

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, DC 20549**

**FORM 8-K**

**CURRENT REPORT  
Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

Date of report (Date of earliest event reported): **October 16, 2019**

**Catabasis Pharmaceuticals, Inc.**  
(Exact Name of Registrant as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-37467**  
(Commission  
File Number)

**26-3687168**  
(IRS Employer  
Identification No.)

**One Kendall Square  
Bldg. 1400E, Suite B14202  
Cambridge, Massachusetts**  
(Address of Principal Executive Offices)

**02139**  
(Zip Code)

Registrant's telephone number, including area code: **(617) 349-1971**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.001 par value per share	CATB	The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 7.01. Regulation FD Disclosure**

On October 17, 2019, Catabasis Pharmaceuticals, Inc. (the “Company”) is making publicly available on its website an updated corporate slide presentation. The updated slide presentation is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

The information in this Current Report on Form 8-K, including Exhibit 99.1, shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 (the “Exchange Act”) or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act, except as expressly set forth by specific reference in such a filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

<b>Exhibit No.</b>	<b>Description</b>
99.1	<a href="#">Corporate slide presentation</a>


**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

CATABASIS PHARMACEUTICALS, INC.

Date: October 16, 2019

By: /s/ Jill C. Milne  
Jill C. Milne  
President and Chief Executive Officer

A photograph of a person sitting on a wooden bench, with their hand being held by another person. The person on the bench is wearing dark pants and a watch. The background is a blurred outdoor setting.

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

## **Catabasis Pharmaceuticals**

October 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 June 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- $\kappa$ B 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- $\kappa$ B 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- $\kappa$ B 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- $\kappa$ B 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- $\kappa$ B, 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

## ▶ Commercial Approach

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

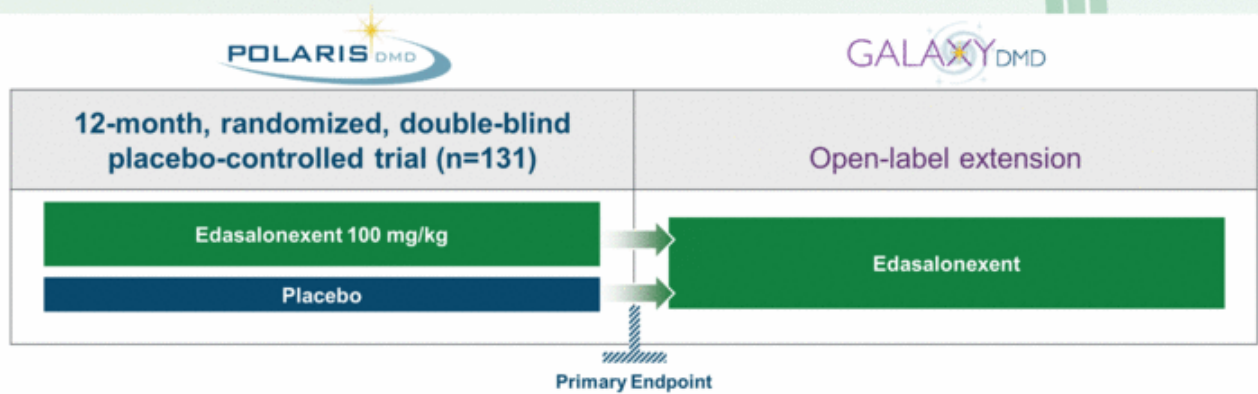


**Developing  
a potential  
foundational  
therapy  
in DMD**

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  $\geq 6$  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 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

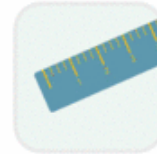


4-Stair Climb



10-Meter Walk/Run

## Additional Outcomes



Growth



Cardiac Health



Bone Health



Patient Reported Outcomes

# PolarisDMD Was Designed Based on Promising MoveDMD Trial Results

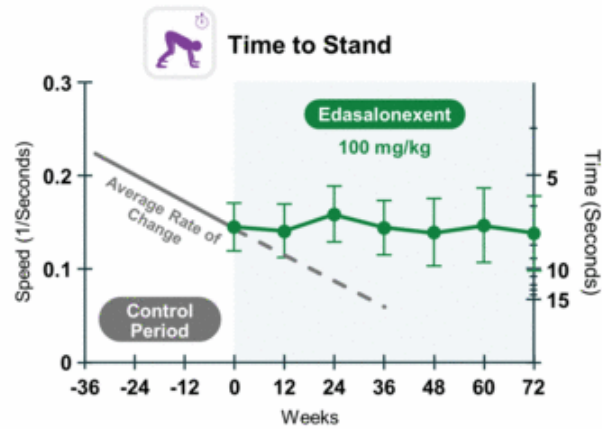
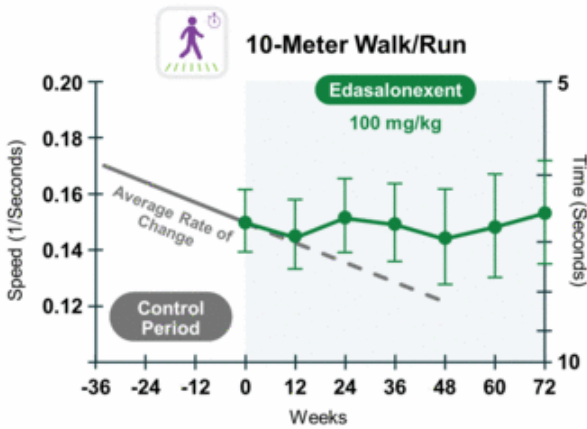
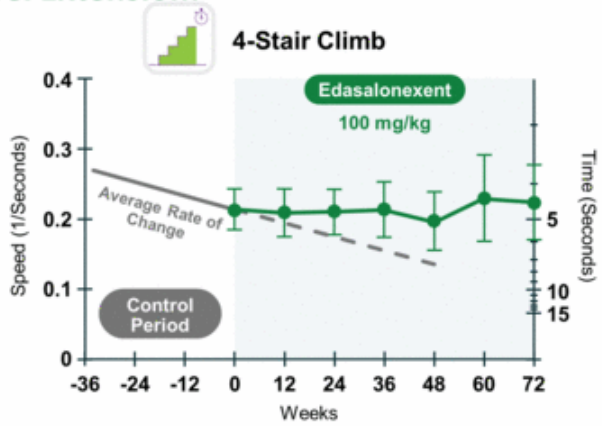
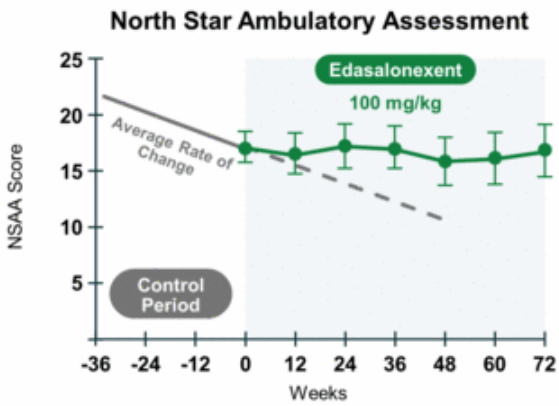
## In Phase 2 MoveDMD Trial and Open-Label Extension:



NF-κB Target Engagement	Biomarkers	Muscle MRI	Functional
<ul style="list-style-type: none"> <li>✓ Inhibited NF-κB targeted gene set in peripheral blood</li> </ul>	<ul style="list-style-type: none"> <li>✓ Decreased CK and other muscle enzymes</li> <li>✓ Decreased CRP, biomarker of inflammation</li> </ul>	<ul style="list-style-type: none"> <li>✓ Improved rate of change in MRI T2 and MRS muscle fat compared to off-treatment control</li> </ul>	<ul style="list-style-type: none"> <li>✓ Preserved NSAA and Timed Function Tests</li> </ul>

# Edasalonexent Demonstrated Clinically Meaningful Slowing of Disease Progression

## In Phase 2 MoveDMD Trial and Open-Label Extension:



**catobasis** 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.

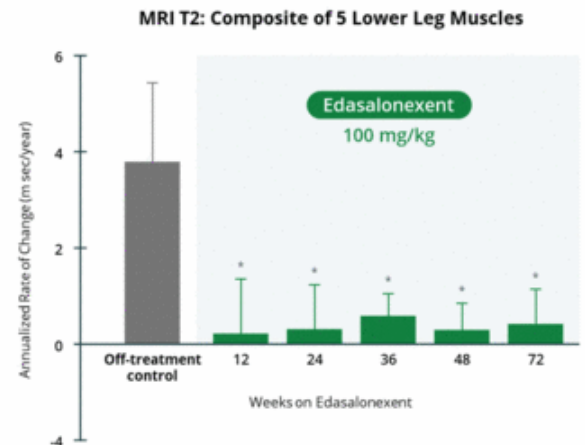
## In Phase 2 MoveDMD Trial and Open-Label Extension:

### ▶ Significantly improved rate of change of MRI T2 compared to off-treatment control period

- MRI T2 increases over time in DMD as inflammation and fat content of muscle increases and inversely correlates with functional abilities

### ▶ Benefits on development measures including growth and cardiac

- Height and weight increases were similar to unaffected boys
- Mean resting heart rate significantly decreased, approaching age-normative heart rate of ~92 beats per minute
  - Cardiomyopathy is the leading cause of death in DMD
  - Elevated resting heart rate is the initial manifestation of cardiac disease in DMD
- These differentiating measures are being further explored in Phase 3



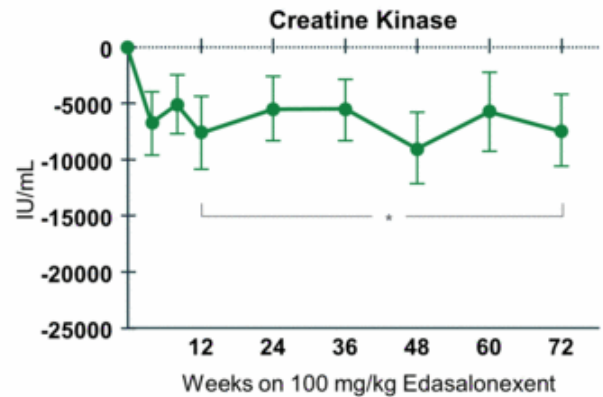
Means + SEM; mixed model comparison with off-treatment period

\* 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

# Edasalonexent Was Well-Tolerated with No Safety Signals

## In Phase 2 MoveDMD Trial and Open-Label Extension:

- ▶ **55+ patient years of exposure**
- ▶ **Well-tolerated, with majority of adverse events mild in nature**
  - Most common related adverse event was diarrhea, generally mild and transient and did not require discontinuation
  - No serious adverse events on treatment (one on placebo)
  - No adverse trends in chemistry, hematology or measures of adrenal function (cortisol and ACTH)
- ▶ **Muscle enzymes significantly decreased on edasalonexent, including CK, supporting a positive impact on muscle health**



# DMD Patient Segmentation and Typical Progression Is Well Established and Understood



2 - 5 years



4 - 7 years



8 - 12 years



12 + years



## Diagnosed by Age 5

- ▶ Affected boys show clinical signs and symptoms

## Early Ambulatory

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

## Late Ambulatory

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

## 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

## 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



# Clear Market Need in DMD with Limited Treatment Options

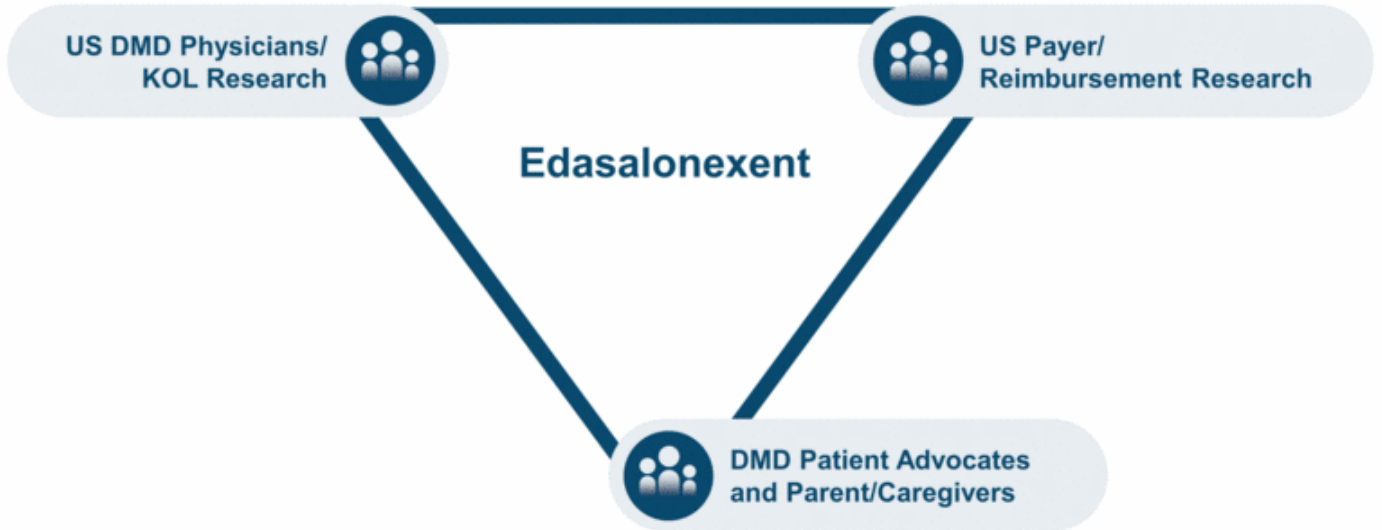


- ▶ **Currently, there is no cure for DMD**
- ▶ **Today, the majority of patients are treated with corticosteroids**
  - Despite broad market utilization, steroids have long-term negative consequences
- ▶ **Only a small portion of the population can be treated with eteplirsen (US) or ataluren (EU)**

## Current Landscape of medical management

Steroids		Mutation Targeted
Deflazacort and Prednisone		Eteplirsen (US) and Ataluren (EU)
<b>Known Benefits:</b> <ul style="list-style-type: none"><li>▶ Delayed loss of muscle function</li></ul>	<b>Known Side Effects:</b> <ul style="list-style-type: none"><li>▶ Osteoporosis with fractures</li><li>▶ Metabolic effects</li><li>▶ Weight gain, obesity</li><li>▶ Growth retardation</li><li>▶ Delayed puberty</li><li>▶ Cataracts</li><li>▶ Muscle atrophy</li><li>▶ Behavioral issues</li><li>▶ Cushingoid appearance</li></ul>	<ul style="list-style-type: none"><li>▶ Safe and tolerable</li><li>▶ Limited labels; no outcomes data</li><li>▶ Limited suitable patient populations (13% for each targeted population)</li></ul>

# 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
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# 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- $\kappa$ B 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

# Catabasis Has Developed Strong Relationships with Global DMD Patient Advocacy Organizations



**US**

- Parent Project Muscular Dystrophy
- Cure Duchenne
- MDA
- Jell Foundation
- Charley's Fund
- CURE RARE DISEASE

**Canada**

- FONDATION LA FORCE
- Stand for Duchenne Canada
- Jesse's JOURNEY
- MUSCULAR DYSTROPHY CANADA / DYSTROPHIE MUSCULAIRE CANADA

**UK**

- DUCHENNE UK
- Muscular Dystrophy UK
- ACTION DUCHENNE

**Sweden**

- smdf

**Europe**

- World Duchenne Organization

**Israel**

- אגודת דוכן (Association of Duchenne)

**Australia**

- SAVE OUR SONS

- ▶ **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



# HERCULES.

**DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS**

19th December 2018

**Duchenne UK are delighted to announce that eight industry partners are supporting Phase 2 of Project HERCULES in 2019**

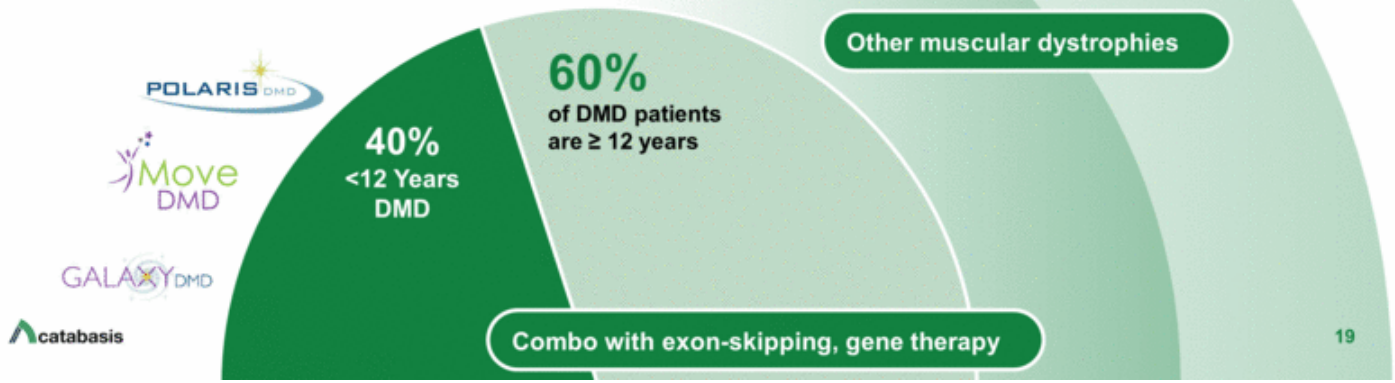
**Catabasis Pharmaceuticals, Pfizer, PTC Therapeutics International Ltd, Roche, Sarepta Therapeutics, Solid Biosciences LLC, Santhera Pharmaceuticals Holding AG and Wave Life Sciences USA, Inc** will join Duchenne UK and other partners in this ground breaking global project.

Project HERCULES will develop a disease level economic model and other tools to better demonstrate the real value of new treatments for DMD for Health Technology Assessments and reimbursement decisions.



## 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- $\kappa$ B 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