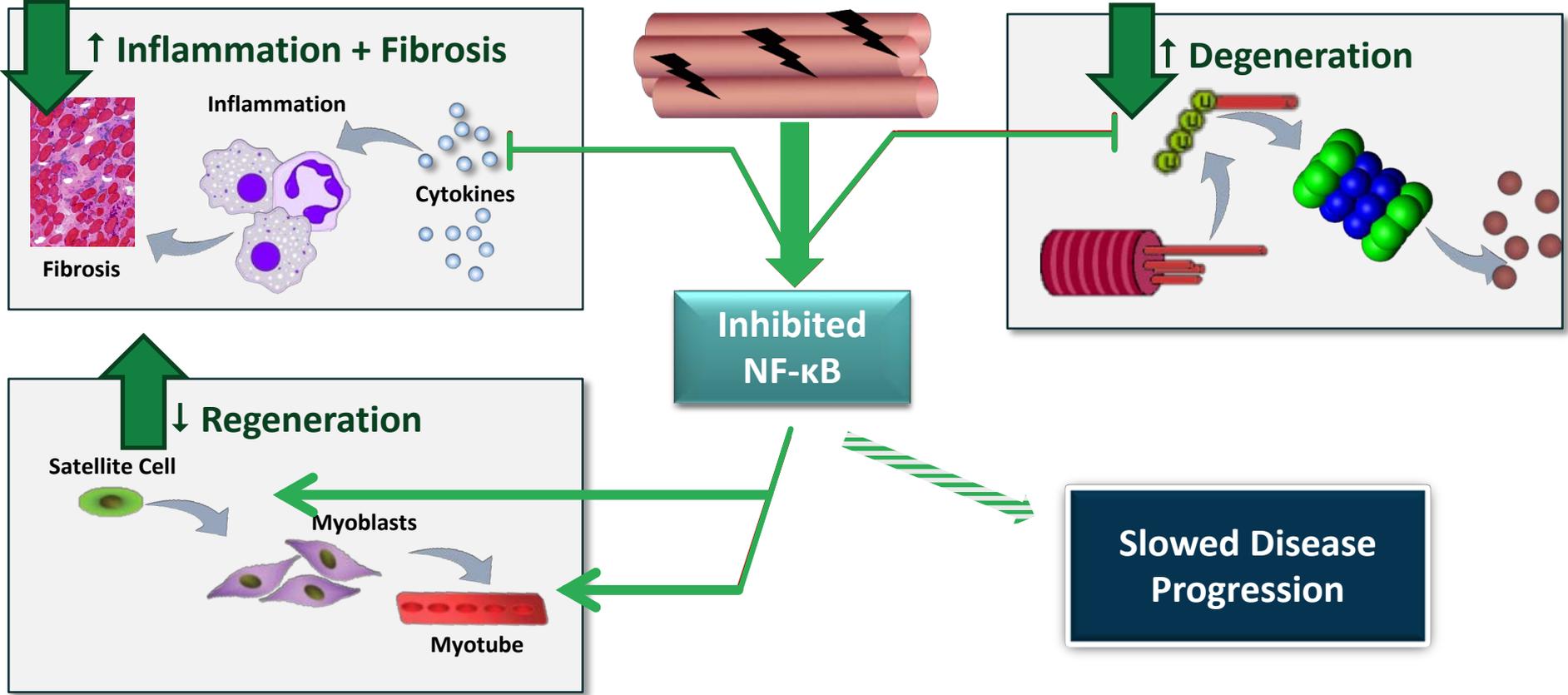
- ▶ Investigational oral disease-modifying agent for all patients with Duchenne, regardless of mutation type: potential for new standard of care
- ▶ Edasalonexent substantially slowed disease progression compared to control
- ▶ Potential treatment alone and also exploring potential to combine with dystrophin-targeted therapies



Edasalonexent Inhibits NF- κ B, a Key Driver of Disease in Duchenne



Edasalonexent



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



Activated NF-kB leads to disease progression in DMD

Skeletal Muscle

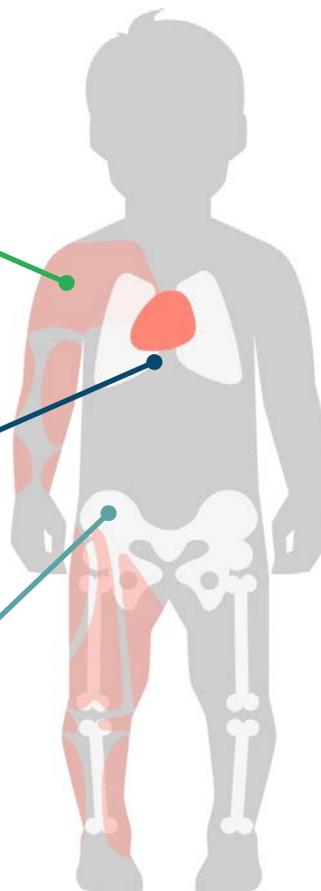
Loss of ambulation, upper limb function, respiratory failure

Heart

Cardiomyopathy

Bone

Fractures



Vision for edasalonexent, an NF-kB inhibitor



Improve skeletal muscle function



Improve cardiac function



Reduce risk of fractures

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

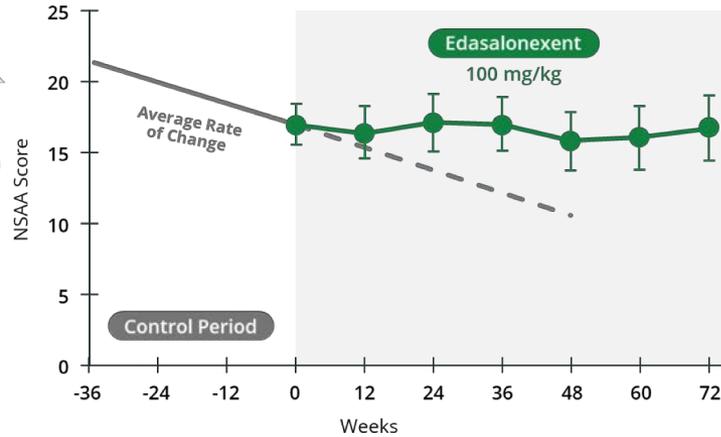


- ✓ Preservation of function as assessed by North Star Ambulatory Assessment and Timed Function Tests compared with rate of change during off-treatment control period

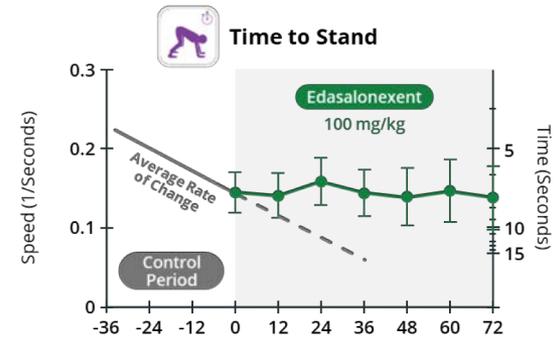
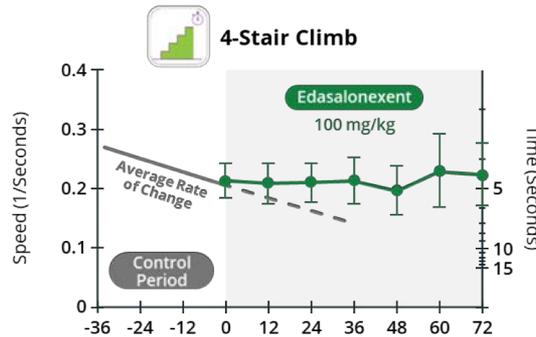
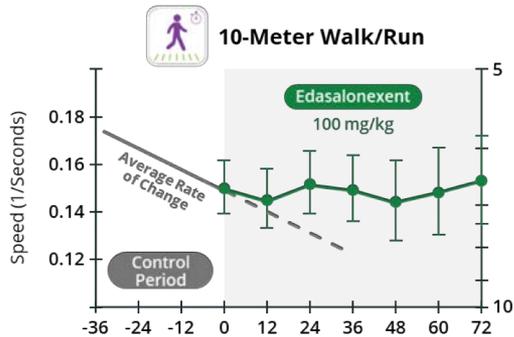
Edasalonexent Preserved Muscle Function Compared to Off-Treatment Period



Edasalonexent Treatment Stabilized North Star Ambulatory Assessment Score

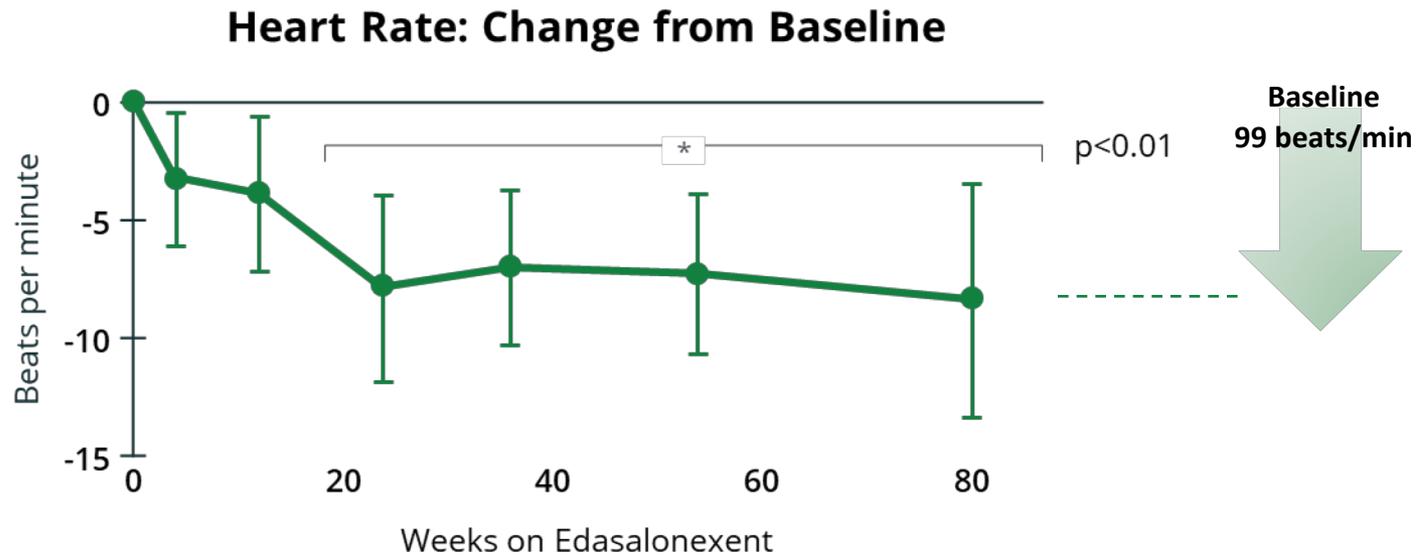


Edasalonexent Treatment Stabilized Timed Function Tests



Edasalonexent Significantly Improved Biomarkers

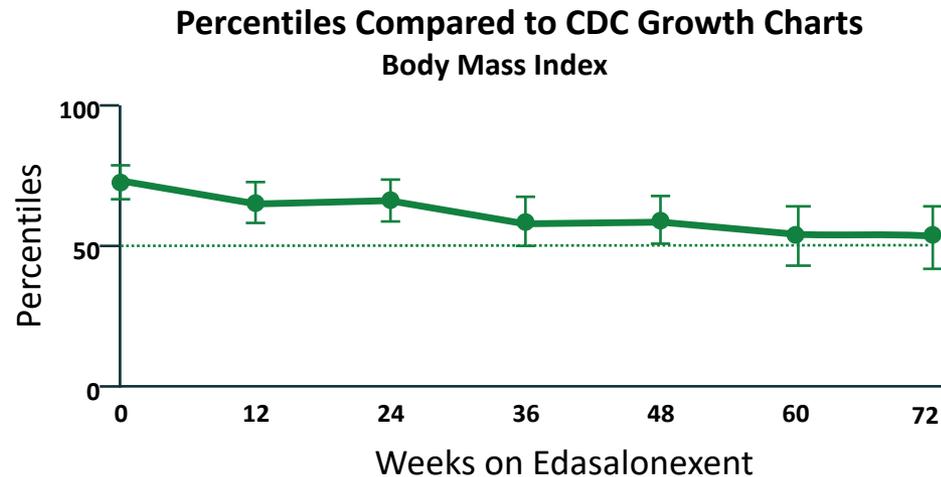
- ▶ Significantly improved CRP and all muscle enzymes, including CK
- ▶ Boys affected by Duchenne have elevated heart rates and edasalonexent treatment decreased heart rate towards age-normative values



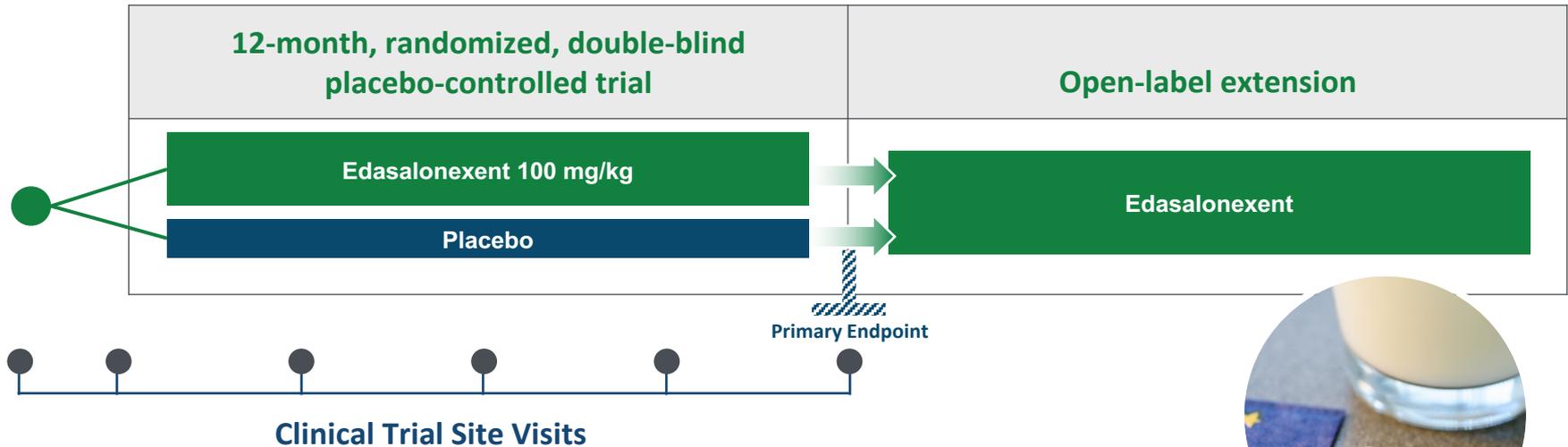
Edasalonexent Is Well Tolerated, with No Safety Signals or Steroid-Associated Side Effects



- ▶ 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

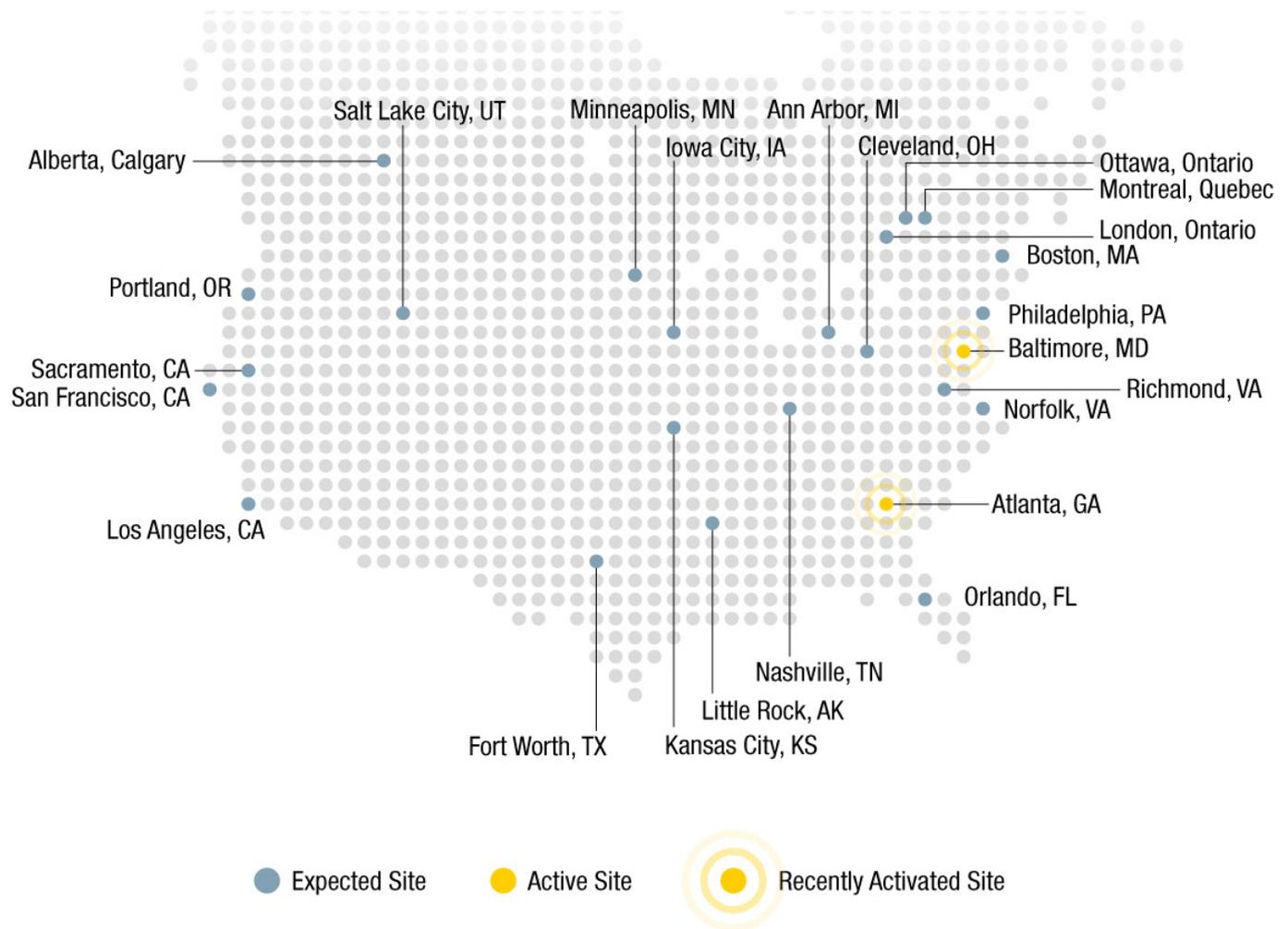
▶ Visits / key assessments every 3 months

- 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



Many Clinical Trial Sites in U.S. and Canada to Improve Patient Access

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



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



The screenshot shows the ClinicalTrials.gov listing for the PolarisDMD Clinical Trial. The title is "PolarisDMD CLINICAL TRIAL". Below the title, there is a brief description: "We are honored and proud to be members of the Duchenne muscular dystrophy (DMD) community in strength and determination. We strive to discover, develop and bring to patients there a meaningful difference in the lives of those who are affected by this disease." The page includes sections for "PolarisDMD Clinical Trial", "News & Updates", and "For Further Information". There is a "Study Description" section with a "Study Details" table. The table has columns for "Study Details", "Study Status", "Study Results", "Study Funding", and "Study Location". The table contains the following information:

Study Details	Study Status	Study Results	Study Funding	Study Location
PolarisDMD	Phase 3	Completed	Public	USA

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



The screenshot shows the Catabasis Quarterly newsletter. The header reads "catabasis QUARTERLY". The main content includes a section titled "The PolarisDMD trial is underway!" and another titled "PolarisDMD Clinical Trial". There are also social media icons for Facebook and Twitter.

**Parent
Project
Muscular
Dystrophy**

And **thank you** PPMD!