



## **Catabasis Pharmaceuticals Reports Second Quarter 2018 Financial Results and Reviews Business Progress**

*-- Edasalonexent Phase 3 PolarisDMD Trial in Duchenne Muscular Dystrophy Expected to Initiate in the Second Half of 2018 --*

**CAMBRIDGE, Mass., August 9, 2018** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported financial results for the second quarter ended June 30, 2018, and reviewed recent business progress.

“We are thrilled to have achieved important milestones that enable the Phase 3 PolarisDMD trial of edasalonexent for registration purposes. We are now completing final preparations and expect to initiate the Phase 3 trial in the coming months,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “Based on muscle effects and stabilization of function seen with edasalonexent treatment in Phase 2, we believe edasalonexent could fundamentally change the course of Duchenne disease progression and the treatment paradigm. We look forward to advancing edasalonexent into the PolarisDMD trial as we progress towards our goal of making edasalonexent available to all those affected by Duchenne with the hope of enabling patients to maintain their functional abilities longer.”

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

- Announced plans for edasalonexent Phase 3 PolarisDMD trial in Duchenne muscular dystrophy (DMD), expected to initiate in the second half of 2018 with top-line results expected in the second quarter of 2020. The trial design was informed by discussions with the U.S. Food and Drug Administration (FDA) as well as input from treating physicians and families of boys affected by Duchenne.
  - The PolarisDMD trial will evaluate the efficacy and safety of edasalonexent in patients with DMD and is intended to support an application for commercial registration of edasalonexent.
  - The randomized, double-blind, placebo-controlled PolarisDMD trial is expected 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. Boys on a stable dose of eteplirsen may be eligible to enroll.
  - The primary efficacy endpoint will be change in the North Star Ambulatory Assessment score after 12 months of treatment with edasalonexent compared to placebo. Key secondary endpoints are planned to include the age-appropriate timed function tests time to stand, 4-stair climb and 10-meter walk/run. Assessments of growth, cardiac and bone health are also planned.

- Presented new edasalonexent clinical biomarker data demonstrating NF-kB target engagement in the MoveDMD trial in boys affected by DMD at the New Directions in Biology and Disease of Skeletal Muscle Conference in June 2018. NF-kB is a fundamental driver of disease progression in DMD.
- Presented new magnetic resonance imaging (MRI) data through one year of edasalonexent treatment at the American Academy of Neurology (AAN) 70th Annual Meeting in April 2018. Edasalonexent significantly slowed DMD disease progression as measured by MRI compared to the off-treatment control period.
- Catabasis closed a \$42 million underwritten public offering in June 2018. The proceeds will be used for the Phase 3 PolarisDMD clinical trial of edasalonexent for the treatment of DMD, as well as for working capital and general corporate purposes.

## **Second Quarter 2018 Financial Results**

**Cash Position:** As of June 30, 2018, Catabasis had cash and cash equivalents of \$49.9 million, compared to \$17.0 million as of March 31, 2018. Based on the Company's current operating plan, Catabasis believes it has sufficient cash to fund operations into Q2 2020. Net cash used in operating activities for the three months ended June 30, 2018 was \$5.6 million, compared to \$5.7 million for the three months ended June 30, 2017. Net cash used in operating activities for the six months ended June 30, 2018 was \$12.4 million, compared to \$13.8 million for the six months ended June 30, 2017.

**R&D Expenses:** Research and development expenses were \$4.2 million for the three months ended June 30, 2018, compared to \$4.5 million for the three months ended June 30, 2017 and \$9.5 million for the six months ended June 30, 2018, compared to \$9.9 million for the six months ended June 30, 2017. The decrease in research and development expenses was primarily attributable to a decrease in direct program costs.

**G&A Expenses:** General and administrative expenses were \$2.4 million for the three months ended June 30, 2018 and 2017, respectively, and \$4.8 million for the six months ended June 30, 2018 and 2017, respectively.

**Operating Loss:** Loss from operations was \$6.6 million for the three months ended June 30, 2018, compared to \$6.9 million for the three months ended June 30, 2017 and \$14.3 million for the six months ended June 30, 2018, compared to \$14.7 million for the six months ended June 30, 2017.

**Net Loss:** Net loss was \$6.5 million, or \$0.20 per share, for the three months ended June 30, 2018, compared to a net loss of \$7.0 million, or \$0.32 per share, for the three months ended June 30, 2017. Net loss for the six months ended June 30, 2018 was \$14.1 million, compared to \$14.9 million for the six months ended June 30, 2017.

## **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 second quarter 2018 financial results.

Participant Toll-Free Dial-In Number: (877) 388-2733  
Participant International Dial-In Number: (541) 797-2984  
Pass Code: 5876459

Please specify to the operator that you would like to join the “Catabasis Second Quarter 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 foundational 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, fibrosis and muscle degeneration and suppresses muscle regeneration. Edasalonexent continues to be dosed in an open-label extension of the MoveDMD Phase 2 clinical trial, and Catabasis is preparing to initiate a single global Phase 3 trial in the second half of 2018 to evaluate the efficacy and safety of edasalonexent for registration purposes. 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 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- $\kappa$ B inhibitor in development for the treatment of Duchenne muscular dystrophy. The global Phase 3 PolarisDMD trial is expected to initiate in the second half of 2018. 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 plans to commence the global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes, and the Company’s expectation that based on its current operating plan it has sufficient cash to fund operations into the second quarter of 2020 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 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.

###

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 June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2018</u>	<u>2017</u>	<u>2018</u>	<u>2017</u>
<b>Operating expenses:</b>				
Research and development	4,239	4,519	9,486	9,917
General and administrative	2,397	2,400	4,789	4,763
Total operating expenses	<u>6,636</u>	<u>6,919</u>	<u>14,275</u>	<u>14,680</u>
Loss from operations	(6,636)	(6,919)	(14,275)	(14,680)
<b>Other income (expense):</b>				
Interest expense	(33)	(127)	(90)	(276)
Interest and investment income	43	44	75	83
Other income, net	147	28	159	23
Total other income (expense), net	<u>157</u>	<u>(55)</u>	<u>144</u>	<u>(170)</u>
Net loss	<u>\$ (6,479)</u>	<u>\$ (6,974)</u>	<u>\$ (14,131)</u>	<u>\$ (14,850)</u>
Net loss per share - basic and diluted	<u>\$ (0.20)</u>	<u>\$ (0.32)</u>	<u>\$ (0.48)</u>	<u>\$ (0.73)</u>
Weighted-average common shares outstanding used in net loss per share - basic and diluted	<u>32,728,771</u>	<u>21,796,194</u>	<u>29,659,358</u>	<u>20,452,200</u>

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

	<u>June 30,</u>	<u>December 31,</u>
	<u>2018</u>	<u>2017</u>
<b>Assets</b>		
Cash and cash equivalents	\$ 49,931	\$ 16,369
Total assets	50,847	17,897
<b>Liabilities and stockholders' equity</b>		
Current portion of notes payable, net of discount	831	2,479
Total liabilities	5,035	6,105
Total stockholders' equity	\$ 45,812	\$ 11,792

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

	<b>Six Months Ended June 30,</b>	
	<b>2018</b>	<b>2017</b>
Net cash used in operating activities	\$ (12,421)	\$ (13,785)
Net cash provided by investing activities	8	14,901
Net cash provided by financing activities	45,975	4,657
Net increase in cash and cash equivalents	<u>\$ 33,562</u>	<u>\$ 5,773</u>