

Edasalonexent (CAT-1004) Program

Oral NF- κ B inhibitor in development for the treatment of Duchenne muscular dystrophy

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November 16, 2019

Forward Looking Statements

This presentation contains, and any oral remarks made in connection with such presentation may contain, 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 September 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 in Duchenne Muscular Dystrophy Regardless of Mutation Type

- ▶ Edasalonexent is an NF- κ B inhibitor and is not a steroid
- ▶ 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 currently being studied in the ongoing Phase 3 PolarisDMD trial



Edasalonexent is an investigational agent not currently approved in any territory

Edasalonexent: Potential for Broad Therapeutic Benefit

Activated NF-κB leads to disease progression in DMD

Skeletal Muscle

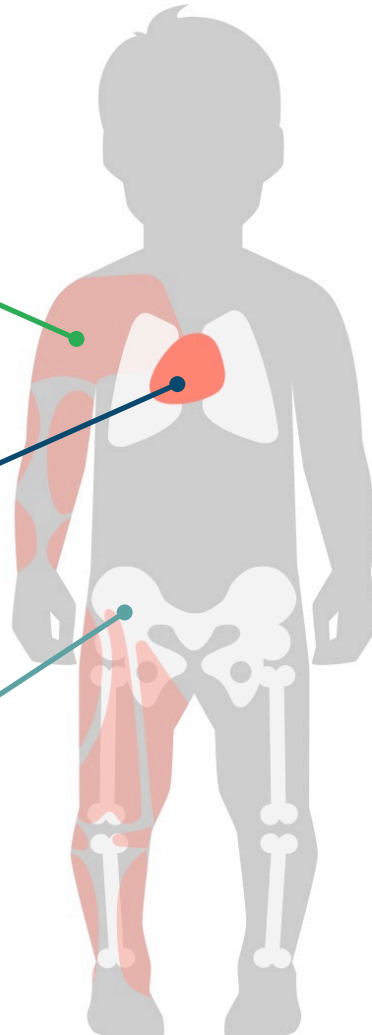
Loss of ambulation, upper limb function, respiratory failure

Heart

Cardiomyopathy

Bone

Fractures



Potential for edasalonexent, an NF-κB inhibitor



Goal: Improve skeletal muscle function



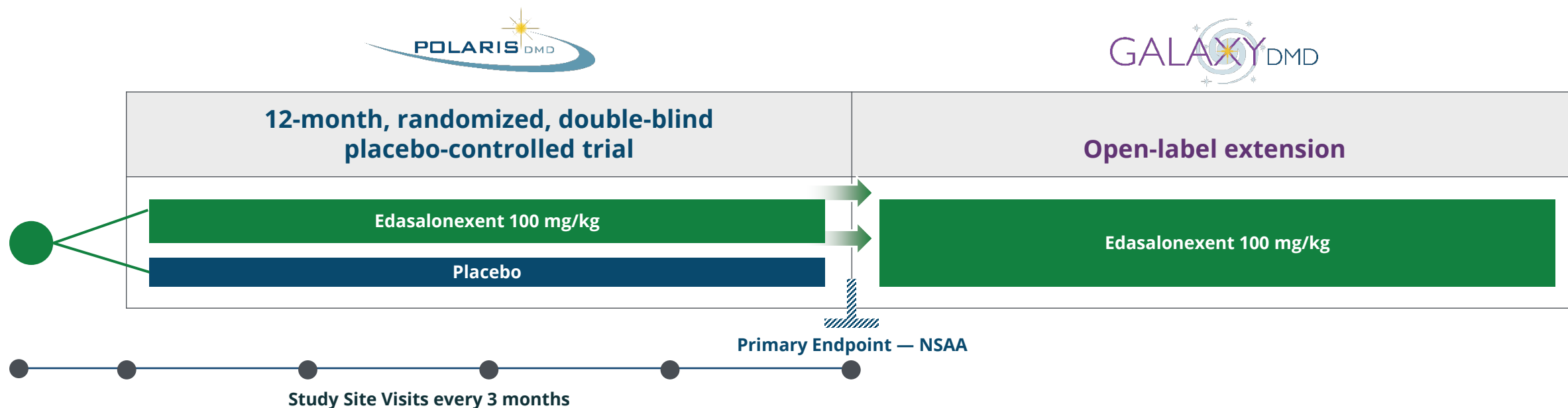
Goal: Preserve cardiac function



Goal: Reduce risk of fractures

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

Fully Enrolled Phase 3 PolarisDMD Trial Designed for Global Registration



- ▶ **Enrolled 131 boys ages 4 to 7 (up to 8th birthday) at 2:1 randomization**
 - Not on corticosteroids for at least 6 months
 - 67% of boys receive drug initially, all boys have the option to receive drug after 12 months through GalaxyDMD
- ▶ **Primary endpoint is the North Star Ambulatory Assessment, secondary endpoints are 3 timed function tests (10-meter walk/run, 4-stair climb, time to stand)**
- ▶ Site visits and key efficacy, safety, and laboratory assessments every 3 months
- ▶ **Top-line results expected in Q4 2020**

Additional Assessments Include Growth, Cardiac, Bone, and Quality of Life Measures



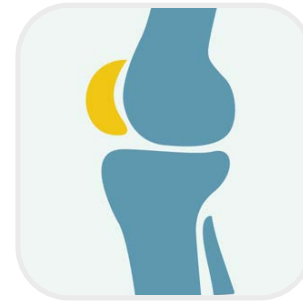
Growth

- ▶ Monitoring height and weight to assess how boys are growing relative to their expected growth curve



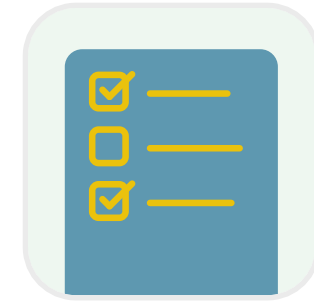
Heart

- ▶ Monitoring heart rate and heart rate variability with an easy to wear at-home small adhesive device at baseline, 6 and 12 months



Bone

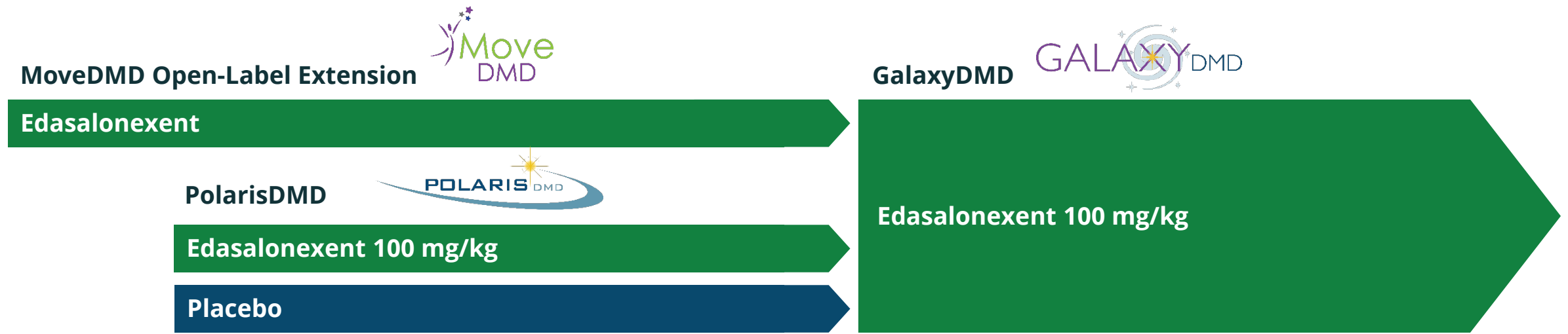
- ▶ X-rays of the spine at baseline and after one year of treatment
- ▶ Bone mineral density by DXA at baseline and after one year of treatment



Patient Reported Outcomes

- ▶ Quality of life questionnaire

GalaxyDMD Trial for Boys Receiving Open-Label Edasalonexent



- ▶ Open-label trial with primary focus to evaluate long-term safety of edasalonexent
- ▶ Site visits are every 6 months
- ▶ GalaxyDMD trial enrolled boys who had completed the MoveDMD trial and their eligible brothers
- ▶ After completing the PolarisDMD trial, boys and their eligible brothers also have the option to enroll in GalaxyDMD

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 CK and biomarkers of inflammation
- ✓ Decrease in heart rate to age-normative values

Muscle MRI Improvements



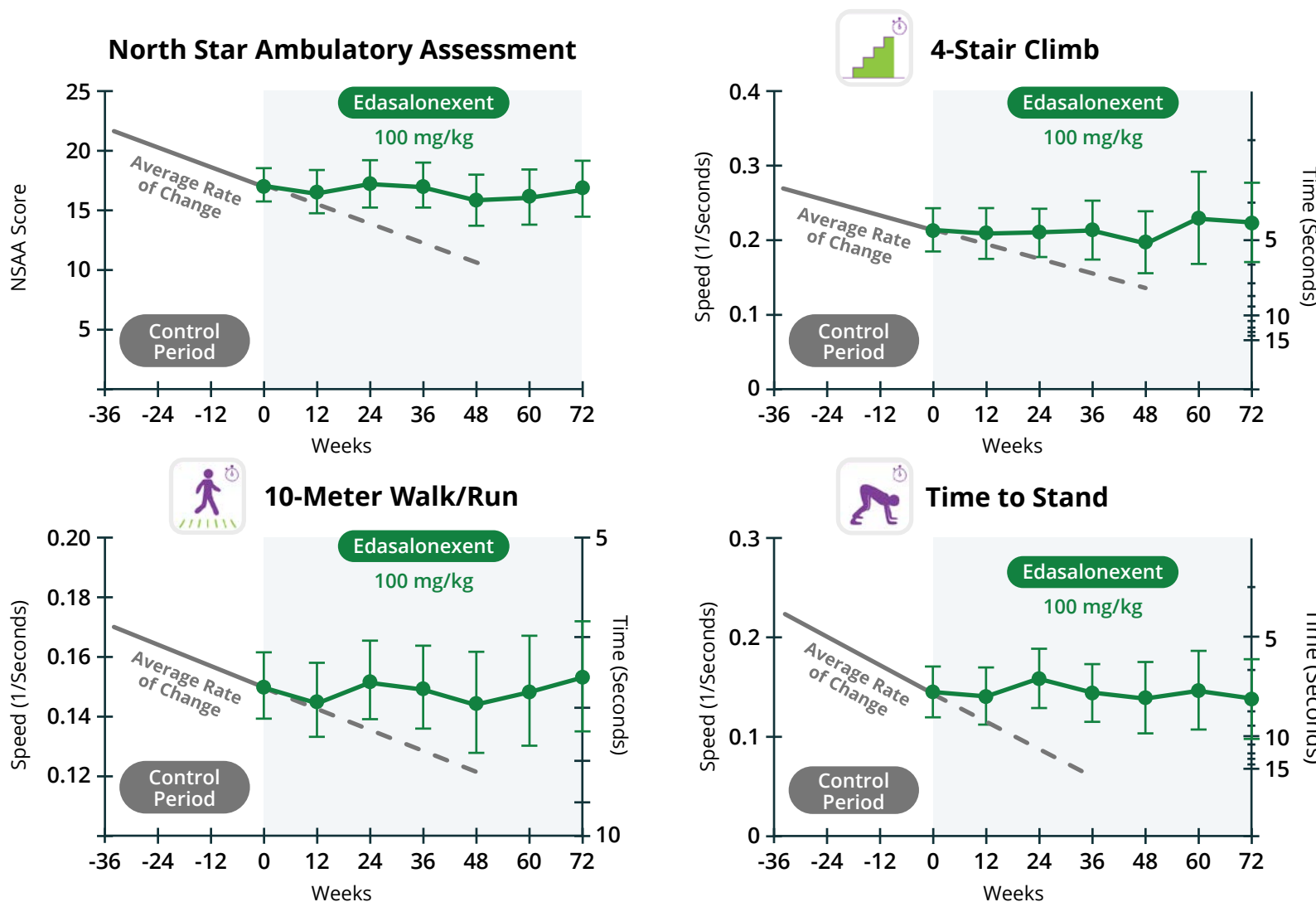
- ✓ Improvement in rate of change in MRI T2 compared with the rate of change during the off-treatment period

Functional Improvements



- ✓ Preservation of function compared with rate of change during off-treatment control period

Edasalonexent Preserved Muscle Function Compared to Off-Treatment Control Period



Growth Continued Similarly to Unaffected Boys

- ▶ Height increased by an average of 2.1 inches/year
- ▶ 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.



- **Over 60 years of cumulative patient exposure**
- **Majority of adverse events mild in nature**
 - Most common treatment-related AE is diarrhea, generally mild and transient
 - No serious adverse events on treatment (one on placebo)
 - No adverse trends in chemistry, hematology, or measures of adrenal function (cortisol and ACTH)



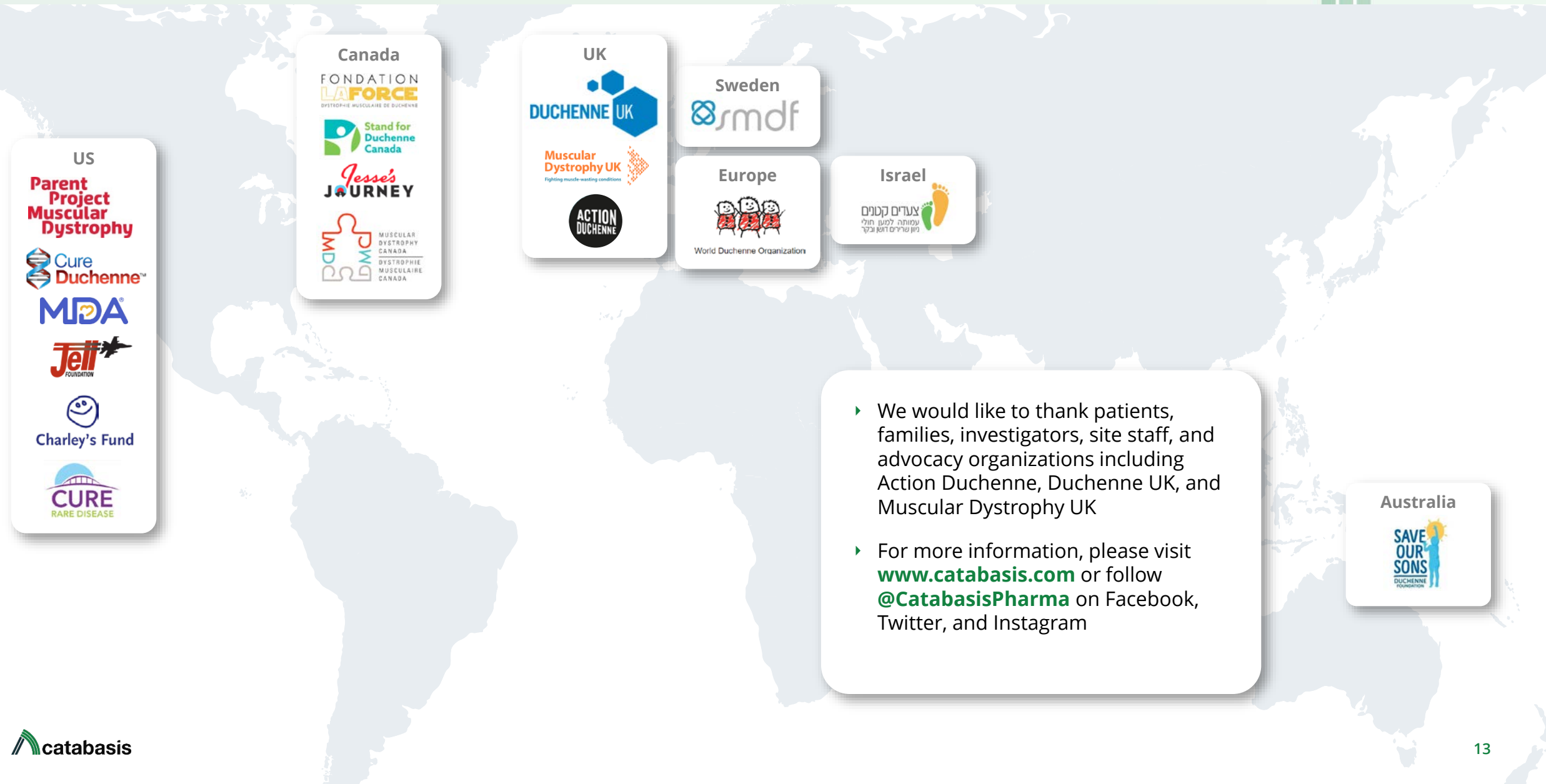
Catabasis' Focus on Edasalonexent for Duchenne



Our goal is for edasalonexent to become a new oral foundational therapy to slow disease progression for all affected by Duchenne

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!



- ▶ We would like to thank patients, families, investigators, site staff, and advocacy organizations including Action Duchenne, Duchenne UK, and Muscular Dystrophy UK
- ▶ For more information, please visit www.catabasis.com or follow [@CatabasisPharma](https://www.facebook.com/CatabasisPharma) on Facebook, Twitter, and Instagram