Our mission is to bring hope and life-changing therapies to patients and families affected by rare diseases

Catabasis Pharmaceuticals

December 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.

Catabasis and Edasalonexent: A Compelling Opportunity in DMD

Potential New Foundational Therapy in Duchenne Muscular Dystrophy (DMD)	▸ Promising disease-modifying oral NF-κB inhibitor ▸ Slowed disease progression compared to off-treatment control period in MoveDMD trial ▸ Fast Track, Rare Pediatric, and Orphan Drug designations from FDA ▸ Orphan Medicinal Product designation from European Commission ▸ Pivotal Phase 3 PolarisDMD trial fully enrolled, top-line results expected in Q4 2020 ▸ NDA filing expected in 2021
Significant Commercial Opportunity	▸ Potential differentiated foundational treatment for all DMD patients ▸ High unmet medical need in clear target market with strong patient advocacy and concentrated Centers of Excellence ▸ Unique mechanism could enable use as mono- or potentially as combination therapy with other treatments such as exon skipping, gene therapies and other approaches ▸ Market research indicates high likelihood of physician adoption and payer coverage
Expansion in DMD and Beyond	▸ Additional trial planned in non-ambulatory DMD patients ▸ Leverage benefits of inhibiting NF-κB in other potential indications
Leadership Depth and Focus	▸ Accomplished industry, financial and clinical leaders ▸ Seasoned team with experience in rare diseases and commercialization ▸ Strong IP position and wholly-owned assets

Edasalonexent: Potential for Broad Therapeutic Benefit

Activated NF-κB leads to disease progression in DMD

Skeletal Muscle

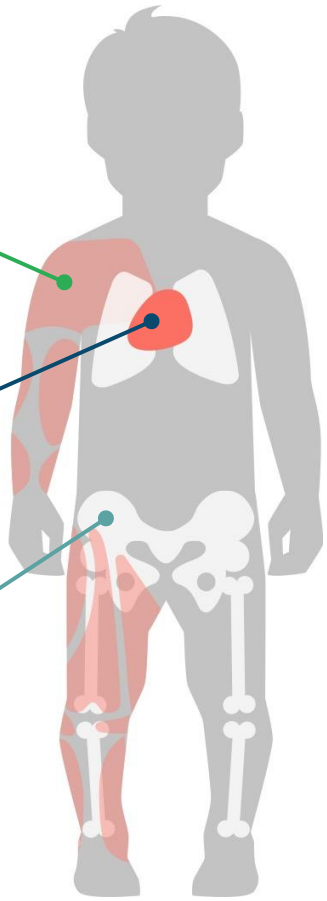
Loss of ambulation, upper limb function, respiratory failure

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

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

Our Vision for Edasalonexent

- Foundational therapy for all DMD patients, regardless of mutation, from time of diagnosis onwards
- Address skeletal and cardiac muscle disease and bone health
- As monotherapy and potential to be used with:
  - Other therapies, including exon-skipping and gene therapies
- Favorably differentiated safety and tolerability profile from other treatments

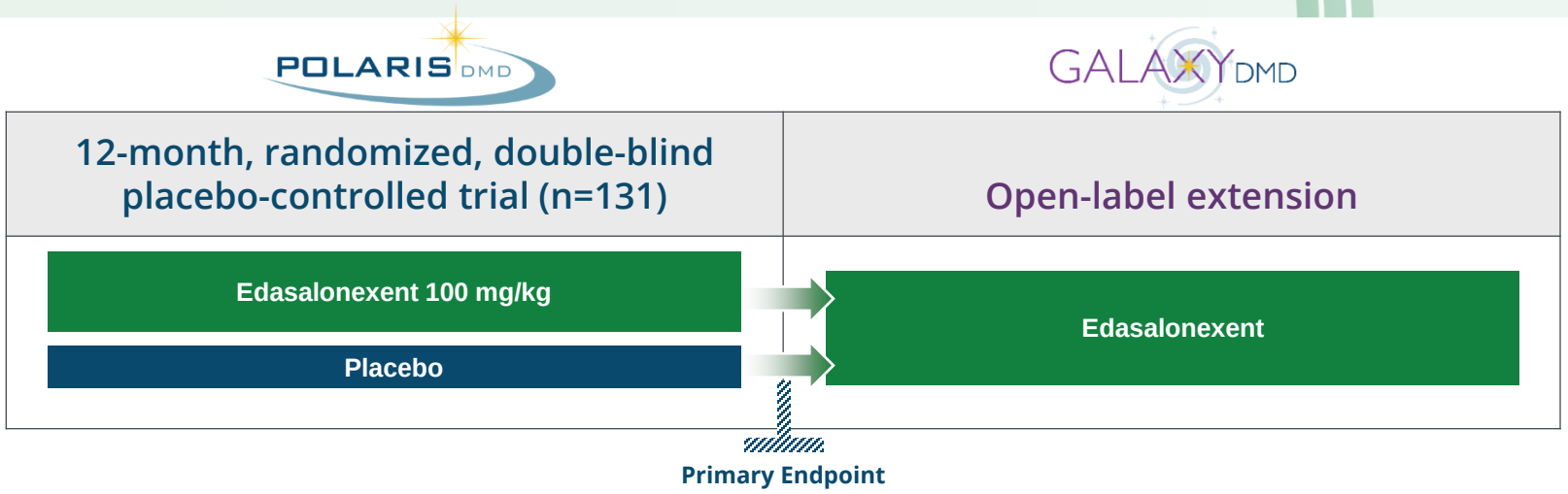
Developing a potential foundational therapy in DMD

Commercial Approach

- Disease-focused specialty sales force in US
- Establish global “go-to-market” strategies

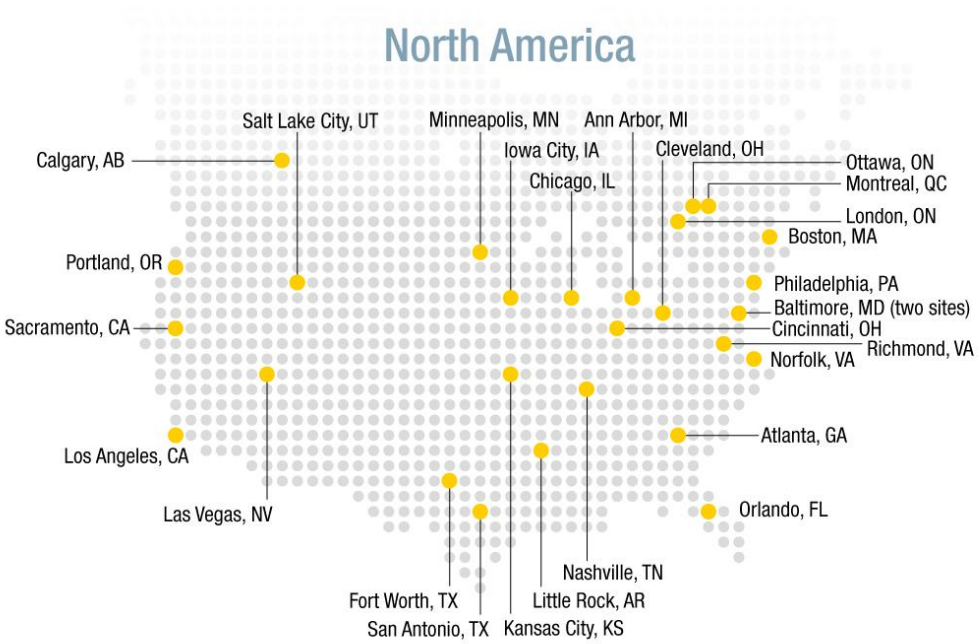
Edasalonexent is an investigational agent not currently approved in any territory

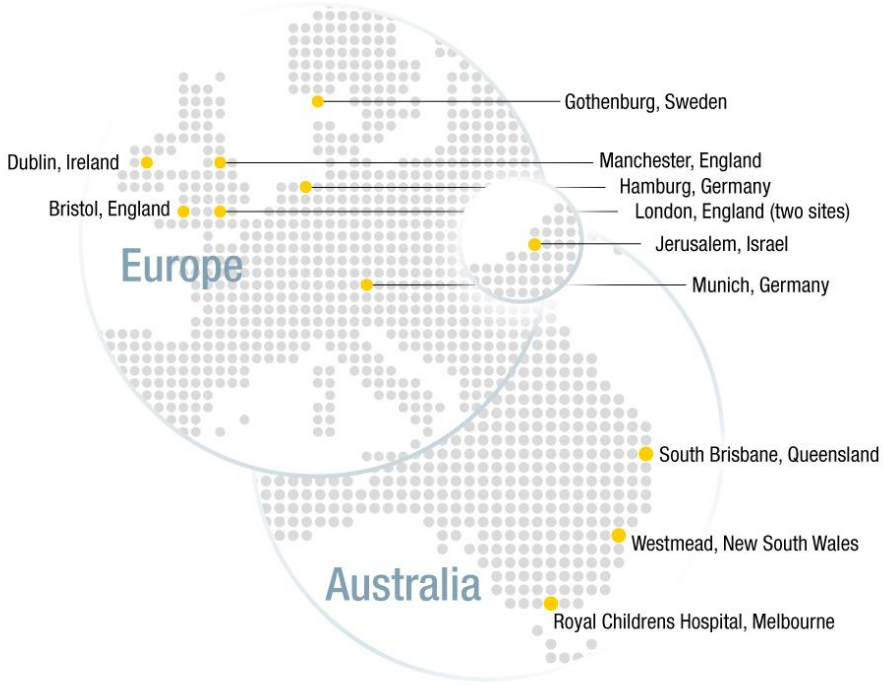
Fully Enrolled Edasalonexent Phase 3 PolarisDMD Trial Designed for Global Registration



- **Goal: Validate results from MoveDMD trial**
- **Eligibility:**
  - All mutations
  - Age 4 to 7 (up to 8th birthday); off steroids for ≥6 months
  - Boys on a stable dose of eteplirsen were eligible to enroll
- **Endpoints: Consistent with regulatory guidance**
  - Primary: Change in North Star Ambulatory Assessment
  - Key secondary: Age-appropriate timed function tests
  - Additional assessments include growth, cardiac and bone measures

Phase 3 PolarisDMD Clinical Trial Fully Enrolled





- **Enrolled 131 patients in 8 countries**

Phase 3 PolarisDMD Trial Incorporates Critical Aspects of Daily Function and Differentiating Assessments

Physical Function Outcomes

Primary Endpoint: North Star Ambulatory Assessment

Assessment measures

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

3 Timed Function Tests

Time to Stand

4-Stair Climb

10-Meter Walk/Run

Additional Outcomes

Growth

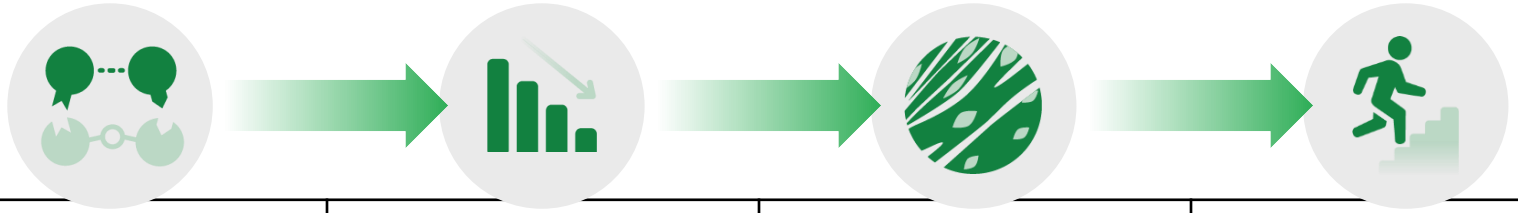
Cardiac Health

Bone Health

Patient Reported Outcomes

PolarisDMD Was Designed Based on Promising MoveDMD Trial Results

In Phase 2 MoveDMD Trial and Open-Label Extension:

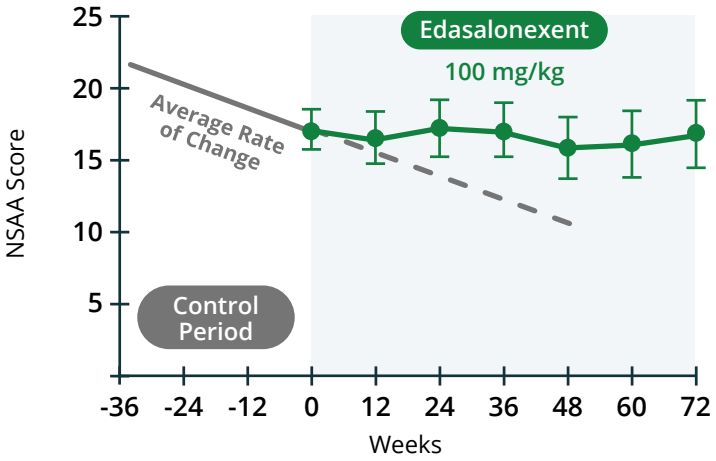


NF-κB Target Engagement	Biomarkers	Muscle MRI	Functional
✓ Inhibited NF-κB targeted gene set in peripheral blood	✓ Decreased CK and other muscle enzymes ✓ Decreased CRP, biomarker of inflammation	✓ Improved rate of change in MRI T2 and MRS muscle fat compared to off-treatment control	✓ Preserved NSAA and Timed Function Tests

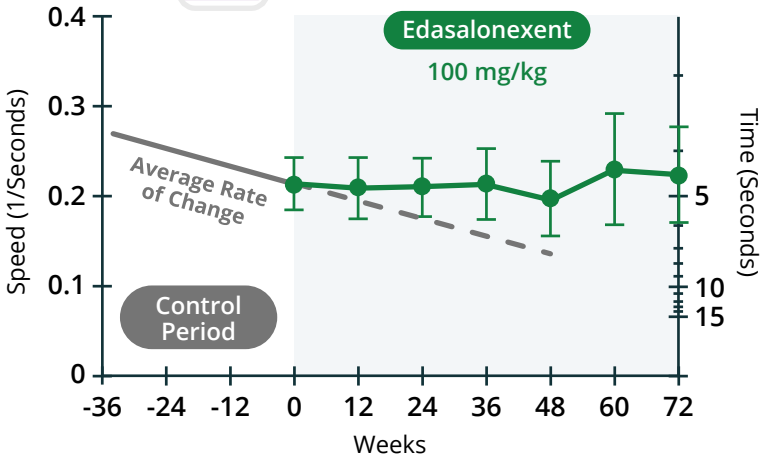
Edasalonexent Demonstrated Clinically Meaningful Slowing of Disease Progression

In Phase 2 MoveDMD Trial and Open-Label Extension:

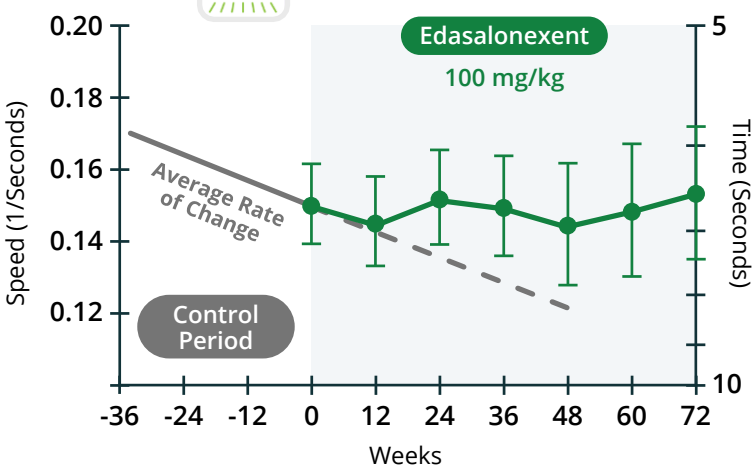
North Star Ambulatory Assessment



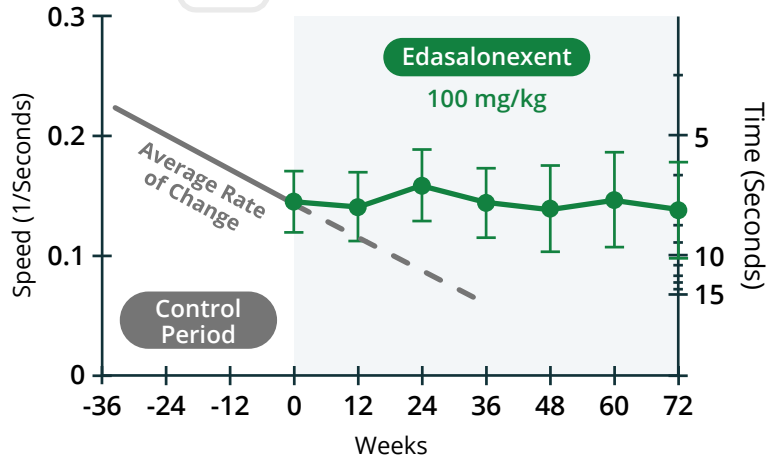
4-Stair Climb



10-Meter Walk/Run



Time to Stand



Better

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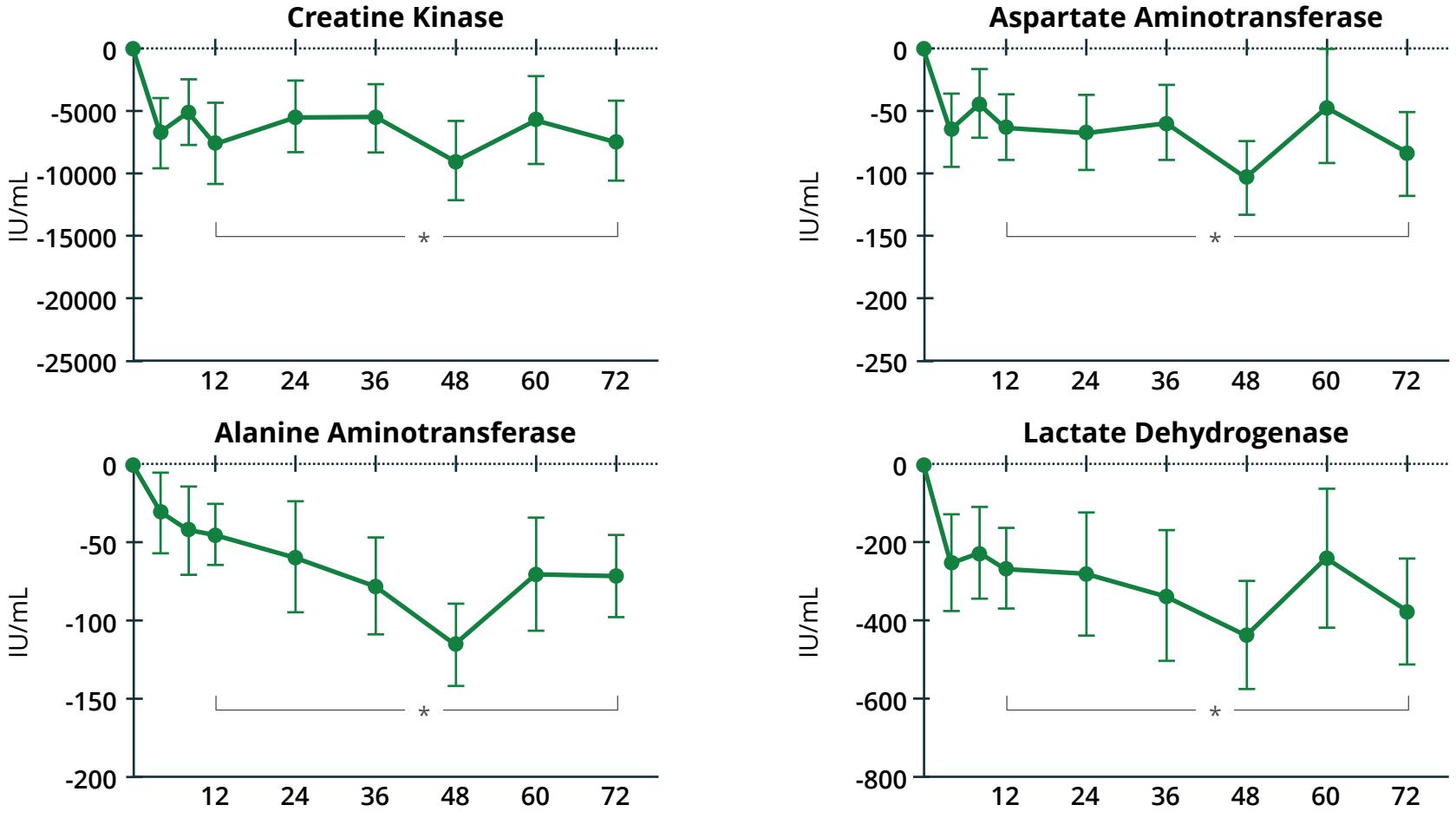
Means ± SEM shown. Includes data of all boys initially started on 100 mg/kg dose (n=16) with 11 boys participating through 72 weeks.
Results are compared to the off-treatment control period changes measured prior to boys in the MoveDMD trial receiving 100 mg/kg edasalonexent.

Muscle Enzymes Significantly Decreased on Edasalonexent, Supporting a Positive Impact on Muscle Health

In Phase 2 MoveDMD Trial and Open-Label Extension:

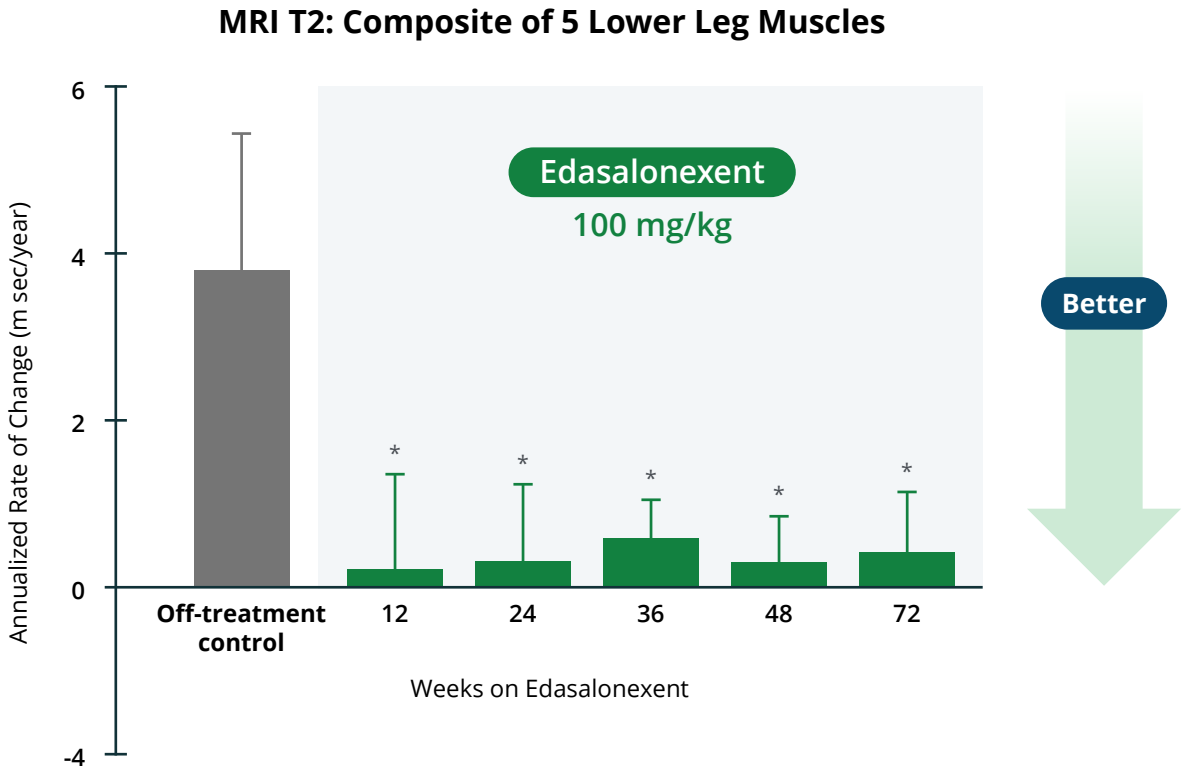


Plasma muscle enzymes are elevated 10 to 100 fold in DMD, indicative of leakage from damaged myocytes

Means ± SEM shown; * p<0.05 for change from baseline after 12 weeks

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

In Phase 2 MoveDMD Trial and Open-Label Extension:



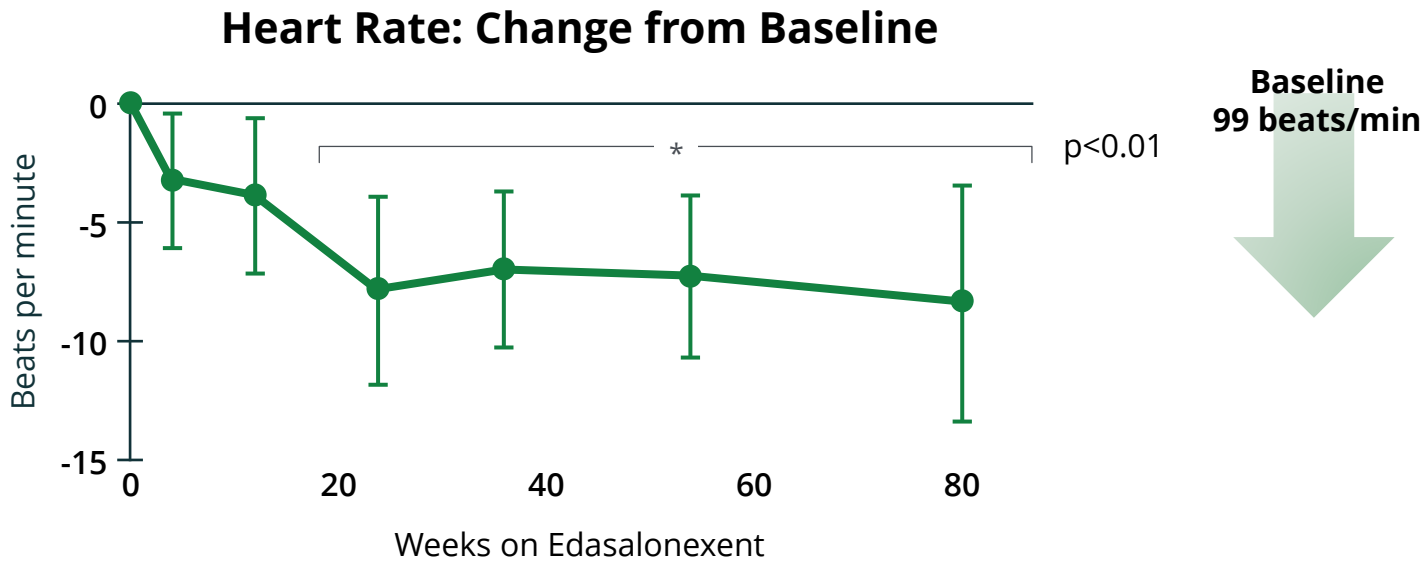
- MRI T2 increases over time in DMD as inflammation and fat content of muscle increases
- A composite MRI T2 measure of five lower leg muscles correlated well with current ability to perform time function tests in the ImagingDMD natural history database

Means + SEM; mixed model comparison with off-treatment period
* Week 12: p=0.002, n=16; Week 24: p=0.004, n=14; Week 36: p=0.032, n=13; Week 48: p=0.018, n=12; Week 72: p=0.052, n=9

Willcocks, et al, 2016, *Ann Neurol.* Presented at WMS 2018.

Edasalonexent Showed Potential for Cardiac Benefits in DMD

In Phase 2 MoveDMD Trial and Open-Label Extension:



- **Elevated resting heart rate is initial manifestation of cardiac disease in DMD**
  - Cardiac failure is a leading cause of mortality in DMD
  - Elevated heart rate triples the risk of cardiomyopathy several years later
  - Inhibiting NF-κB had positive effects on fibrosis in *mdx* and GRMD animal models
- **In MoveDMD trial, mean resting heart rate significantly decreased, approaching age-normative heart rate ~92 beats per minute**

Thomas, et al. *Pediatr Cardiol.* 2012 33(7):1175-9.
Hammers, et al. *JCI Insight* 2016 1(21): e90341
Fleming, et al., *Lancet* 2011 377: 1011-18

Edasalonexent Was Well Tolerated Without Known Side Effects of Steroids

In Phase 2 MoveDMD Trial and Open-Label Extension:

- **60+ patient years of exposure**
- **Well tolerated, with majority of adverse events mild in nature**
  - Most common related adverse event was diarrhea, which did not require discontinuation
- **Boys on edasalonexent in our Phase 2 MoveDMD trial and open-label extension grew similarly to unaffected boys**
  - Height increased by an average of 2.1 inches/year
  - Weight increased by an average of 2.9 pounds/year
  - Both increases are in line with typical height and weight increases of unaffected boys

Phase 3 PolarisDMD and Phase 2 MoveDMD Trials Have Similar Baseline Characteristics

- **Analysis shows that Phase 3 trial enrolled the expected patient population**
  - Comparison of baseline age and function (NSAA, time to stand, 4-stair climb, and 10-meter walk/run) were similar in both trials; there were no significant differences in baseline characteristics between the two trials*
- **Findings support the assumptions on which the Phase 3 trial was powered**

	PolarisDMD (n=131)	MoveDMD (n=23)
Age (years)	5.7 ± 1.0	6.0 ± 1.1
Percent enrolled patients that had not taken steroids	98%	100%
North Star Ambulatory Assessment (NSAA) score	20.8 ± 4.7	20.1 ± 5.5
10-Meter Walk/Run speed (1/s)	0.181 ± 0.037	0.168 ± 0.045
4-Stair Climb speed (1/s)	0.265 ± 0.097	0.254 ± 0.110
Time to Stand speed (1/s)	0.212 ± 0.070	0.193 ± 0.080

Means ± standard deviation shown
*Kolmogorov-Smirnov test used to assess for population distribution differences

DMD Patient Prevalence Population Is Well-Defined

Affects **1 in 3,500-5,000 Males*** Worldwide

Approximately
15,000
Males* in the US

Approximately
19,000
Males* in the EU

- Because Duchenne gene is found on the X-chromosome, it primarily affects males, while females are typically carriers

Source: McDonald C., et al., *Lancet Neurol.* 2018, 17:389-91.
* Some females do present with DMD, exact prevalence unknown

DMD Patient Segmentation and Typical Progression Is Well Established and Understood

2 - 5 years

Diagnosed by Age 5

- Affected boys show clinical signs and symptoms

4 - 7 years

Early Ambulatory

- Gower's Maneuver
- Waddling gait
- Maybe toe-walking
- Climbs stairs slowly

8 - 12 years

Late Ambulatory

- Labored gait
- Losing ability to climb stairs and rise from floor

12 + years

Early Non-Ambulatory

- May be able to self-propel for some time
- Able to maintain posture
- May develop scoliosis

Late Non-Ambulatory

- Upper limb function and postural maintenance is increasingly limited
- Declining respiratory function
- Cardiac disease manifested

Source: http://www.dmd-guide.org/en-US/, CureDuchenne; All ages represented in the progression timeline are generalized and approximations

Clear Market Need in DMD with Limited Treatment Options

- **Currently, there is no cure for DMD**
- **Today, the majority of patients are treated with corticosteroids**
  - Despite broad market utilization, steroids have long-term negative consequences
- **Only a small portion of the population can be treated with eteplirsen (US) or ataluren (EU)**

Current Landscape of medical management

Steroids

Deflazacort and Prednisone

Known Benefits:

- Delayed loss of muscle function

Known Side Effects:

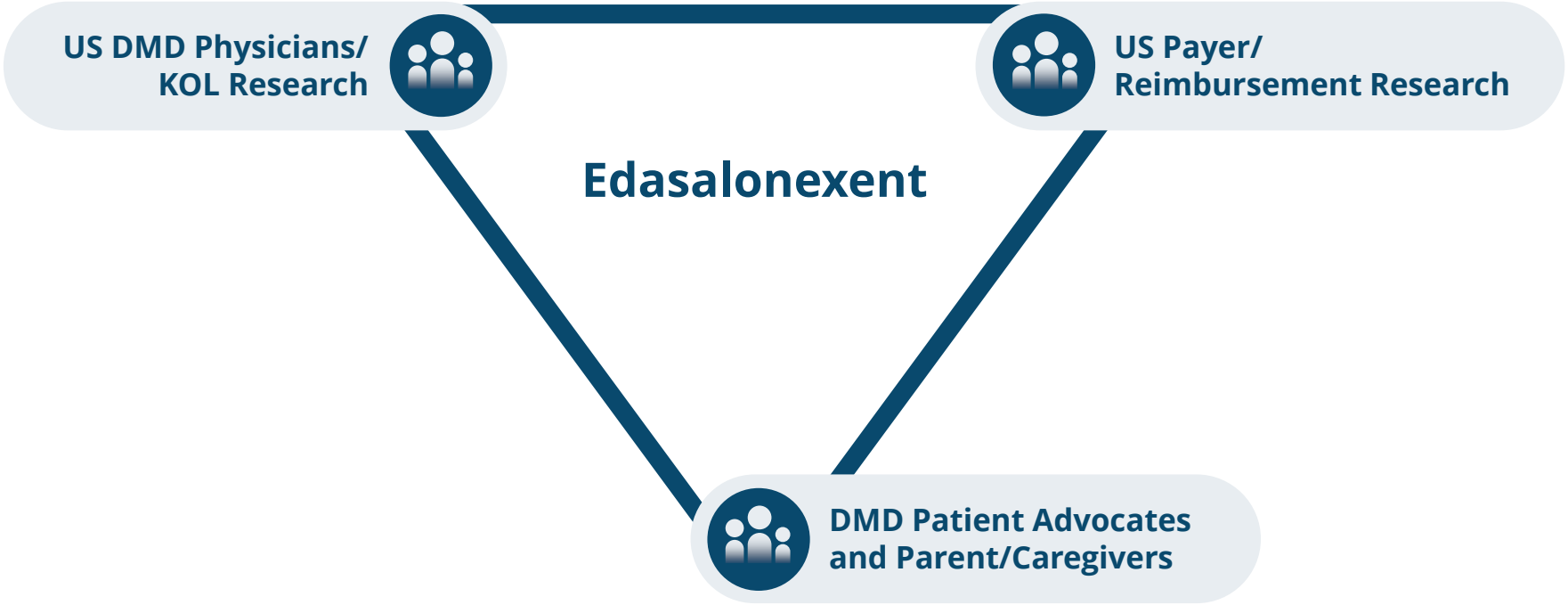
- Osteoporosis with fractures
- Metabolic effects
- Weight gain, obesity
- Growth retardation
- Delayed puberty
- Cataracts
- Muscle atrophy
- Behavioral issues
- Cushingoid appearance

Mutation Targeted

Eteplirsen (US) and Ataluren (EU)

- Safe and tolerable
- Both approvals require additional studies
- Limited suitable patient populations (13% for each targeted population)

Research Shows Support for Edasalonexent for DMD Among Key Stakeholders



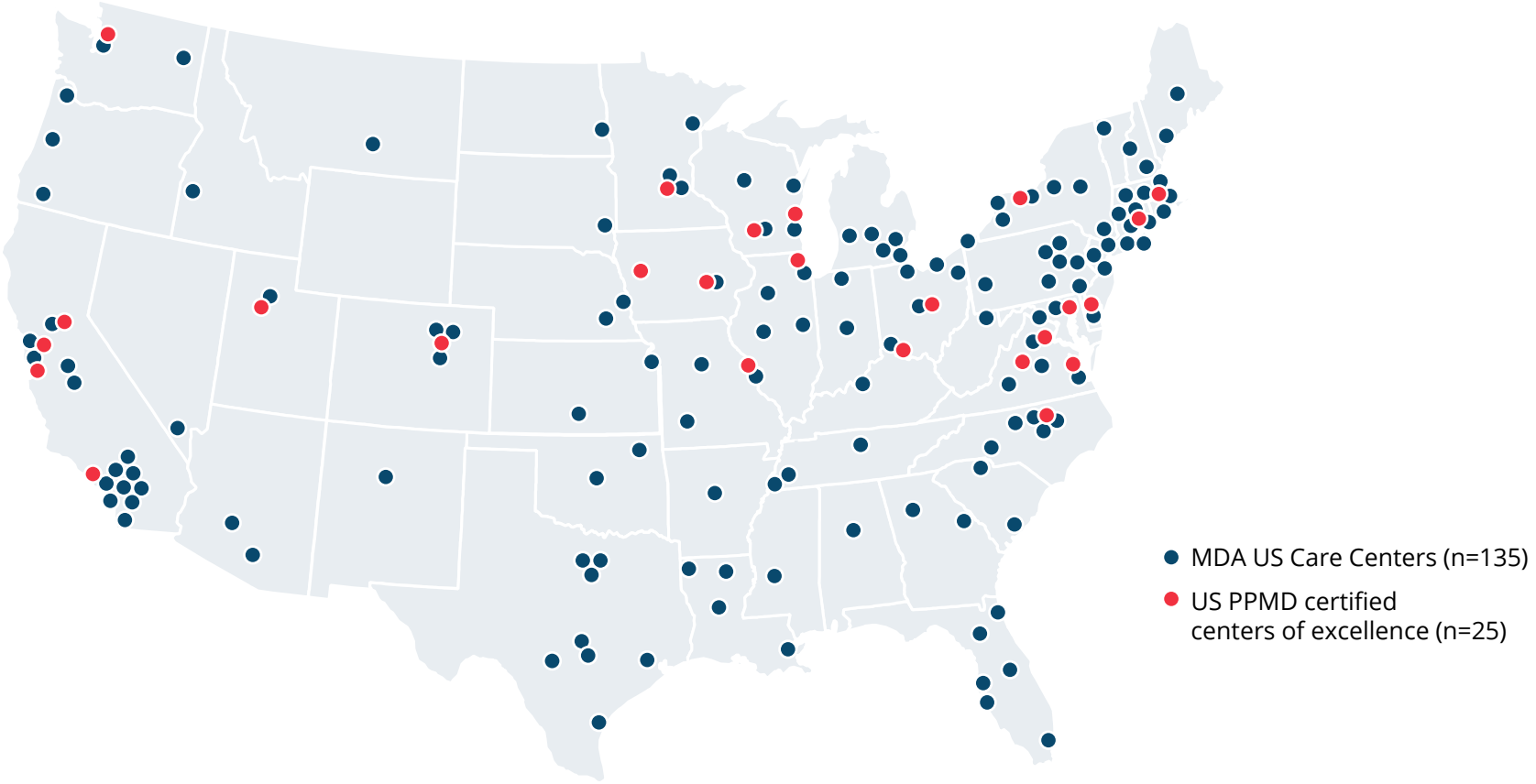
Potential to meet the needs of the DMD community:

- Strong efficacy profile in MoveDMD
- Well tolerated and no safety concerns to date
- A potential treatment for all boys
- Use as mono- or potential combo-therapy
- Age appropriate development similar to peers

Sources: Catabasis Market Research conducted by Health Advances, 2018 and Blueprint Partnership, 2019

Most US DMD Patients Have Access to Expert Care and Treatment

- **Concentrated centers of excellence enable targeted sales and medical affairs field efforts**
  - Targeting specialists for education and awareness of the role of NF-κB in DMD and the potential for edasalonexent to impact disease progression



Source: MDA US Care Centers Listing, September 2019 https://www.mda.org/care/care-center-list and US PPMD certified centers of excellence

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Catabasis Has Developed Strong Relationships with Global DMD Patient Advocacy Organizations

US

Parent Project Muscular Dystrophy

Cure Duchenne

MDA

Jett Foundation

Charley's Fund

CURE RARE DISEASE

Canada

FONDATION LAFORCE

Stand for Duchenne Canada

Jesse's Journey

MDMC Muscular Dystrophy Canada / Dystrophie musculaire Canada

UK

DUCHENNE UK

Muscular Dystrophy UK

ACTION DUCHENNE

Sweden

smdf

Europe

World Duchenne Organization

Israel

צעדים קטנים

Australia

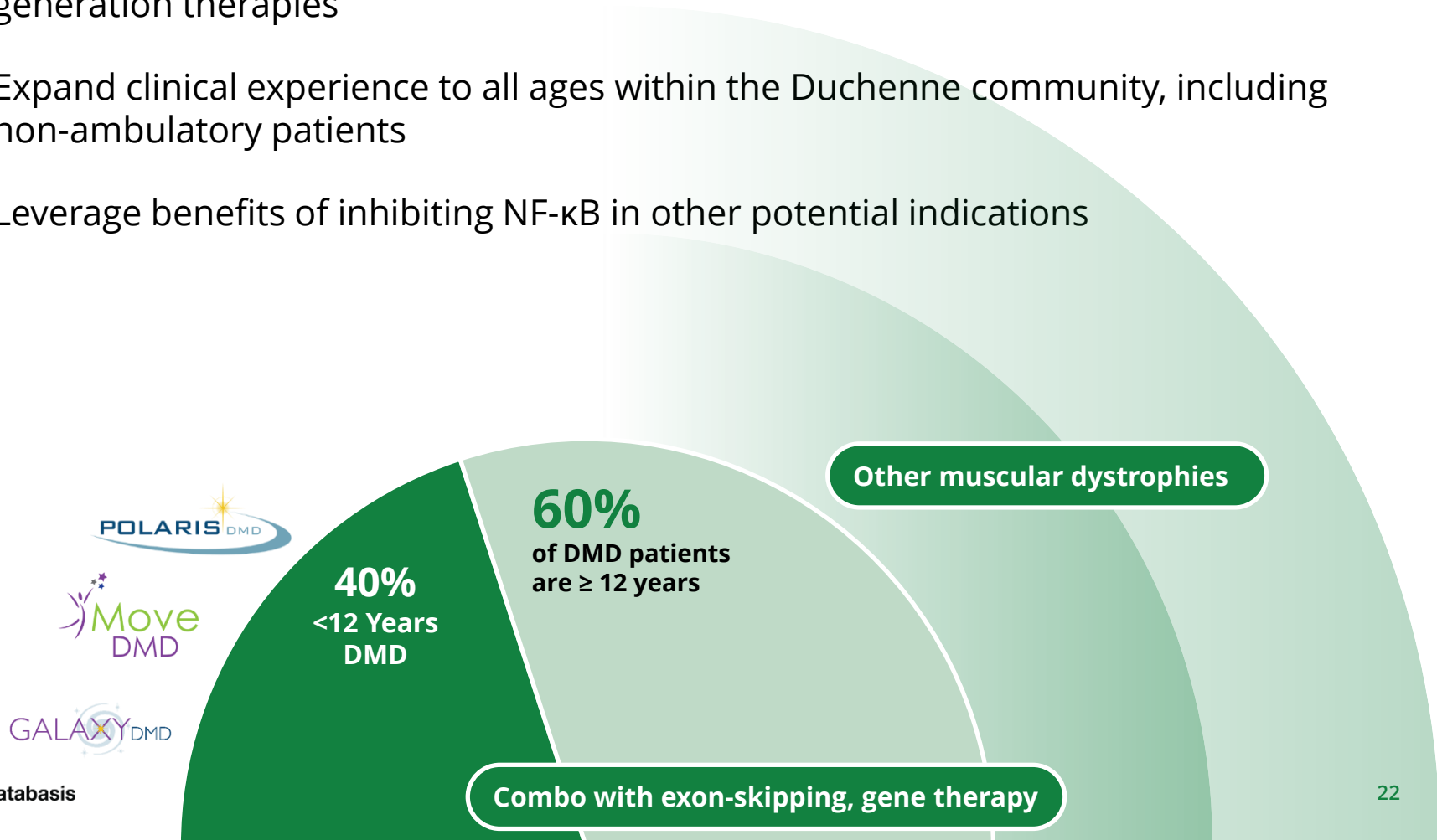
SAVE OUR SONS DUCHENNE FOUNDATION

- **Commitment to patients, caregivers and the community** to help individuals with DMD
- **Proud to partner with DMD Advocacy organizations** to enroll Polaris Phase 3
- **Ongoing collaborations** to drive education and awareness among treaters and payors

Edasalonexent: Beyond Ambulatory DMD

Potential Opportunities

- Demonstrate ability to be used in combination with dystrophin-targeted and next-generation therapies
- Expand clinical experience to all ages within the Duchenne community, including non-ambulatory patients
- Leverage benefits of inhibiting NF-κB in other potential indications



Catabasis Is Striving to Improve the Lives of Patients Affected by DMD

NF-κB Targeted MOA

- Chronic activation of NF-κB is a well-recognized driver of disease progression in DMD
- Edasalonexent inhibits NF-κB and has a novel mechanism among the therapies available or in development for DMD with broad potential benefits
- Edasalonexent slowed disease progression with a favorable safety profile in MoveDMD trial

Potential Foundational Therapy

- Potential for edasalonexent to be used as monotherapy or in combination with current and next-generation DMD treatments
- Oral therapy

Favorable Market Profile

- Strong interest from physicians and KOLs
- Market research indicates high likelihood of physician adoption and payer coverage
- Potential to meet the needs and desires of the DMD community

Relationship Focus

- Developing best-in-class internal capabilities and forming critical partnerships to execute a flawless clinical trial and subsequent launch

Market Preparation

- Hired Chief Commercial Officer
- Commercialization planning underway