Edasalonexent
(CAT-1004) Program

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

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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 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 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 Annual Report on Form 10-K for the period ended December 31, 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.
Catabasis’ Focus on Edasalonexent for Duchenne

- 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 an oral new foundational therapy to slow disease progression for all people affected by Duchenne at all ages as a single agent and in combination with other therapies.

- PolarisDMD, a Phase 3 clinical trial of edasalonexent, is enrolling boys with Duchenne
Edasalonexent: Potential to Slow Disease Progression for All Those Affected by Duchenne

‣ 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 Phase 2 MoveDMD trial and open-label extension, edasalonexent substantially slowed disease progression compared to off-treatment control period

‣ 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

Vision for Edasalonexent, and NF-κB inhibitor

Goal: Improve skeletal muscle function
Goal: Preserve cardiac function
Goal: Reduce risk of fractures

NF-κB is a key link between loss of dystrophin and disease pathology; plays fundamental role in the initiation and progression of skeletal muscle, respiratory and cardiac disease in DMD
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 pre-specified MoveDMD analyses
  - Compared off-treatment control period disease progression with available natural history data

- Open-label extension enabled assessment of safety and efficacy following longer term treatment

**Phase 1**
- Off-Treatment Period
- 31 boys ages 4 to 7 with DMD not on corticosteroids randomized
- 7 days

**Phase 2**
- 12 weeks
- 100 mg/kg
- Placebo
- 67 mg/kg

**Open-Label Extension**
- 100 mg/kg

- **Phase 1**
  - 31 boys ages 4 to 7 with DMD not on corticosteroids randomized
  - 7 days

- **Phase 2**
  - 12 weeks
  - 100 mg/kg (Open-Label Extension)
  - Placebo
  - 67 mg/kg

- **Open-Label Extension**
  - 100 mg/kg
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 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

**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
- Decrease in muscle fat accumulation
In the Phase 2 MoveDMD Trial and Open-Label Extension, Edasalonexent Preserved Muscle Function Compared to Off-Treatment Period

Edasalonexent Treatment Stabilized North Star Ambulatory Assessment Score

Edasalonexent Treatment Stabilized Timed Function Tests

Means ± SEM shown
Safety: Edasalonexent Has Been Well-Tolerated to Date

- 50+ years of patient exposure
  - Majority of adverse events mild in nature

- Boys on edasalonexent in our Phase 2 MoveDMD and open-label extension trial grew similarly to unaffected boys
  - Height increased by an average of 2.1 inches/year, while weight increased by an average of 2.9 lbs/year, both in line with typical height and weight increases of unaffected boys
Positive MoveDMD Data Support Phase 3 Registration Trial for Edasalonexent

12-month, randomized, double-blind placebo-controlled trial

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<th>Open-label extension</th>
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<tr>
<td>Edasalonexent, 100 mg/kg/day</td>
<td>Edasalonexent</td>
</tr>
<tr>
<td>Placebo</td>
<td>Edasalonexent</td>
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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 to Improve Patient Access, with 22 Open for Enrollment

Nearly 40 sites expected globally, many sites active and enrolling patients
Thank You!

- Patients and families
- Patient groups
- ImagingDMD Investigators and Staff
- For questions regarding the Phase 3 clinical trial:
  - Email Joanne Donovan, M.D., Ph.D. and the Clinical Team: DMDtrials@catabasis.com
- For frequent updates on edasalonexent and PolarisDMD
  - Follow @CatabasisPharma on Facebook, Twitter and Instagram

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