



## **Catabasis Pharmaceuticals Reports First Quarter 2017 Financial Results and Reviews Business Progress**

*-- Improvements Seen Across Functional Assessments in Boys Treated with Edasalonexent for 12 Weeks in Part B of the MoveDMD® Trial in Duchenne Muscular Dystrophy --*

*-- MoveDMD Trial Progressing, Part C Interim Results Expected in Q3 2017 --*

*-- Rare Disease Pipeline Programs Advancing in Cystic Fibrosis and Neurodegenerative Diseases --*

**CAMBRIDGE, Mass., May 11, 2017** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported financial results for the first quarter ended March 31, 2017, and reviewed recent business progress.

“We made important progress across our portfolio in the first quarter, including the presentation of encouraging results from our edasalonexent MoveDMD trial and the advancement of our rare disease pipeline,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “Our recently completed prespecified analysis of functional assessments in Part B of the MoveDMD trial strengthens our confidence in the potential of edasalonexent as a novel treatment for boys with Duchenne muscular dystrophy.”

Dr. Milne continued, “We see consistent numerical improvements across prespecified functional analyses of boys treated with edasalonexent. We expect to report interim results from Part C of the MoveDMD trial in the third quarter, and we look forward to gaining important information on the persistence of treatment signals with edasalonexent as well as determining functional endpoints for possible future clinical trials. The MoveDMD trial is progressing as planned, and we are focused on the next steps for the development of edasalonexent while continuing to advance our pipeline programs in cystic fibrosis and neurodegenerative diseases.”

### **Recent and Upcoming Corporate Highlights**

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

- Continued execution of open-label extension (Part C) of the MoveDMD trial in which patients are treated with edasalonexent following completion of Part B. The institutional review boards have approved moving all boys in Part C to the higher 100 mg/kg/day dose group of edasalonexent and extending Part C to 60 weeks. Catabasis intends to report results from Part C in 2017, with an interim update in Q3 after all boys participating have completed 24 weeks of dosing with edasalonexent.
- In April, presented data from the MoveDMD trial of edasalonexent at the American Academy of Neurology 69<sup>th</sup> Annual Meeting. The prespecified analysis of Part B data showed numerical improvement in rates of change across five functional assessments for patients on 12 weeks of edasalonexent compared to off-treatment prior to Part B dosing.

These results are in addition to and consistent with numerical improvements in the same functional assessments with edasalonexent compared to placebo as reported in the top-line results from Part B in January. These functional assessments are meaningful to boys affected by Duchenne and are known to correlate with loss of milestones and disease progression. The MoveDMD trial was not powered for functional assessments and these analyses were generally not statistically significant.

- In March, reported results from the Catabasis and Sarepta joint research collaboration showing increased dystrophin expression in the *mdx* mouse with edasalonexent in combination with an exon-skip modality. The companies believe that these results warrant further research.
- In January, the primary endpoint for Part B of MRI T2, an exploratory early biomarker, was not met. However, we continue to see numerical improvements across the functional assessments, as described above, which have precedence as endpoints in pivotal trials in DMD.

### **Additional Rare Disease Programs**

- In January, published research on CAT-5571, a novel activator of autophagy and potential oral treatment for cystic fibrosis (CF), in the Journal of Medicinal Chemistry; upcoming presentation on CAT-5571 planned at the European Cystic Fibrosis Conference in June.
- Continued ongoing preclinical activities exploring the potential of CAT-4001 in diseases such as amyotrophic lateral sclerosis (ALS) and Friedreich's ataxia.

### **First Quarter 2017 Financial Results**

**Cash Position:** As of March 31, 2017, Catabasis had cash and cash equivalents of \$31.8 million, compared to \$38.5 million in cash, cash equivalents and available-for-sale securities as of December 31, 2016. Catabasis expects that its current cash and cash equivalents as of April 30, 2017, will fund operating expenses, debt service and capital expenditure requirements based on its current operating plan for at least 12 months from today. Net cash used in operating activities for the three months ended March 31, 2017 was \$8.1 million, compared to \$9.1 million for the three months ended March 31, 2016.

**R&D Expenses:** Research and development expenses were \$5.4 million for the three months ended March 31, 2017, compared to \$6.4 million for the three months ended March 31, 2016. The decrease in research and development expenses was primarily attributable to the completion of certain clinical activities.

**G&A Expenses:** General and administrative expenses were \$2.4 million for the three months ended March 31, 2017, compared to \$2.8 million for the three months ended March 31, 2016. The decrease in general and administrative expenses was primarily attributable to decreased G&A headcount.

**Operating Loss:** Loss from operations was \$7.8 million for the three months ended March 31, 2017, compared to \$9.2 million for the three months ended March 31, 2016.

**Net Loss:** Net loss was \$7.9 million, or \$0.41 per share, for the three months ended March 31, 2017, compared to a net loss of \$9.4 million for the three months ended March 31, 2016.

### **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 first quarter 2017 financial results.

Participant Toll-Free Dial-In Number: (877) 388-2733  
Participant International Dial-In Number: (541) 797-2984  
Pass Code: 99023963

Please specify to the operator that you would like to join the “Catabasis First Quarter 2017 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 DMD, regardless of their underlying mutation. Edasalonexent inhibits NF- $\kappa$ B, a protein that is activated in DMD and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. We are currently conducting the MoveDMD trial, a three-part clinical trial investigating the safety and efficacy of edasalonexent in boys ages 4 – 7 affected with DMD (any confirmed mutation). The third part of the trial, an open-label extension with edasalonexent, is ongoing. 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. For a summary of clinical results reported to-date, please visit [www.catabasis.com](http://www.catabasis.com).

### **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.

### **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- $\kappa$ B, two pathways that have been implicated in FA and ALS. Catabasis has shown that CAT-4001 modulates the Nrf2 and NF- $\kappa$ B 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 Quarterly Report on Form 10-Q for the period ended March 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 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.

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**Catabasis Pharmaceuticals, Inc.**  
**Condensed Consolidated Statements of Operations**  
(In thousands, except share and per share data)  
*(Unaudited)*

	<b>Three Months Ended March 31,</b>	
	<b>2017</b>	<b>2016</b>
<b>Operating expenses:</b>		
Research and development	\$ 5,398	\$ 6,436
General and administrative	2,363	2,770
Total operating expenses	<u>7,761</u>	<u>9,206</u>
Loss from operations	(7,761)	(9,206)
<b>Other (expense) income:</b>		
Interest expense	(149)	(243)
Interest and investment income	39	53
Other expense, net	(5)	(22)
Total other expense, net	<u>(115)</u>	<u>(212)</u>
Net loss	<u>\$ (7,876)</u>	<u>\$ (9,418)</u>
Net loss per share - basic and diluted	<u>\$ (0.41)</u>	<u>\$ (0.61)</u>
Weighted-average common shares outstanding used in net loss per share - basic and diluted	<u>19,093,273</u>	<u>15,335,516</u>

**Catabasis Pharmaceuticals, Inc.**  
**Condensed Consolidated Balance Sheets**  
(In thousands)  
*(Unaudited)*

	<b>March 31,</b>	<b>December 31,</b>
	<b>2017</b>	<b>2016</b>
<b>Assets</b>		
Cash and cash equivalents	\$ 31,795	\$ 23,596
Available-for-sale securities	-	14,931
Total assets	33,422	40,209
<b>Liabilities and stockholders' equity</b>		
Current portion of notes payable, net of discount	3,260	3,243
Notes payable, net of current portion and discount	1,657	2,479
Total liabilities	9,467	11,123
Total stockholders' equity	\$ 23,955	\$ 29,086

**Catabasis Pharmaceuticals, Inc.**  
**Condensed Consolidated Statements of Cash Flows**  
(In thousands)  
*(Unaudited)*

	<b>Three Months Ended March 31,</b>	
	<b>2017</b>	<b>2016</b>
Net cash used in operating activities	\$ (8,105)	\$ (9,147)
Net cash provided by (used in) investing activities	14,901	(29,069)
Net cash provided by (used in) financing activities	1,403	(746)
Net increase (decrease) in cash and cash equivalents	\$ 8,199	\$ (38,962)