PolarisDMD: Phase 3 Trial of Edasalonexent, a Novel NF-kB Inhibitor for Duchenne

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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 June 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.

Today’s Webinar Will Address

- Our vision for and introduction to edasalonexent
- Edasalonexent clinical results
- Phase 3 PolarisDMD trial
- Goal for the future
- Q&A

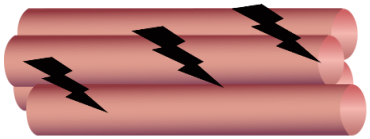
Our Vision for Edasalonexent Is to Improve Tomorrow for Those Affected by Duchenne

Our Goal: for Edasalonexent to Benefit All Boys and Men Affected with Duchenne at All Stages

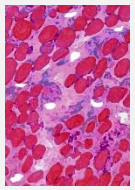
- Edasalonexent can be taken on its own and also may enhance the efficacy of dystrophin-targeted therapies such as exon skipping and gene therapy

We Are Taking a Different Approach to Treating Duchenne: Activated NF-κB Is a Key Factor in Disease Progression in DMD

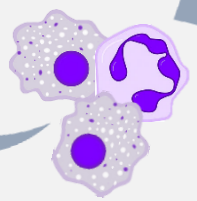
No Dystrophin
+
Mechanical Stress



Inflammation + Fibrosis



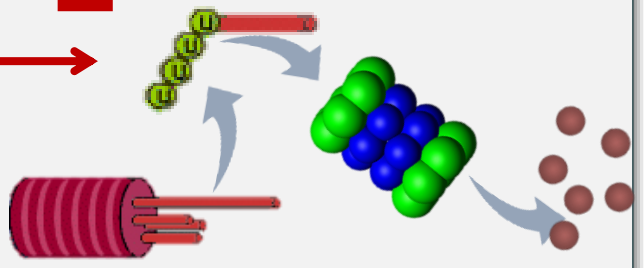
Inflammation



Cytokines

Fibrosis

Degeneration

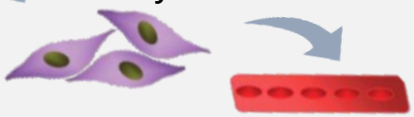


Activated NF-κB

Regeneration

Satellite Cell

Myoblasts



Myotube

Disease Progression

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

Edasalonexent

Inflammation + Fibrosis

Inflammation

Cytokines

Fibrosis

Degeneration

Inhibited NF-κB

Regeneration

Satellite Cell

Myoblasts

Myotube

Slowed Disease Progression

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

Activated NF-kB leads to disease progression in DMD

Skeletal Muscle

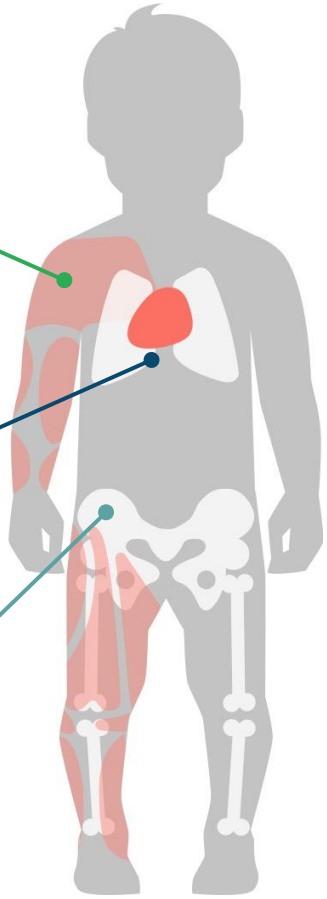
Loss of ambulation, upper limb function, respiratory failure

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

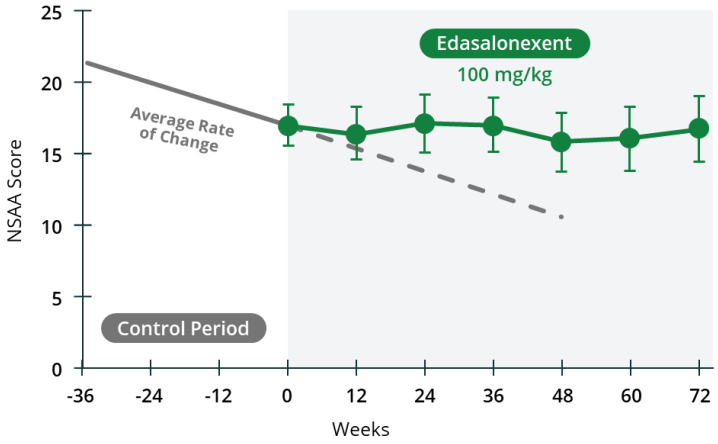
Functional Improvements

- ✓ 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

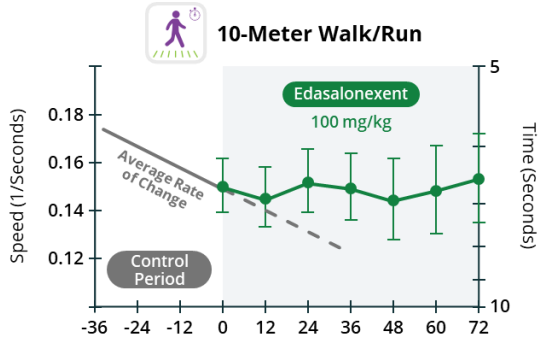
Edasalonexent Treatment Stabilized North Star Ambulatory Assessment Score

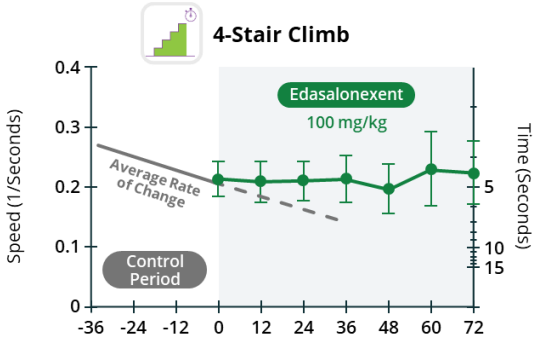
Better

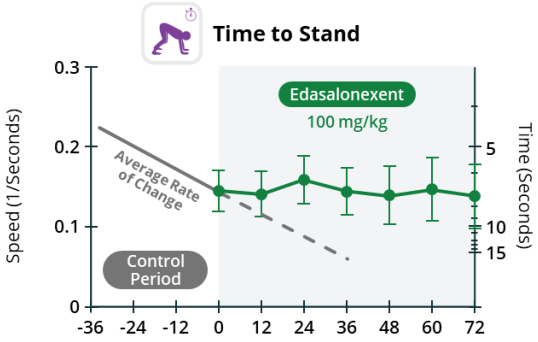


Edasalonexent Treatment Stabilized Timed Function Tests

Better





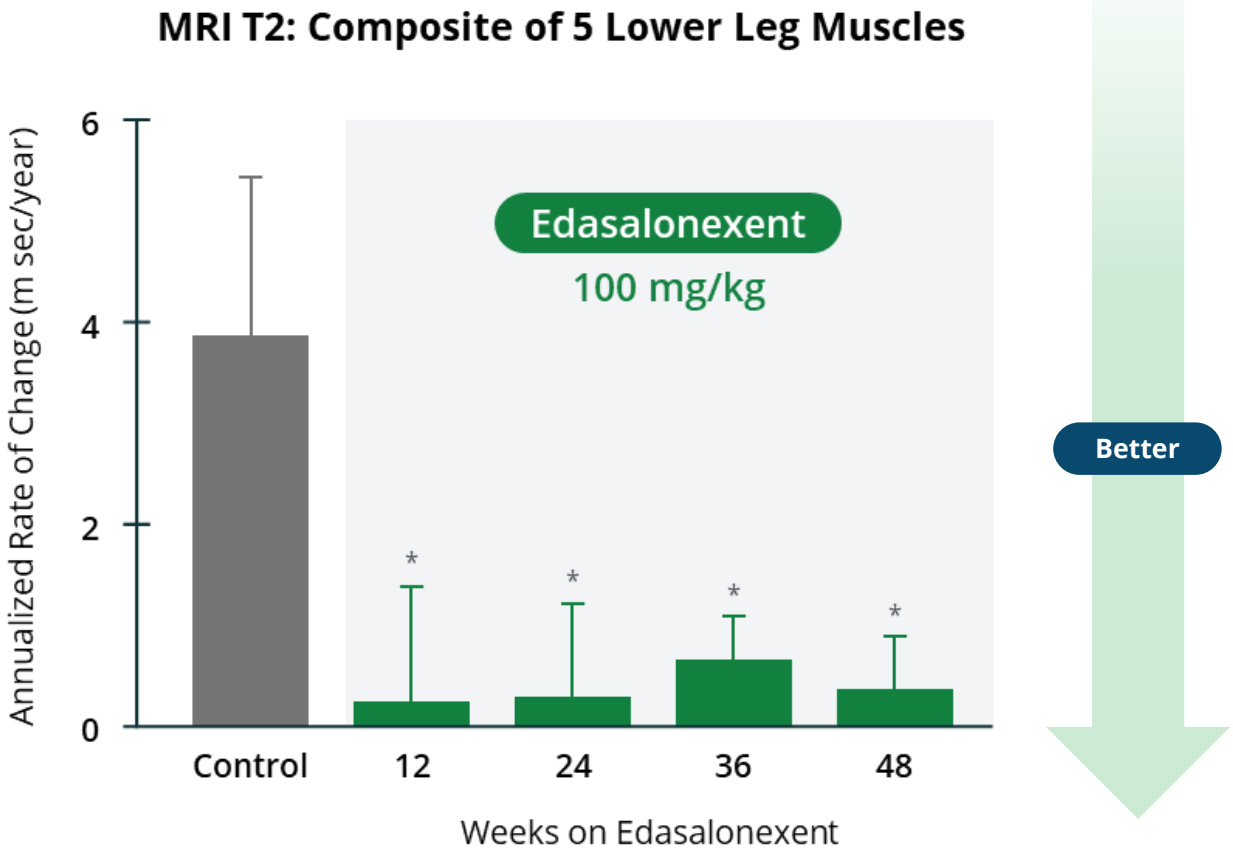


catabasis

Means ± SEM shown

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

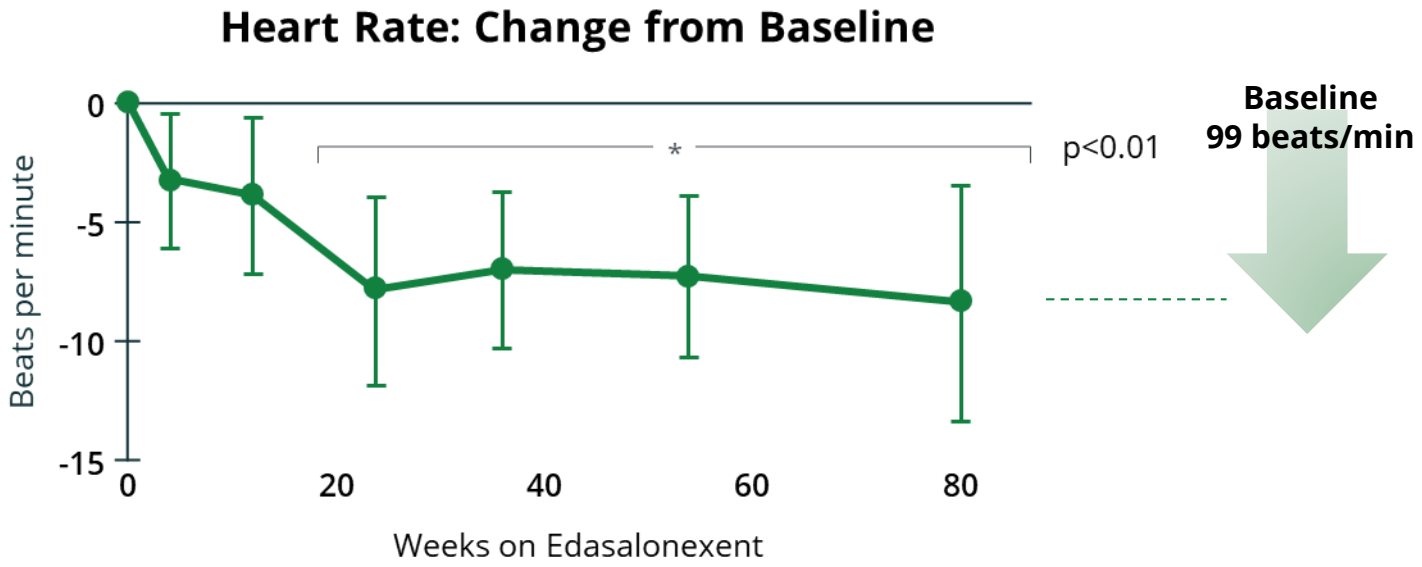
- MRI is a non-invasive approach to assess disease progression in DMD



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

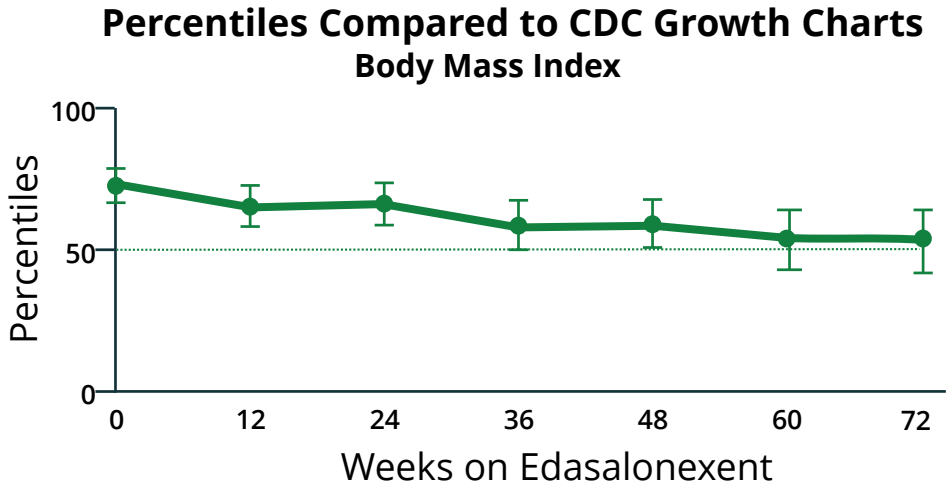
Edasalonexent Significantly Improved Biomarkers

- 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

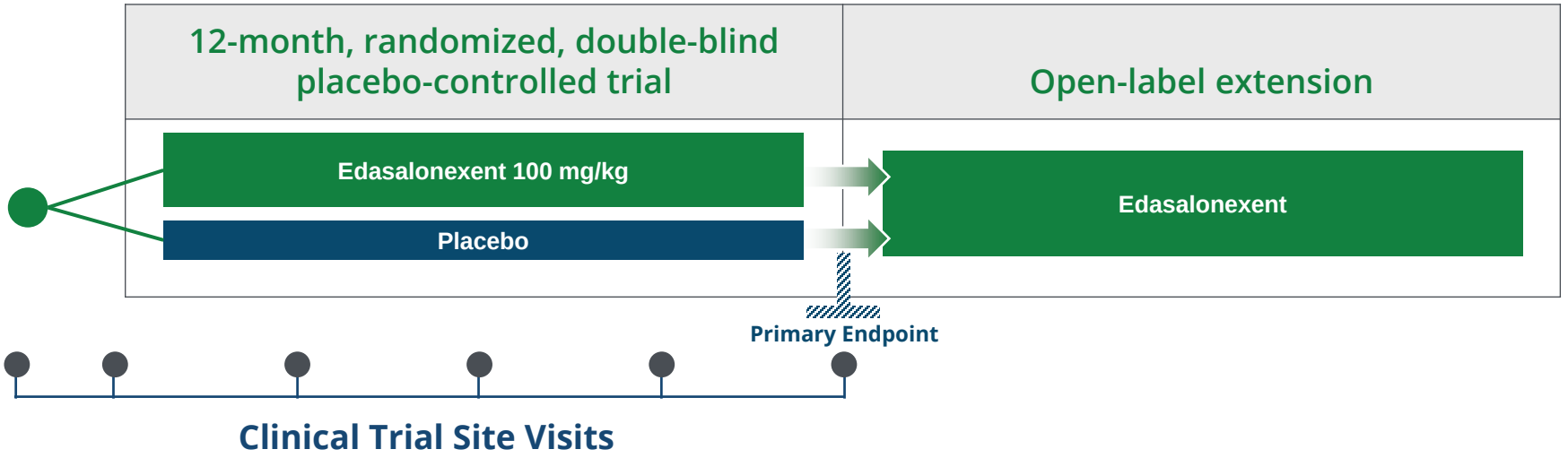


Edasalonexent Is Well Tolerated, with No Safety Signals or Steroid-Associated Side Effects

- 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



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

PolarisDMD Trial Patient Eligibility

INCLUSION CRITERIA
Written consent (and assent)
Diagnosis of Duchenne based on a clinical phenotype with increased serum CK and **documentation of mutation(s)** in dystrophin gene
Male sex by birth
Age **≥4.0 to <8.0** years (at time of consent)
Able to perform **stand from supine without assistance in ≤ 10 seconds**
Able to perform the 10MWT and 4-stair climb
Able to **swallow placebo capsules** at the Screening Visit
Followed by medical professional who coordinates Duchenne care

PolarisDMD Trial Patient Eligibility

EXCLUSION CRITERIA
Within 24 weeks prior to Day 1: corticosteroid use
Within ~4 weeks: other investigational drugs or ongoing participation in any other therapeutic clinical trial
Within 4 weeks prior to Day 1: immunosuppressive therapy, warfarin and other specific medications detailed on clinicaltrials.gov
Within 3 months prior to Day 1: human growth hormone
Within 12 weeks prior to Day 1: documented hepatitis B, hepatitis C, or HIV or a known risk factor for hepatitis such as a blood transfusion
Other prior or ongoing medical conditions

Exception: inhaled, intranasal, and topical corticosteroids permitted

Exception: eteplirsen (stable dose for at least 24 weeks prior to Day 1) permitted

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, which is known to be decreased in DMD

Bone

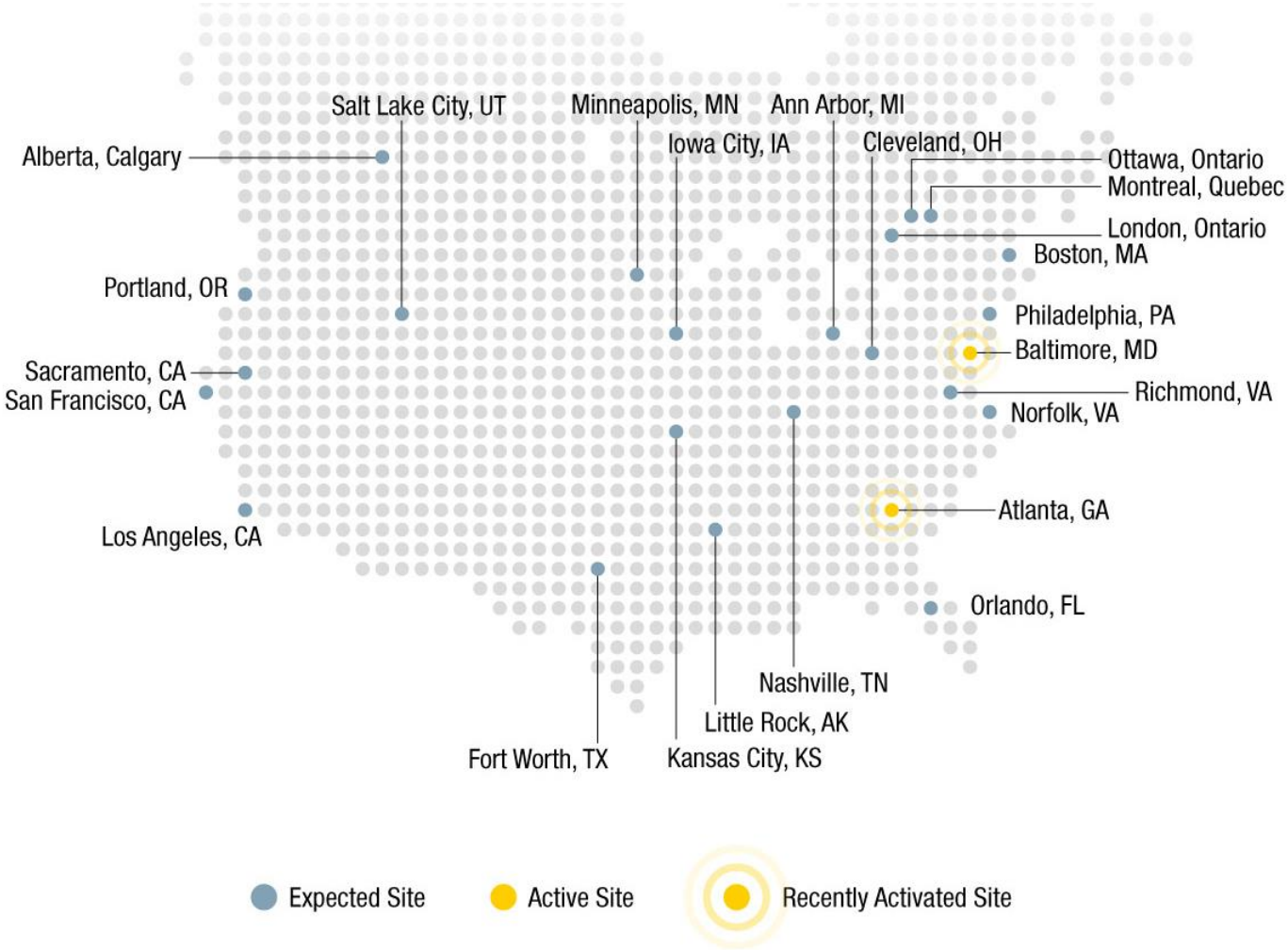
- Lateral thoracolumbar spine radiograph will be collected at baseline and after one year of treatment
- Bone mineral density by DXA will be collected at baseline and after one year of treatment

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 M&Ms
- **Medi-straws provided to facilitate capsule swallowing**

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



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

- **Reasons to consider this trial**
  - Edasalonexent positive clinical results to date
  - Edasalonexent has been safe and well tolerated in clinical trials
    - Not a steroid and no evidence of steroid-associated side effects
  - PolarisDMD trial designed to minimize impact on families
    - Clinical trial site visits every 3 months
    - Costs for travel to the trial sites provided
  - Oral drug

POLARIS DMD

Edasalonexent Is a 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

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

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!