Edasalonexent (CAT-1004) Program

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

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Catabasis Pharmaceuticals
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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 trial in Duchenne muscular dystrophy, or DMD, to evaluate the efficacy and safety of edasalonexent for registration purposes, 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 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 June 30, 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 standard of care 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, has been initiated.
Edasalonexent: Potential to Slow Disease Progression for All Those Affected by Duchenne

- Investigational oral disease-modifying agent for all patients with Duchenne, regardless of mutation type: potential for new standard of care
- Edasalonexent substantially slowed disease progression compared to control
- Potential treatment alone and also exploring potential to combine with dystrophin-targeted therapies
Edasalonexent inhibits NF-κB, which is the key link between absence of dystrophin and disease pathology and plays a fundamental role in the initiation and progression skeletal and cardiac muscle disease in DMD.

Edasalonexent is an oral small molecule that inhibits NF-κB and improves skeletal, diaphragm and cardiac disease in mouse and dog models of 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
  - To confirm consistency of patient 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
- 31 boys ages 4 to 7 with DMD not on corticosteroids randomized
- 7 days
- Off-Treatment Period

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

Open-Label Extension
- 100 mg/kg
Edasalonexent: Translation from Biomarkers to Functional Improvements in Duchenne

**NF-κB Target Engagement**

**Biomarker Improvements**

**Muscle MRI Improvements**

**Functional Improvements**

In adults and boys in MoveDMD:

- Decrease in activated NF-κB
- Decrease in NF-κB gene expression

In MoveDMD:

- Decrease in C-reactive protein, a biomarker of inflammation from baseline
- Decrease in muscle enzymes from baseline
- Improvement in rate of change in MRI T2 compared to control
- Decrease in muscle fat accumulation compared to control
- Slowing of decline in function as assessed by NSAA and Timed Function Tests compared to control
North Star Ambulatory Assessment Score
Stabilized with Edasalonexent Treatment

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

Includes data of all boys initially started on 100 mg/kg dose (n=16)

Means ± SEM shown
All Timed Function Tests Speed Stabilized with Edasalonexent Treatment

Pre-Specified Analyses

10-Meter Walk/Run
- Disease progression on edasalonexent improved compared with rate of change during off-treatment control period

4-Stair Climb

Time to Stand

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

Means ± SEM shown
Includes data of all boys initially started on 100 mg/kg dose (n=16)
Safety: Edasalonexent Well-Tolerated

- No safety signals to date

- Boys on edasalonexent grow similar to unaffected boys
  - Favorably differentiated from typical weight gain and curtailed growth seen with corticosteroid standard of care

Percentiles Compared to CDC Growth Charts

Heart rate is known to be elevated in Duchenne. On edasalonexent, heart rate decreased toward values for unaffected boys at this age

Heart Rate: Change from Baseline

Positive MoveDMD Data Support Phase 3 Registration Trial for Edasalonexent

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

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<thead>
<tr>
<th>Edasalonexent, 100 mg/kg/day</th>
<th>Placebo</th>
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<td>Edasalonexent</td>
<td>Edasalonexent</td>
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Open-label extension

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
Phase 3 PolarisDMD Planned Clinical Trial Sites

- Global trial with U.S. sites anticipated in:
  - Portland, OR
  - Salt Lake City, UT
  - Sacramento, CA
  - San Francisco, CA
  - Los Angeles, CA
  - Minneapolis, MN
  - Ann Arbor, MI
  - Cleveland, OH
  - Philadelphia, PA
  - Baltimore, MD
  - Richmond, VA
  - Norfolk, VA
  - Nashville, TN
  - Atlanta, GA
  - Fort Worth, TX
  - Little Rock, AR
  - Orlando, FL
  - Boston, MA

- Sites also anticipated in Canada, Europe, Israel and Australia

- Enrollment of approximately 125 boys, 2:1 randomization, accounting for dropouts

- Sites are opening now and in coming weeks
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 and Twitter