



Edasalonexent (CAT-1004) Program

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

Catabasis Pharmaceuticals
February 15th 2018

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 plans to commence a single global Phase 3 trial in Duchenne muscular dystrophy, or DMD, in the first half of 2018 to evaluate the efficacy and safety of edasalonexent for registration purposes, our plans to report top-line results from this trial in 2020 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 the Company’s product candidates, including the final trial design of our planned Phase 3 trial in DMD; availability and timing of results from preclinical studies and clinical trials, including the availability of top-line results from our planned Phase 3 trial in DMD in 2020; 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; our ability to obtain financing on acceptable terms and in a timely manner to fund our planned Phase 3 trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes; availability of funding sufficient for the Company’s foreseeable and unforeseeable operating expenses and capital expenditure requirements; other matters that could affect the availability or commercial potential of the Company’s product candidates; and general economic and market conditions and other factors discussed in the “Risk Factors” section of the Company’s Quarterly Report on Form 10-Q for the period ended September 30, 2017, which is on file with the Securities and Exchange Commission, and in other filings that the Company 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

- ▶ 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 to become a foundational oral therapy to slow the rate of progression for all people affected by Duchenne at all ages as stand alone agent and in combination with other therapies.

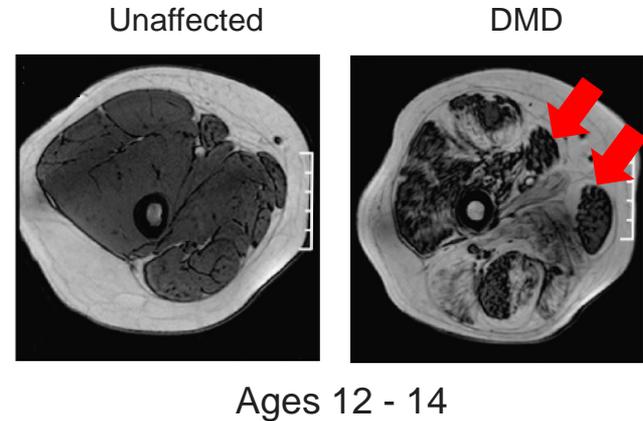


- ▶ **Results of the MoveDMD clinical trial support edasalonexent's advancement into Phase 3 clinical trials, planned to start in the first half of 2018.**

Edasalonexent (CAT-1004), an Investigational Drug Candidate Being Developed for DMD

▶ Why Edasalonexent?

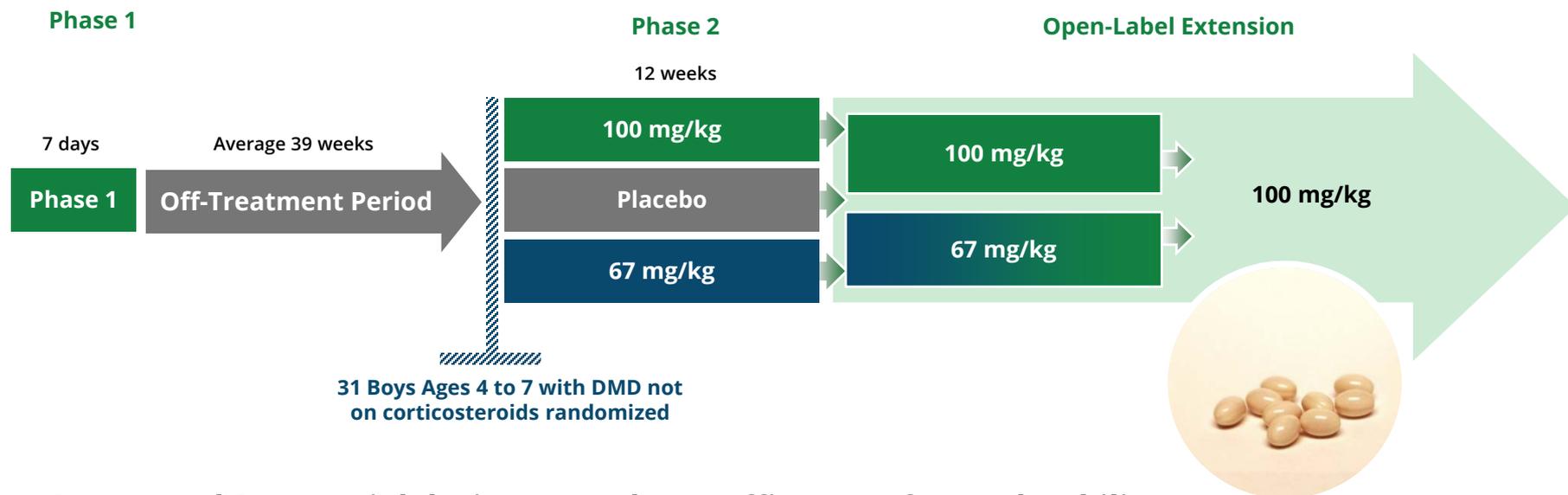
- In Duchenne, lack of dystrophin and mechanical stress activate NF- κ B in muscles, leading to muscle degeneration, inflammation, fibrosis and inhibition of muscle regeneration and ultimately loss of function
- Steroids suppress inflammation but have significant side effects
- Pre-clinical models support positive effects on skeletal, respiratory and cardiac muscle
- Phase 1 trials in adults showed no safety signals and that edasalonexent targets NF- κ B



▶ Catabasis has been conducting the MoveDMD trial to understand the effects of edasalonexent in young boys with Duchenne



MoveDMD Trial Designed to Inform Phase 3

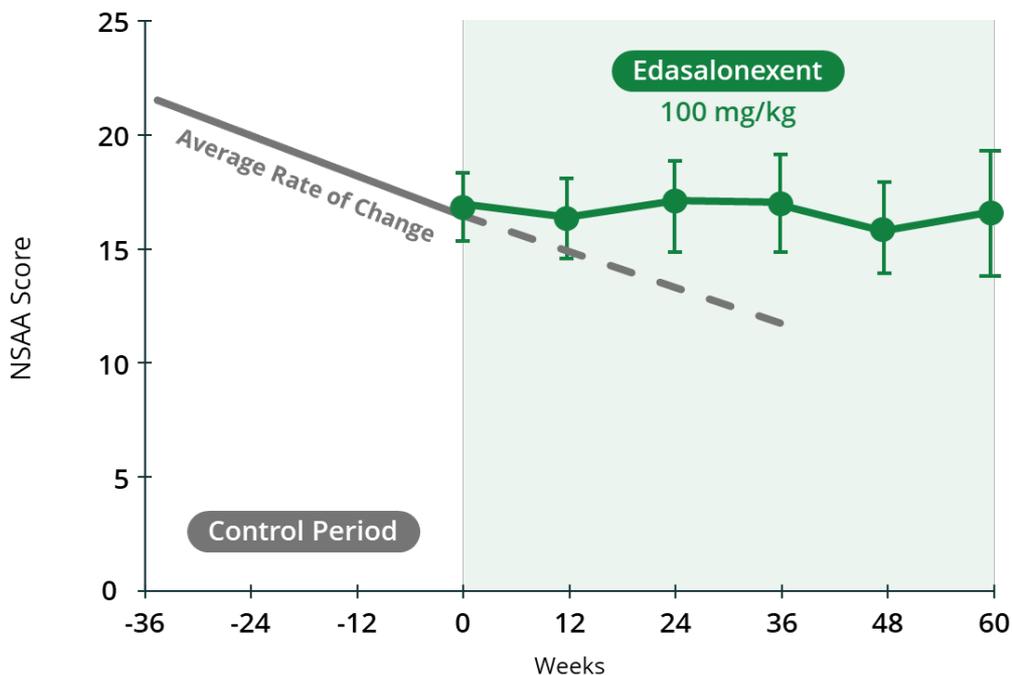


- ▶ **Integrated 3-part trial design to evaluate efficacy, safety, tolerability**
 - Assessments included North Star Ambulatory Assessment, age-appropriate timed function tests, MRI
- ▶ **Off-treatment control period measurements between Phase 1 and Phase 2**
 - Provided internal control for planned analyses
- ▶ **Phase 2 showed favorable trends towards the slowing of disease progression after 12 weeks with no safety issues**
- ▶ **Open-label extension enabled assessment of safety and efficacy following longer term treatment**

North Star Ambulatory Assessment Score Stabilized with Edasalonexent Treatment



North Star Ambulatory Assessment

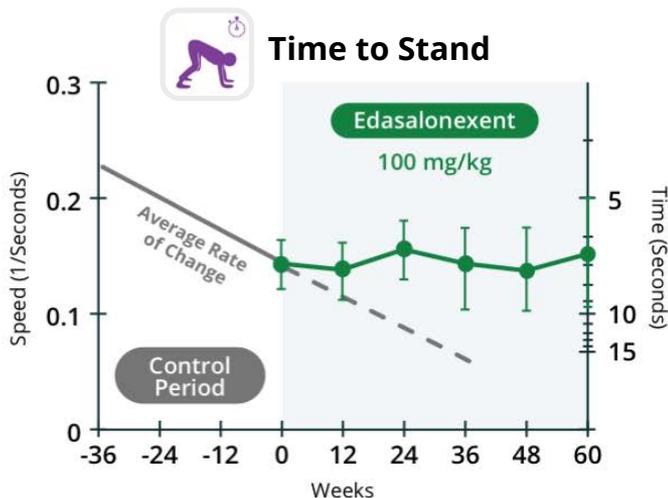
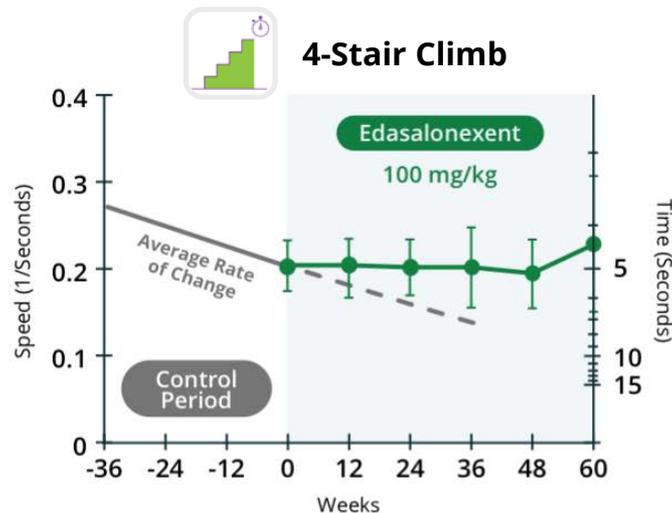
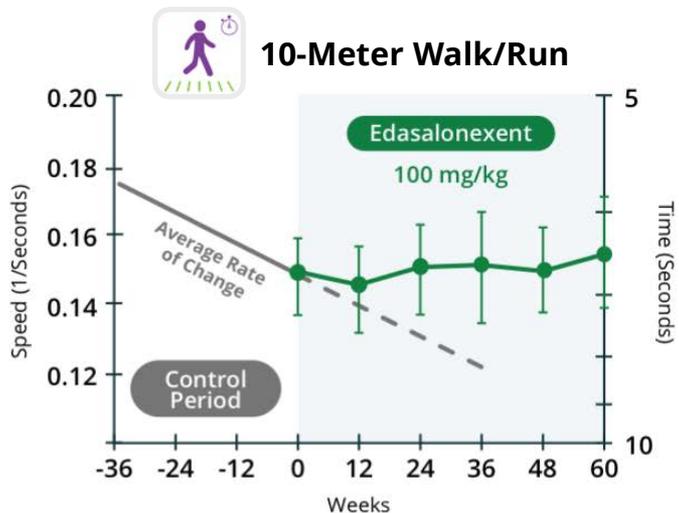


► Disease progression on edasalonexent improved compared with rate of change during off-treatment control period

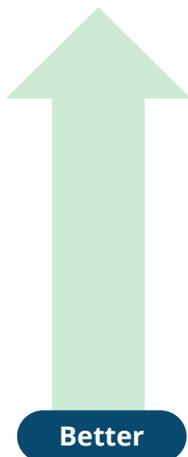
All Timed Function Tests Speed Stabilized with Edasalonexent Treatment



Pre-Specified Analyses

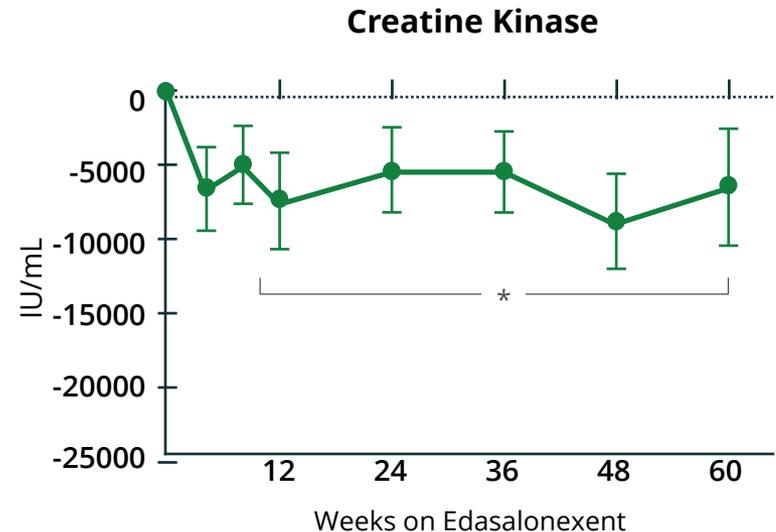


- ▶ Disease progression on edasalonexent improved compared with rate of change during off-treatment control period

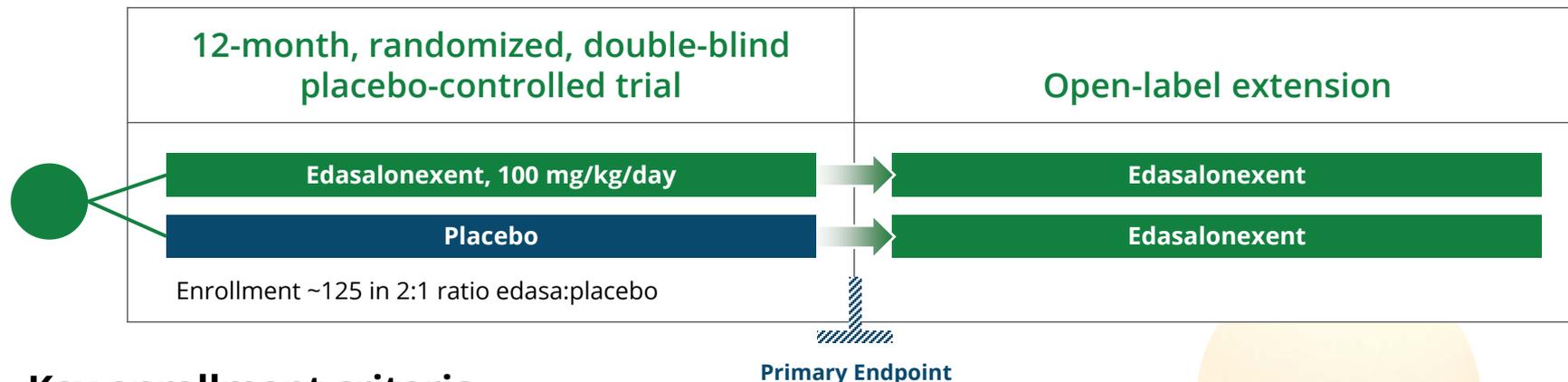


Edasalonexent: Well Tolerated without Safety Signals

- ▶ **No safety signals to date**
- ▶ **Well tolerated, with most adverse events mild in nature, mostly gastrointestinal**
 - Most common treatment-related adverse event was mild and transient diarrhea
 - No serious treatment-related adverse events or dose reductions
- ▶ **No adverse trends in laboratory tests**
 - Decreases in all 4 muscle enzymes
- ▶ **Growth: Age appropriate weight, height and BMI changes through 60 weeks of treatment**
- ▶ **Heart rate decreased toward normal values at this age**



Positive MoveDMD Data Support Planned Global Phase 3 Registration Trial for Edasalonexent



▶ Key enrollment criteria

- Age 4 to 7th birthday
- Able to complete timed function tests
- Not on corticosteroids for at least 6 months
- Not on other investigational therapies for at least 2 months, 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, 6 minute walk test or MRI

▶ Locations: US, Canada, Europe and Australia – specific sites to be determined



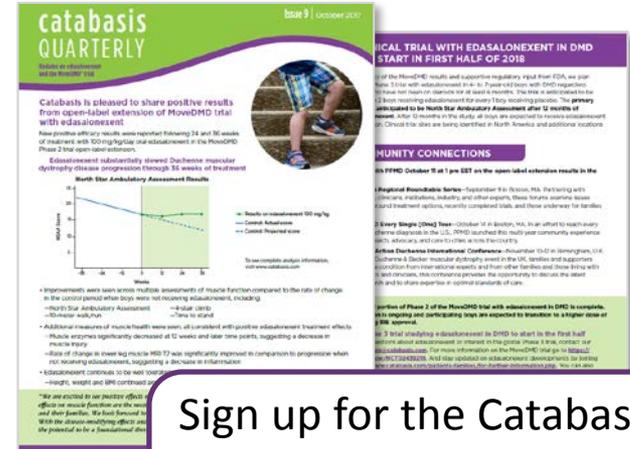
Edasalonexent: Potential to Slow Disease Progression for All Boys with DMD

- ▶ Investigational oral disease-modifying agent for all patients with DMD, regardless of mutation type
- ▶ Edasalonexent substantially slowed DMD disease progression through 60 weeks
- ▶ Phase 3 expected to start in first half of 2018
- ▶ Potential as monotherapy and also exploring potential to combine with dystrophin-targeted and other therapies



Thank You

- ▶ Patients and families
- ▶ Patient groups
- ▶ ImagingDMD Investigators and Staff
- ▶ For questions:
 - Email Joanne Donovan, M.D., Ph.D. and the Clinical Team: DMDtrials@catabasis.com



The image shows a preview of the 'catabasis QUARTERLY' newsletter. The header includes the title 'catabasis QUARTERLY' and the issue information 'Issue 9 | October 2018'. The main content area features a headline: 'Catabasis is pleased to share positive results from open-label extension of MovDMD trial with edasalonexent'. Below this is a line graph titled 'North Star Ambulatory Assessment Results' showing 'mMTC (min)' on the y-axis (0 to 10) and 'Weeks' on the x-axis (-4 to 16). Three data series are plotted: 'Needs no assistance 100 mg/kg' (solid line with circles), 'Needs Assistance' (dashed line with squares), and 'Cannot Ambulate' (dotted line with triangles). The graph shows a general downward trend for the 'Needs no assistance' group and an upward trend for the 'Needs Assistance' group. To the right of the graph is a small photo of a child running. Below the graph is a list of key findings and a quote from a parent. The right side of the newsletter preview contains sections for 'CLINICAL TRIAL WITH EDASALONEXENT IN DMD START IN FIRST HALF OF 2018' and 'COMMUNITY CONNECTIONS', including details about a regional Roundtable Series and a 'Every Single One' (ESO) Tour.

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