



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

*-- Edasalonexent Substantially Slowed Duchenne Muscular Dystrophy Disease Progression through 36 Weeks; Plan to Initiate Phase 3 Trial in the First Half of 2018 --*

*-- Additional Preclinical Data Demonstrate Potential of CAT-5571 as a Treatment for Cystic Fibrosis; Plan to Initiate Phase 1 Trial in the Second Half of 2018 --*

**CAMBRIDGE, Mass., November 9, 2017** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported financial results for the third quarter ended September 30, 2017, and reviewed recent business progress.

“We have achieved an important milestone for the edasalonexent program,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “In our Phase 2 open-label extension of the MoveDMD<sup>®</sup> trial, we saw improvements across assessments of muscle function and muscle health as well as a continued strong safety profile through 36 weeks of edasalonexent treatment. Based on the consistency of these promising data from prespecified analyses as well as supportive input from the FDA, we plan to further evaluate edasalonexent as a potential novel disease-modifying therapy for boys with Duchenne regardless of mutation type in a global Phase 3 trial that we plan to begin in the first half of 2018.”

Dr. Milne continued, “Our team also advanced our research of CAT-5571, a potential oral treatment for cystic fibrosis-associated respiratory infections, which are the leading cause of morbidity and mortality for patients with cystic fibrosis. We expect to initiate a Phase 1 trial for CAT-5571 in the second half of 2018.”

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

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

- In the MoveDMD Phase 2 trial and open-label extension, sustained disease-modifying effects were seen through 36 weeks of treatment with edasalonexent. Across all assessments of muscle function, prespecified analyses showed improvements in the rate of decline after 24 and 36 weeks of oral 100 mg/kg/day edasalonexent treatment in the open-label extension compared to the rate of change in the control period for boys prior to receiving edasalonexent treatment. The totality of these data provides clinically meaningful evidence that edasalonexent substantially slowed the progression of DMD. Additionally, statistically significant changes in supportive measures of muscle health were seen in muscle enzymes and the magnetic resonance imaging (MRI) T2 composite measure of lower leg muscles. Edasalonexent continued to be well tolerated with no safety

signals observed in the trial. These data were presented at the World Muscle Society Conference in October.

- New biomarker results from the MoveDMD Phase 2 trial and open-label extension showed that C-reactive protein (CRP) was significantly decreased with edasalonexent at 12 and 24 weeks compared to baseline in the 100 mg/kg/day treatment group. CRP is a well-characterized blood test marker that provides a global assessment of inflammation. CRP is elevated in boys affected by DMD. The significant decrease observed in CRP supports the biological activity of NF- $\kappa$ B inhibition by edasalonexent treatment decreasing inflammation.
- Based on the consistency of the MoveDMD results and supportive regulatory input from FDA, Catabasis plans to initiate a single 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.

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

- In preclinical models of CF, CAT-5571 improved cellular clearance of the opportunistic and often fatal pathogen *Burkholderia cenocepacia* as reported at the North American Cystic Fibrosis Conference in November. This activity has the potential to address cystic fibrosis-associated respiratory infections by enhancing the clearance of pathogens, including *Pseudomonas aeruginosa* and *Burkholderia cenocepacia*, which are the leading cause of morbidity and mortality for patients with CF. CAT-5571 is designed to restore host defense by activating autophagy, which is known to be depressed in CF.
- Catabasis expects to initiate a Phase 1 trial for CAT-5571 in the second half of 2018 and report top-line results in 2019.

### **Third Quarter 2017 Financial Results**

**Cash Position:** As of September 30, 2017, Catabasis had cash and cash equivalents of \$21.7 million, compared to \$29.4 million in cash, cash equivalents and available-for-sale securities as of June 30, 2017. Catabasis' current operating plan provides for cash to fund operations through August 2018. To advance edasalonexent in the Phase 3 trial, Catabasis expects to seek additional funds through equity or debt financings or through collaboration or licensing transactions. Net cash used in operating activities for the three months ended September 30, 2017 was \$7.4 million, compared to \$6.9 million for the three months ended September 30, 2016. Net cash used in operating activities for the nine months ended September 30, 2017 was \$21.2 million, compared to \$24.9 million for the nine months ended September 30, 2016. Recognized revenue for the three and nine months ended September 30, 2017 was \$0.3 million from an option agreement with an unaffiliated party.

**R&D Expenses:** Research and development expenses were \$4.8 million for the three months ended September 30, 2017, compared to \$5.9 million for the three months ended September 30, 2016 and \$14.7 million for the nine months ended September 30, 2017, compared to \$19.2 million for the nine months ended September 30, 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 September 30, 2017, compared to \$2.3 million for the three months ended September 30, 2016 and \$7.2 million for the nine months ended September 30, 2017, compared to \$7.7 million for the nine months ended September 30, 2016.

**Operating Loss:** Loss from operations was \$7.0 million for the three months ended September 30, 2017, compared to \$8.3 million for the three months ended September 30, 2016, and \$21.6 million for the nine months ended September 30, 2017, compared to \$26.9 million for the nine months ended September 30, 2016.

**Net Loss:** Net loss was \$7.0 million, or \$0.31 per share, for the three months ended September 30, 2017, compared to a net loss of \$8.4 million, or \$0.54 per share, for the three months ended September 30, 2016. Net loss for the nine months ended September 30, 2017 was \$21.9 million, compared to \$27.3 million for the nine months ended September 30, 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 third quarter 2017 financial results.

Participant Toll-Free Dial-In Number: (877) 388-2733  
Participant International Dial-In Number: (541) 797-2984  
Pass Code: 99108456

Please specify to the operator that you would like to join the “Catabasis Third 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-κB, a protein that is activated in DMD and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. In the Phase 2 and open-label extension of the MoveDMD trial investigating the safety and efficacy of edasalonexent in boys enrolled at ages 4 to 7 affected with DMD (any confirmed mutation), edasalonexent substantially slowed DMD disease progression through 36 weeks of treatment. Across all key assessments of muscle function, consistent improvements were observed in the rate of decline after 24 and 36 weeks of oral 100 mg/kg/day edasalonexent treatment compared to the rate of change in the control period for boys prior to receiving edasalonexent treatment. Improvements were also seen across measures of muscle health. Edasalonexent continued to

be well tolerated with no safety signals observed in the trial. 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. 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 that is being developed as a potential host-directed therapy for cystic fibrosis. CAT-5571 is designed to restore host defense by activating autophagy, a mechanism for recycling cellular components and digesting pathogens. Autophagy is depressed in CF, and by restoring autophagy, CAT-5571 reestablishes host defense to enhance the clearance of pathogens, including *Pseudomonas aeruginosa* and *Burkholderia cenocepacia*, in preclinical models of CF. People with CF suffer from persistent lung infections with opportunistic pathogens such as *P. aeruginosa* and *B. cenocepacia*, causing chronic infections that are difficult to eradicate and lead to respiratory failure. 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 August 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 Quarterly Report on Form 10-Q for the period ended September 30, 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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Investor and Media Contact

Andrea Matthews

Catabasis Pharmaceuticals, Inc.

T: (617) 349-1971

[amatthews@catabasis.com](mailto:amatthews@catabasis.com)

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

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Revenue	\$ 250	\$ -	\$ 250	\$ -
Operating expenses:				
Research and development	4,776	5,936	14,693	19,190
General and administrative	2,426	2,347	7,189	7,695
Total operating expenses	<u>7,202</u>	<u>8,283</u>	<u>21,882</u>	<u>26,885</u>
Loss from operations	(6,952)	(8,283)	(21,632)	(26,885)
Other (expense) income:				
Interest expense	(105)	(199)	(381)	(662)
Interest and investment income	45	50	128	183
Other (expense) income, net	(5)	13	18	82
Total other expense, net	<u>(65)</u>	<u>(136)</u>	<u>(235)</u>	<u>(397)</u>
Net loss	<u>\$ (7,017)</u>	<u>\$ (8,419)</u>	<u>\$ (21,867)</u>	<u>\$ (27,282)</u>
Net loss per share - basic and diluted	<u>\$ (0.31)</u>	<u>\$ (0.54)</u>	<u>\$ (1.03)</u>	<u>\$ (1.77)</u>
Weighted-average common shares outstanding used in net loss per share - basic and diluted	<u>22,563,174</u>	<u>15,512,608</u>	<u>21,163,591</u>	<u>15,407,747</u>

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

	<u>September 30,</u>	<u>December 31,</u>
	<u>2017</u>	<u>2016</u>
<b>Assets</b>		
Cash and cash equivalents	\$ 21,713	\$ 23,596
Available-for-sale securities	-	14,931
Total assets	23,313	40,209
<b>Liabilities and stockholders' equity</b>		
Current portion of notes payable, net of discount	3,296	3,243
Notes payable, net of current portion and discount	-	2,479
Total liabilities	7,656	11,123
Total stockholders' equity	\$ 15,657	\$ 29,086

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

	<b>Nine Months Ended September 30,</b>	
	<b>2017</b>	<b>2016</b>
Net cash used in operating activities	\$ (21,199)	\$ (24,874)
Net cash provided by (used in) investing activities	14,883	(21,300)
Net cash provided by financing activities	4,433	9,858
Net decrease in cash and cash equivalents	<u>\$ (1,883)</u>	<u>\$ (36,316)</u>