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 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 Annual Report on Form 10-K for the year ended December 31, 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 a stand alone agent and in combination with other therapies.

- Results of the MoveDMD clinical trial support edasalonexent’s advancement into Phase 3 clinical trial.
Edasalonexent (CAT-1004), an Investigational Drug Candidate Being Developed for DMD

Why Edasalonexent?

- In Duchenne, lack of dystrophin and mechanical stress activate NF-κB 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-κB.

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
  - Provides internal control for pre-specified MoveDMD analyses
  - To confirm consistency of patient off-treatment control period disease progression with available natural history data

- 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

Means ± SEM shown

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

- **10-Meter Walk/Run**
  - Average Rate of Change
  - Edasalonexent 100 mg/kg
  - Control Period

- **4-Stair Climb**
  - Average Rate of Change
  - Edasalonexent 100 mg/kg
  - Control Period

- **Time to Stand**
  - Average Rate of Change
  - Edasalonexent 100 mg/kg
  - Control Period

- Disease progression on edasalonexent improved compared with rate of change during off-treatment control period
Changes in Fat Fraction on Edasalonexent Consistent with Slowing of Disease Progression

- Increases in fat fraction correlate with worsened and predict future loss of functional milestones*

- Following 48 weeks of edasalonexent the rate of increase in fat fraction of the soleus and vastus lateralis was substantially decreased as compared to the off-treatment control period

- In the ImagingDMD natural history study, boys were largely on steroids

Baseline fat fraction in the soleus was 9.3% and in the VL 13.1%
At 48 weeks, MRS T2, reflecting inflammation only, decreased by -1.1 and -1.2 msec for the soleus and VL, respectively.


### MR Spectroscopy

<table>
<thead>
<tr>
<th>Muscle</th>
<th>MoveDMD Off-Treatment Control Period Annualized Rate</th>
<th>MoveDMD 48 weeks on Edasalonexent</th>
<th>ImagingDMD Natural History Study* 1 Year Change</th>
</tr>
</thead>
<tbody>
<tr>
<td>Soleus</td>
<td>2.6%</td>
<td>0.85%</td>
<td>3%</td>
</tr>
<tr>
<td>Vastus lateralis</td>
<td>10.4%</td>
<td>5.9%</td>
<td>7%</td>
</tr>
</tbody>
</table>
Edasalonexent: Well Tolerated without Safety Signals

- No safety signals in MoveDMD trial to date
- Well tolerated, with majority of adverse events being mild in nature, mostly gastrointestinal
- No adverse trends in hematology, chemistry, renal or adrenal function, calcium and phosphate
- Growth: Age-appropriate increases in weight and height
- Heart rate decreased toward normal values at this age

Creatine Kinase

- *p<0.05 for change from baseline after 12 weeks

BMI

- Percentile on Standard Growth Curve

Weeks on Edasalonexent

IU/mL

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

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

<table>
<thead>
<tr>
<th>Group</th>
<th>Dosing</th>
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<tbody>
<tr>
<td>Edasalonexent, 100 mg/kg/day</td>
<td>Placebo</td>
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</tbody>
</table>

**Open-label extension**

<table>
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<td>Edasalonexent</td>
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Enrollment ~125 in 2:1 ratio edasa:placebo

### Key enrollment criteria
- Age 4 to 7\textsuperscript{th} 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, 6 minute walk test or MRI

### Expected 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 compared to control through 60 weeks

- Preparing for Phase 3 clinical trial

- 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

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