



## **Catabasis Pharmaceuticals Reports Fourth Quarter and Full Year 2017 Financial Results and Reviews Business Progress**

*-- Preparations Underway for Phase 3 Trial Following Positive Data Showing Substantially Slowed Duchenne Muscular Dystrophy Disease Progression Through More than One Year of Edasalonexent Treatment --*

*-- Plan to Initiate Phase 1 Trial for CAT-5571, a Potential Treatment for Cystic Fibrosis, Based on Preclinical Data Showing Potential of CAT-5571 to Clear Pathogens --*

**CAMBRIDGE, Mass., March 15, 2018** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported financial results for the fourth quarter and full year ended December 31, 2017, and reviewed recent business progress.

“We achieved several important clinical milestones in 2017 as we work toward our vision of improving the lives of those affected by rare diseases,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “Edasalonexent demonstrated consistent and sustained disease-modifying effects in boys with Duchenne muscular dystrophy through more than a year of treatment compared to control without evidence of adverse effects associated with current standard of care. We also made progress in the evaluation of CAT-5571, a potential oral treatment for cystic fibrosis. In 2018, we are focused on advancing our edasalonexent program into Phase 3 and bringing our cystic fibrosis program into the clinic.”

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

#### **Edasalonexent (CAT-1004) for the Treatment of Duchenne Muscular Dystrophy (DMD)**

Consistent and sustained disease-modifying effects of edasalonexent following 48 and 60 weeks of treatment in boys with DMD were reported at the XVI International Conference on Duchenne and Becker Muscular Dystrophy in February 2018.

- Preservation of muscle function and substantially slowed DMD disease progression compared to the pre-specified control period was observed through more than a year of edasalonexent treatment. Consistent improvements in all assessments of muscle function were observed following 48 and 60 weeks of oral 100 mg/kg/day edasalonexent treatment compared to the rates of change in the control period for boys prior to receiving edasalonexent treatment.
- Statistically significant changes from baseline in multiple non-effort based biomarkers of muscle health were seen.
- Edasalonexent continued to be well tolerated with no safety signals observed.

Additional data on edasalonexent were presented this week at the 2018 Muscular Dystrophy Association Clinical Conference.

- Height and weight growth following 48 and 60 weeks of edasalonexent treatment was age-appropriate and on track with standard growth curves for unaffected boys in the same age range. Body mass index (BMI) trended towards a decrease. This profile is favorably differentiated from the typical profile associated with the corticosteroid standard of care in DMD which includes weight gain and curtailed growth.
- Heart rate data from boys treated with edasalonexent decreased toward age-normative values through 48 weeks of treatment. Cardiac failure is a leading cause of mortality in DMD. In the 4-7 year old age range, boys typically have resting tachycardia, a heart rate that exceeds the normal resting rate, which is the first cardiac manifestation in DMD.

Also presented at the 2018 Muscular Dystrophy Association Clinical Conference were new data from the ImagingDMD natural history study, a collaboration led by the University of Florida and independent of the MoveDMD trial.

- The ImagingDMD study annually assessed timed function tests, and a subset analysis was presented of 28 boys initially aged 5 to 8.5 years old at times when they were not taking corticosteroids. The timed function tests performed in the study were the same as those assessed in the MoveDMD trial, which was conducted at ImagingDMD sites.
- The observations in the ImagingDMD natural history study were generally consistent with the absolute functional abilities as well as declines in abilities experienced by boys in the off-treatment control period of the Catabasis MoveDMD trial. These data provide important corroboration that the MoveDMD off-treatment control period observations are characteristic of the expected natural history and provide added confidence in the slowing of disease progression treatment effects observed with edasalonexent.

Catabasis plans to initiate a global Phase 3 trial with edasalonexent in patients with DMD regardless of mutation type in the first half of 2018 with top-line results expected in 2020, dependent on raising capital.

### **CAT-5571 for the Treatment of Cystic Fibrosis (CF)**

Data demonstrating CAT-5571 improved cellular clearance of bacteria in preclinical models of CF were presented at the North American Cystic Fibrosis Conference in November 2017. CAT-5571 enhanced the clearance of the opportunistic and often fatal pathogen *Burkholderia cenocepacia*.

Catabasis expects to initiate a Phase 1 trial for CAT-5571 in the second half of 2018 and report top-line results in 2019, based on our current operating plan. This is supported by preclinical data showing CAT-5571 enhances the clearance of multiple types of pathogens.

### **Fourth Quarter and Full Year 2017 Financial Results**

**Cash Position:** As of December 31, 2017, Catabasis had cash and cash equivalents of \$16.4 million, compared to \$21.7 million as of September 30, 2017 and \$38.5 million in cash, cash

equivalents and available-for-sale securities as of December 31, 2016. Following December 31, 2017, Catabasis raised an additional \$8.3 million in net proceeds under an at-the-market offering program. Catabasis' current operating plan provides for cash to fund operations through September 2018. To advance edasalonexent in the Phase 3 trial and CAT-5571 in the Phase 1 trial, Catabasis expects to seek additional funds through equity or debt financings and/or through partnering or licensing transactions. Net cash used in operating activities for the three months ended December 31, 2017 was \$5.6 million, compared to \$8.0 million for the three months ended December 31, 2016. Net cash used in operating activities for the full year 2017 was \$26.8 million, compared to \$32.9 million for the full year 2016. Recognized revenue for the three months ended December 31, 2017 was \$0.3 million and \$0.5 million for the full year 2017 from an option agreement with an unaffiliated party.

**R&D Expenses:** Research and development expenses were \$4.0 million for the three months ended December 31, 2017, compared to \$6.3 million for the three months ended December 31, 2016 and \$18.7 million for the full year 2017, compared to \$25.5 million for the full year 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 \$1.7 million for the three months ended December 31, 2017, compared to \$2.4 million for the three months ended December 31, 2016 and \$8.9 million for the full year 2017, compared to \$10.1 million for the full year 2016.

**Operating Loss:** Loss from operations was \$5.5 million for the three months ended December 31, 2017, compared to \$8.7 million for the three months ended December 31, 2016, and \$27.1 million for the full year 2017, compared to \$35.6 million for the full year 2016.

**Net Loss:** Net loss was \$5.5 million, or \$0.24 per share, for the three months ended December 31, 2017, compared to a net loss of \$8.8 million, or \$0.47 per share, for the three months ended December 31, 2016. Net loss for the full year 2017 was \$27.4 million, or \$1.26 per share, compared to \$36.1 million for the full year 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 fourth quarter and full year 2017 financial results.

Participant Toll-Free Dial-In Number: (877) 388-2733  
Participant International Dial-In Number: (541) 797-2984  
Pass Code: 7693708

Please specify to the operator that you would like to join the "Catabasis Fourth Quarter and Full Year 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. Edasalonexent continues to be dosed in the open-label extension of the MoveDMD Phase 2 clinical trial and Catabasis plans to initiate a single global Phase 3 trial to evaluate the efficacy and safety of edasalonexent for registration purposes in the first half of 2018, dependent on raising capital. 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**

CAT-5571 is an investigational oral small molecule designed to restore host defense by activating autophagy that is being developed for the treatment of cystic fibrosis (CF). Autophagy is a mechanism for recycling cellular components and digesting pathogens, which is depressed in CF. People with CF suffer from persistent lung infections with opportunistic pathogens such as *Pseudomonas aeruginosa* and *Burkholderia cenocepacia*, causing chronic infections that are difficult to eradicate and lead to respiratory failure. CAT-5571 has been shown to restore autophagy, reestablish host defense and enhance the clearance of pathogens, including *P. aeruginosa* and *B. cenocepacia*, in preclinical models of CF. CAT-5571 has the potential to augment the efficacy of antibiotics and could also be used with other CF therapies, including transmembrane conductance receptor (CFTR) targeted agents.

### **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<sup>SM</sup> 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 including, among other things, statements about the Company's plans to commence a single global Phase 3 trial in DMD in the first half of 2018 to evaluate the efficacy and safety of edasalonexent for registration purposes, the Company's plans to report top-line results from this trial in 2020, the Company's plans to initiate a Phase 1 trial for CAT-5571 in the second half of 2018 and report top-line results in 2019

and the Company's expectation that its current operating plan provides for cash to fund operations through September 2018, 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, including the final trial design of the Company's 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 the Company's planned Phase 3 trial in DMD in 2020 and from the Company's planned Phase 1 trial in CF in 2019; 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; the Company's ability to obtain financing on acceptable terms and in a timely manner to fund the Company's planned Phase 3 trial of edasalonexent in DMD for registration purposes and the Company's planned Phase 1 trial of CAT-5571 in CF; 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 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 December 30,</b>		<b>Year Ended December 31,</b>	
	<b>2017</b>	<b>2016</b>	<b>2017</b>	<b>2016</b>
Revenue	\$ 250	\$ -	\$ 500	\$ -
Operating expenses:				
Research and development	3,989	6,260	18,682	25,450
General and administrative	1,723	2,413	8,912	10,108
Total operating expenses	<u>5,712</u>	<u>8,673</u>	<u>27,594</u>	<u>35,558</u>
Loss from operations	(5,462)	(8,673)	(27,094)	(35,558)
Other (expense) income:				
Interest expense	(81)	(175)	(462)	(837)
Interest and investment income	32	59	160	242
Other income, net	14	11	32	93
Total other expense, net	<u>(35)</u>	<u>(105)</u>	<u>(270)</u>	<u>(502)</u>
Net loss	<u>\$ (5,497)</u>	<u>\$ (8,778)</u>	<u>\$ (27,364)</u>	<u>\$ (36,060)</u>
Net loss per share - basic and diluted	\$ (0.24)	\$ (0.47)	\$ (1.26)	\$ (2.22)
Weighted-average common shares outstanding used in net loss per share - basic and diluted	<u>23,218,476</u>	<u>18,699,480</u>	<u>21,681,534</u>	<u>16,230,190</u>

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

	<b>As of December 31,</b>	
	<b>2017</b>	<b>2016</b>
<b>Assets</b>		
Cash and cash equivalents	\$ 16,369	\$ 23,596
Available-for-sale securities	-	14,931
Total assets	17,897	40,209
<b>Liabilities and stockholders' equity</b>		
Current portion of notes payable, net of discount	2,479	3,243
Notes payable, net of current portion and discount	-	2,479
Total liabilities	6,105	11,123
Total stockholders' equity	\$ 11,792	\$ 29,086

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

	<b>Year Ended December 31,</b>	
	<b>2017</b>	<b>2016</b>
Net cash used in operating activities	\$ (26,836)	\$ (32,858)
Net cash provided by (used in) investing activities	14,883	(15,490)
Net cash provided by financing activities	4,726	9,164
Net decrease in cash and cash equivalents	<u>\$ (7,227)</u>	<u>\$ (39,184)</u>