



## **Catabasis Pharmaceuticals Reports Fourth Quarter and Full Year 2018 Financial Results and Reviews Business Progress**

*-- Edasalonexent Phase 3 PolarisDMD Trial Globally Enrolling Boys with Duchenne Muscular Dystrophy --*

**CAMBRIDGE, Mass., March 14, 2019** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported financial results for the fourth quarter and full year ended December 31, 2018, and reviewed recent business progress.

“We are off to a strong start in 2019, executing on our Phase 3 trial and further strengthening our financial position and Board of Directors to support our plans for transitioning to a commercial-stage organization. We continue to generate valuable data on edasalonexent that further reinforce its potential as a foundational therapy for Duchenne,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “We look forward to building on this momentum as we work toward our goal of bringing hope and life-changing therapies to patients and their families.”

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

- The Phase 3 PolarisDMD clinical trial evaluating edasalonexent in boys affected by Duchenne muscular dystrophy (DMD) is progressing well with patients enrolling at clinical trial sites globally.
  - The Phase 3 PolarisDMD trial is a one-year, randomized, double-blind, placebo-controlled trial. Catabasis plans to enroll approximately 125 patients ages 4 to 7 (up to 8<sup>th</sup> birthday) regardless of mutation type who have not been on steroids for at least 6 months.
  - Top-line results from the Phase 3 PolarisDMD trial are expected in the second quarter of 2020, and the trial is intended to support an application for commercial registration of edasalonexent.
- Recent data from the MoveDMD trial continue to support edasalonexent as a potential foundational treatment for DMD.
  - Boys treated with corticosteroids, the current standard of care in DMD, typically experience excess weight gain, curtailed growth and substantially increased body mass index (BMI). In contrast, positive growth data from the MoveDMD trial showed boys treated with edasalonexent grew age appropriately in both height and weight, resulting in decreased BMI that approached the average BMI for unaffected boys.
  - In addition to positive efficacy and safety results with edasalonexent as a monotherapy, MoveDMD also provided the foundation for combination therapy for the treatment of DMD. The combination of edasalonexent and the approved therapy EXONDYS 51<sup>®</sup>, developed by Sarepta Therapeutics, was well tolerated

with no safety signals. Two boys received this combination for an average of 1 year. These clinical safety data combined with preclinical data showing edasalonexent increased dystrophin expression in combination with exon-skipping therapy support the potential of edasalonexent to enhance dystrophin-targeted therapies such as EXONDYS 51 and other therapies in development.

- Catabasis' Board of Directors was strengthened with new appointments of Gregg Lapointe and Joanne T. Beck, Ph.D.
- Catabasis closed a \$20 million underwritten public offering in February 2019. The proceeds will be used for clinical trial and certain NDA-enabling activities; initial investments in commercial and medical affairs infrastructure to support our planned transition to a commercial-stage company; and for working capital and other general corporate purposes.

#### **Fourth Quarter and Full Year 2018 Financial Results**

**Cash Position:** As of December 31, 2018, Catabasis had cash, cash equivalents and short-term investments of \$37.6 million, compared to \$43.2 million as of September 30, 2018 and \$16.4 million as of December 31, 2017. Following December 31, 2018, Catabasis raised an additional \$20.5 million in net proceeds from equity financings. Based on the Company's current operating plan, Catabasis believes it has sufficient cash to fund operations into the fourth quarter of 2020. Net cash used in operating activities for the three months ended December 31, 2018 was \$5.3 million, compared to \$5.6 million for the three months ended December 31, 2017. Net cash used in operating activities for the full year 2018 was \$23.5 million, compared to \$26.8 million for the full year 2017.

**R&D Expenses:** Research and development expenses were \$3.7 million for the three months ended December 31, 2018, compared to \$4.0 million for the three months ended December 31, 2017 and \$17.0 million for the full year 2018, compared to \$18.7 million for the full year 2017.

**G&A Expenses:** General and administrative expenses were \$2.4 million for the three months ended December 31, 2018, compared to \$1.7 million for the three months ended December 31, 2017 and \$9.3 million for the full year 2018, compared to \$8.9 million for the full year 2017.

**Operating Loss:** Loss from operations was \$6.1 million for the three months ended December 31, 2018, compared to \$5.5 million for the three months ended December 31, 2017, and \$26.4 million for the full year 2018, compared to \$27.1 million for the full year 2017.

**Net Loss:** Net loss was \$6.1 million, or \$0.85 per share, for the three months ended December 31, 2018, compared to a net loss of \$5.5 million, or \$2.37 per share, for the three months ended December 31, 2017. Net loss for the full year 2018 was \$25.9 million, or \$5.12 per share, compared to \$27.4 million for the full year 2017. All per share figures give effect to the one-for-ten reverse stock split of Catabasis common stock that was effective on December 28, 2018.

#### **Conference Call and Webcast**

Catabasis will host a conference call and webcast at 8:30am ET today to provide an update on corporate developments and to discuss fourth quarter and full year 2018 financial results.

Participant Toll-Free Dial-In Number: (877) 388-2733  
Participant International Dial-In Number: (541) 797-2984  
Pass Code: 3873249

Please specify to the operator that you would like to join the “Catabasis Fourth Quarter and Full Year 2018 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 new standard of care for all patients affected by DMD, regardless of their underlying mutation. Edasalonexent inhibits NF-kB, which is a key link between loss of dystrophin and disease progression in DMD. NF-kB has a fundamental role in skeletal and cardiac muscle disease in DMD. We are currently enrolling our global Phase 3 PolarisDMD trial to evaluate the efficacy and safety of edasalonexent for registration purposes. In our MoveDMD Phase 2 trial and open-label extension, we observed that edasalonexent preserved muscle function and substantially slowed disease progression compared to rates of change in a control period, and significantly improved biomarkers of muscle health and inflammation. Edasalonexent continues to be dosed in the open-label extension of the MoveDMD trial. 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, please visit [www.catabasis.com](http://www.catabasis.com).

#### **About Catabasis**

At Catabasis Pharmaceuticals, our mission is to bring hope and life-changing therapies to patients and their families. Our lead program is edasalonexent, an NF-kB inhibitor in development for the treatment of Duchenne muscular dystrophy. Our global Phase 3 PolarisDMD trial is currently enrolling boys affected by Duchenne. For more information on edasalonexent and our Phase 3 PolarisDMD trial, 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 global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, the Company’s planned transition to a commercial-stage organization 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; 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, 2018, 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.**  
**Consolidated Statements of Operations**  
(In thousands, except share and per share data)  
*(Audited)*

	<b>Year Ended December 31,</b>	
	<b>2018</b>	<b>2017</b>
Revenue	\$ -	\$ 500
Operating expenses:		
Research and development	17,042	18,682
General and administrative	9,329	8,912
Total operating expenses	<u>26,371</u>	<u>27,594</u>
Loss from operations	(26,371)	(27,094)
Other income (expense):		
Interest expense	(100)	(462)
Interest and investment income	425	160
Other income, net	176	32
Total other income (expense), net	<u>501</u>	<u>(270)</u>
Net loss	<u>\$ (25,870)</u>	<u>\$ (27,364)</u>
Net loss per share - basic and diluted	<u>\$ (5.12)</u>	<u>\$ (12.62)</u>
Weighted-average common shares outstanding used in net loss per share - basic and diluted	<u>5,054,823</u>	<u>2,168,153</u>

**Catabasis Pharmaceuticals, Inc.**  
**Selected Consolidated Balance Sheets Data**  
(In thousands)  
*(Audited)*

	<b>December 31,</b>	<b>December 31,</b>
	<b>2018</b>	<b>2017</b>
<b>Assets</b>		
Cash and cash equivalents	\$ 15,294	\$ 16,369
Short-term investments	22,276	-
Total assets	39,169	17,897
<b>Liabilities and stockholders' equity</b>		
Current portion of notes payable, net of discount	-	2,479
Total liabilities	4,227	6,105
Total stockholders' equity	\$ 34,942	\$ 11,792

**Catabasis Pharmaceuticals, Inc.**  
**Selected Consolidated Statements of Cash Flows Data**  
(In thousands)  
*(Audited)*

	<b>Year Ended December 31,</b>	
	<b>2018</b>	<b>2017</b>
Net cash used in operating activities	\$ (23,465)	\$ (26,836)
Net cash (used in) provided by investing activities	(21,905)	14,883
Net cash provided by financing activities	44,295	4,726
Net decrease in cash and cash equivalents	<u>\$ (1,075)</u>	<u>\$ (7,227)</u>