



## Catabasis Pharmaceuticals Reports Fourth Quarter and Full Year 2016 Financial Results and Recent Corporate Highlights

March 16, 2017

-- Part C of MoveDMD<sup>®</sup> Trial of Edasalonexent in Duchenne Muscular Dystrophy Ongoing --

-- Continued Advancement of Rare Disease Pipeline with CAT-5571, a Potential Treatment for Cystic Fibrosis, and CAT-4001, a Potential Treatment for Neurodegenerative Diseases --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 16, 2017-- [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced financial results for the fourth quarter and full year ended December 31, 2016, and corporate highlights.

"The recently reported safety, tolerability and plasma exposure data for edasalonexent in patients with Duchenne muscular dystrophy from Part B of the MoveDMD trial are reassuring, and Part C of the trial is continuing on track," said Jill C. Milne, Chief Executive Officer of Catabasis. "Although we did not achieve the MRI T2 composite endpoint at 12 weeks in Part B, we are encouraged by the improvements in the function-associated exploratory endpoints, including the timed function tests, North Star Ambulatory Assessment, PODCI and muscle strength, and are hopeful that extended exposure will provide evidence of functional benefits."

Dr. Milne continued, "Looking forward in 2017, Part C of the MoveDMD trial will allow us to collect data for up to 48 weeks of treatment and provide important information about dose and activity with extended duration, as well as endpoints for possible future clinical trials of edasalonexent. Following additional data analysis from Part C, we will determine the next steps for edasalonexent in DMD. Catabasis is also moving the development of our rare disease pipeline forward, with advances in CAT-5571 for cystic fibrosis and CAT-4001 for neurodegenerative diseases including Friedreich's ataxia and ALS."

### Recent and Upcoming Corporate Highlights

#### Edasalonexent (CAT-1004) and the MoveDMD Trial

- Announced top-line results from Part B of the MoveDMD trial of edasalonexent in January. The primary efficacy endpoint of MRI T2 was not met. The edasalonexent 100 mg/kg/day treatment group consistently showed numerical improvement versus placebo across multiple timed function tests and the North Star Ambulatory Assessment, although as expected the changes were not statistically significant. The 67 mg/kg/day treatment group had mixed results compared with both the 100 mg/kg/day treatment group and placebo, which in each case were not statistically significant.
- Announced two publications about edasalonexent: Phase 1 clinical data in the Journal of Clinical Pharmacology and preclinical research in JCI Insight.
- Ongoing open-label extension (Part C) of the MoveDMD trial in which patients continue on edasalonexent for 36 weeks following completion of Part B. Catabasis intends to report the results from Part C in 2017, with an interim update in Q2.

#### New Edasalonexent Updates

- Upcoming presentation this month at the 2017 Muscular Dystrophy Association Scientific Conference, including: Part B data for the pediatric outcomes data collection instrument (PODCI) and muscle strength function-associated exploratory endpoints, which showed numerical improvement versus placebo for both edasalonexent treatment groups at 12 weeks as well as a review of previously released results from the MoveDMD trial.
- In the Catabasis and Sarepta joint research collaboration, established to explore a combination drug treatment approach for DMD, increased dystrophin protein expression was seen with an exon-skip modality in combination with edasalonexent in the designated mouse model of DMD. The companies believe that these results warrant further research.

#### Additional Rare Disease Programs

- Announced the publication of research on CAT-5571, a novel activator of autophagy and potential oral treatment for cystic fibrosis (CF), in the Journal of Medicinal Chemistry.
- Presented preclinical data for CAT-5571 at the North American Cystic Fibrosis Conference. CAT-5571, an activator of

autophagy, in combination with lumacaftor/ivacaftor, enhanced cell-surface trafficking and function of CF transmembrane conductance regulator (CFTR) in bronchial epithelial cells from CF patients with the F508del mutation. Catabasis also presented that CAT-5571 enhanced the clearance of *Pseudomonas aeruginosa* infection in preclinical models of CF, irrespective of CFTR mutation status.

- Ongoing preclinical activities exploring the potential of CAT-4001 in diseases such as amyotrophic lateral sclerosis (ALS) and Friedreich's ataxia.

## Corporate

- Catabasis hosted its first Investor Day in New York on November 17, 2016, focused on its strategy in rare diseases and its pipeline, including edasalonexent and other programs.
- Catabasis had three recent executive team promotions: Ted Hibben to Chief Business Officer, Andrew Nichols, Ph.D., to Chief Scientific Officer, and Angelika Fretzen, Ph.D., to Senior Vice President of Product Development.

## Fourth Quarter and Full Year 2016 Financial Results

**Cash Position:** At December 31, 2016, Catabasis had cash, cash equivalents and marketable securities of \$38.5 million, compared to \$47.3 million as of September 30, 2016 and \$62.8 million as of December 31, 2015. Catabasis expects that its cash, cash equivalents and marketable securities at December 31, 2016 will fund operating expenses, debt service and capital expenditure requirements based on its current operating plan through March 31, 2018, assuming no unscheduled repayment of indebtedness prior to such date. Net cash used in operating activities for the three months ended December 31, 2016 was \$8.0 million, compared to \$8.7 million for the three months ended December 31, 2015. Net cash used in operating activities for the full year 2016 was \$32.9 million, net of financings, compared to \$29.8 million for the full year 2015.

**R&D Expenses:** Research and development expenses were \$6.3 million for the three months ended December 31, 2016, compared to \$6.7 million for the three months ended December 31, 2015 and \$25.5 million for the full year 2016, compared to \$23.0 million for the full year 2015. The increase in research and development expenses for the full year 2016 relative to the full year 2015 was primarily attributable to increased direct program costs related to the edasalonexent MoveDMD trial. The decrease in research and development expenses for the fourth quarter of 2016 relative to the fourth quarter of 2015 was primarily attributable to having one program in active clinical trials in Q4 2016 compared to two in Q4 2015.

**G&A Expenses:** General and administrative expenses were \$2.4 million for the three months ended December 31, 2016, compared to \$2.7 million for the three months ended December 31, 2015 and \$10.1 million for the full year 2016, compared to \$8.6 million for the full year 2015. The increase in general and administrative expenses for the full year 2016 relative to the prior year was primarily attributable to increased employee compensation costs.

**Operating Loss:** Loss from operations was \$8.7 million for the three months ended December 31, 2016, compared to \$9.3 million for the three months ended December 31, 2015, and \$35.6 million for the full year 2016, compared to \$31.7 million for the full year 2015.

**Net Loss:** Net loss was \$8.8 million, or \$0.47 per share, for the three months ended December 31, 2016, compared to a net loss of \$9.6 million for the three months ended December 31, 2015. Net loss for the full year 2016 was \$36.1 million, or \$2.22 per share, compared to \$32.6 million for the full year 2015.

## Conference Call and Webcast

Catabasis will host a conference call and webcast at 4:30pm ET today to provide an update on corporate developments and to discuss fourth quarter and full year 2016 financial results.

Participant Toll-Free Dial-In Number: (877) 388-2733  
Participant International Dial-In Number: (541) 797-2984  
Pass Code: 74976422

Please specify to the operator that you would like to join the "Catabasis Fourth Quarter and Full Year 2016 Results Call."

Interested parties may access a live audio webcast of the conference call via the investor section of the Catabasis website, [www.catabasis.com](http://www.catabasis.com). Please connect to the Catabasis website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. The webcast will be archived for 90 days.

## About Edasalonexent (CAT-1004)

Edasalonexent (CAT-1004) is an investigational oral small molecule that is being developed as a potential disease-modifying therapy for all patients affected by Duchenne muscular dystrophy (DMD or Duchenne), regardless of their underlying mutation. Edasalonexent inhibits NF-κB, a protein that is activated in Duchenne and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. In animal models of DMD, edasalonexent produced beneficial effects in skeletal, including diaphragm, and cardiac muscle and improved function. The FDA has granted orphan drug, fast track and rare pediatric disease designations and the European Commission has granted orphan medicinal product designation to edasalonexent for the treatment of DMD. We have previously reported safety, tolerability and reduction in NF-κB activity in Phase 1 trials in adults. We are currently conducting the MoveDMD<sup>®</sup> trial, a three-part clinical trial investigating the safety and efficacy of edasalonexent in boys ages 4 – 7 affected with DMD (any confirmed mutation). Part A of the trial evaluated the safety, tolerability and pharmacokinetics of, and NF-κB target engagement with, edasalonexent in 17 boys with DMD. Part B of the trial was a double-blind, placebo-controlled evaluation of the safety and efficacy of edasalonexent over a 12-week period in 31 boys. The primary efficacy endpoint for Part B was average change from baseline to week 12 in MRI T2 measures in boys given edasalonexent compared to placebo. Additional efficacy endpoints included age-appropriate timed function tests (10-meter walk/run, 4-stair climb and time to stand), North Star Ambulatory Assessment (NSAA), the pediatric outcomes data collection instrument (PODCI) and muscle strength. Part C is an open-label extension with edasalonexent for 36 weeks beyond Part B and will evaluate longer term safety and efficacy with the same clinical end points as Part B. From the MoveDMD trial, we have reported that edasalonexent was well tolerated with no safety signals. We reported top-line data for Part B indicating that the primary efficacy endpoint was not met. The edasalonexent 100 mg/kg/day treatment group consistently showed numerical improvement versus placebo across multiple timed function tests and the North Star Ambulatory Assessment, although

as expected the changes were not statistically significant. The 67 mg/kg/day treatment group had mixed results compared with both the 100 mg/kg/day treatment group and placebo, which in each case were not statistically significant. Part C of the MoveDMD trial is ongoing.

#### About CAT-5571

Catabasis is developing CAT-5571 as a potential oral treatment for cystic fibrosis (CF) with potential effects on both the cystic fibrosis transmembrane conductance regulator (CFTR) and on the clearance of *Pseudomonas aeruginosa*. CAT-5571 is a small molecule that activates autophagy, a process that maintains cellular homeostasis and host defense mechanisms, and is known to be impaired in CF. Catabasis has shown in preclinical studies that CAT-5571, in combination with lumacaftor/ivacaftor, enhances cell-surface trafficking and function of CFTR with the F508del mutation. Catabasis has also shown that CAT-5571 enhances the clearance of *P. aeruginosa* infection in preclinical models of CF, irrespective of CFTR mutation status.

#### About CAT-4001

Catabasis is developing CAT-4001 as a potential treatment for neurodegenerative diseases such as Friedreich's ataxia (FA) and amyotrophic lateral sclerosis (ALS). CAT-4001 is a small molecule that activates Nrf2 and inhibits NF-κB, two pathways that have been implicated in FA and ALS. Catabasis has shown that CAT-4001 modulates the Nrf2 and NF-κB pathways in both cellular assays and animal models.

#### About Catabasis

At Catabasis Pharmaceuticals, our mission is to bring hope and life-changing therapies to patients and their families. Our SMART (Safely Metabolized And Rationally Targeted) linker drug discovery platform enables us to engineer molecules that simultaneously modulate multiple targets in a disease. We are applying our SMART linker platform to build an internal pipeline of product candidates for rare diseases and plan to pursue partnerships to develop additional product candidates. For more information on the Company's drug discovery platform and pipeline of drug candidates, please visit [www.catabasis.com](http://www.catabasis.com).

#### Forward Looking Statements

Any statements in this press release about future expectations, plans and prospects for the Company, including statements about future clinical trial plans and other statements containing the words "believes," "anticipates," "plans," "expects," "may" and similar expressions, constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development of the Company's 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; 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, 2016, 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 press release represent the Company's views as of the date of this press release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this release.

#### Catabasis Pharmaceuticals, Inc.

#### Condensed Consolidated Statements of Operations

(In thousands, except share and per share data)

(Unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2016	2015	2016	2015
Operating expenses:				
Research and development	\$ 6,260	\$ 6,670	\$ 25,450	\$ 23,030
General and administrative	2,413	2,663	10,108	8,629
Total operating expenses	8,673	9,333	35,558	31,659
Loss from operations	(8,673)	(9,333)	(35,558)	(31,659)
Other (expense) income:				
Interest expense	(175)	(269)	(837)	(978)
Interest and investment income	59	-	242	-

Other income, net	11	(4	)	93	7
Total other expense, net	(105	)	(273	)	(502) (971)
Net loss	\$ (8,778	)	\$ (9,606	)	\$ (36,060) \$ (32,630)
Net loss per share - basic and diluted	\$ (0.47	)	\$ (0.63	)	\$ (2.22 ) \$ (4.06 )
Weighted-average common shares outstanding used in net loss per share - basic and diluted	18,699,480		15,298,810		16,230,190 8,041,948

## Catabasis Pharmaceuticals, Inc.

### Condensed Consolidated Balance Sheets

(In thousands)

(Unaudited)

	As of December 31,	
	2016	2015
<b>Assets</b>		
Cash and cash equivalents	\$ 23,596	\$ 62,780
Available-for-sale securities	14,931	-
Total assets	40,209	64,169
<b>Liabilities and stockholders' equity</b>		
Current portion of notes payable, net of discount	3,243	3,173
Notes payable, net of current portion and discount	2,479	5,720
Total liabilities	11,123	13,676
Total stockholders' equity	\$ 29,086	\$ 50,493

## Catabasis Pharmaceuticals, Inc.

### Condensed Consolidated Statements of Cash Flows

(In thousands)

(Unaudited)

	Year Ended December 31,	
	2016	2015
Net cash used in operating activities	\$ (32,858 )	\$ (29,793 )
Net cash used in investing activities	(15,490 )	(421 )
Net cash provided by financing activities	9,164	78,326
Net (decrease) increase in cash and cash equivalents	\$ (39,184 )	\$ 48,112

View source version on businesswire.com: <http://www.businesswire.com/news/home/20170316006156/en/>

Source: Catabasis Pharmaceuticals, Inc.

Corporate and Media Contact  
Catabasis Pharmaceuticals, Inc.  
Andrea Matthews, 617-349-1971  
[amatthews@catabasis.com](mailto:amatthews@catabasis.com)