



## **Catabasis Pharmaceuticals Reports Second Quarter 2020 Financial Results and Reviews Business Progress**

*-- Top-Line Results Expected in Q4 2020 from Fully Enrolled Edasalonexent Global Phase 3 PolarisDMD Trial in Duchenne Muscular Dystrophy --*

**BOSTON, Mass., August 10, 2020** – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported financial results for the second quarter ended June 30, 2020 and reviewed recent business progress.

“We remain on track for top-line results from our Phase 3 PolarisDMD trial for edasalonexent in Duchenne and we are looking forward to reporting the results later this year,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “In anticipation of data, we are continuing to advance the program and are working on preparations for a New Drug Application as well as our strategy for commercialization and establishment of our supply chain. We see edasalonexent as a potential foundational therapy for all patients with DMD, regardless of underlying mutation.”

### **Phase 3 PolarisDMD Trial and GalaxyDMD Open-Label Extension**

- Top-line results from the fully enrolled Phase 3 PolarisDMD trial of edasalonexent in Duchenne muscular dystrophy (DMD) are expected in Q4 2020.
  - The Phase 3 trial enrolled 131 boys enrolled across 8 countries and is intended to support a new drug application (NDA) in 2021 for commercial registration of edasalonexent.
  - Due to the COVID-19 pandemic, Catabasis has implemented steps designed to enable continued safe conduct for patients and maintain study integrity, including the option to deliver study drug to patients’ homes, increased flexibility in the timing of patient visits, and utilization of telehealth.
- The open-label extension GalaxyDMD trial is enrolling boys who have completed the Phase 3 PolarisDMD trial as well as their interested eligible siblings. The trial is progressing as planned, with visits every six months.

### **Edasalonexent Highlights**

- Catabasis presented information on the edasalonexent program at the 2020 Muscular Dystrophy Association Virtual Poster Session and the Parent Project Muscular Dystrophy Virtual Annual Conference.
  - North Star Ambulatory Assessment (NSAA), the primary endpoint in the Phase 3 PolarisDMD trial, was shown to be a consistent and reproducible measure of function in young boys with DMD in an analysis of the screening and baseline values from the Phase 3 PolarisDMD trial before treatment with edasalonexent.

- The Phase 3 PolarisDMD trial was found to have enrolled a similar patient population as the Phase 2 MoveDMD trial. An analysis found overall similar baseline characteristics in the patient populations in the two trials. There were no significant differences between the two trials in baseline age, North Star Ambulatory Assessment (NSAA) score and all three timed function test values. These findings are believed to support the assumptions on which the Phase 3 trial was powered.
- In the Phase 2 MoveDMD trial and open-label extension, clinical findings support edasalonexent being associated with age-normative growth without negative impact on bone health with no evidence of adrenal insufficiency or clinically significant changes in adrenal function.
- Plans are progressing for the edasalonexent non-ambulatory trial in partnership with Duchenne UK and the trial is expected to initiate in 2021.

### **Corporate Highlights**

- Catabasis entered into an agreement with the Bill & Melinda Gates Medical Research Institute to study CAT-5571 in drug-sensitive and drug-resistant tuberculosis.
- Catabasis named Ben Harshbarger as Senior Vice President, adding extensive senior legal and compliance experience to the executive team.
- The Company plans to host a virtual investor and analyst event with a leading key opinion leader on Friday, September 11, 2020, to discuss edasalonexent and its potential in patients with DMD.

### **Second Quarter 2020 Financial Results**

**Cash Position:** As of June 30, 2020, Catabasis had cash, cash equivalents and short-term investments of \$53.9 million, compared to \$55.1 million as of March 31, 2020. Based on the Company's current operating plan, Catabasis expects that it has sufficient cash to fund operations through a potential NDA filing and through the third quarter of 2021. Net cash used in operating activities for the three months ended June 30, 2020 was \$7.5 million, compared to \$5.7 million for the three months ended June 30, 2019.

**R&D Expenses:** Research and development expenses were \$6.8 million for the three months ended June 30, 2020, compared to \$5.2 million for the three months ended June 30, 2019.

**G&A Expenses:** General and administrative expenses were \$2.8 million for the three months ended June 30, 2020, compared to \$2.2 million for the three months ended June 30, 2019.

**Operating Loss:** Loss from operations was \$9.6 million for the three months ended June 30, 2020, compared to \$7.3 million for the three months ended June 30, 2019.

**Net Loss:** Net loss was \$9.5 million, or \$0.53 per share, for the three months ended June 30, 2020, compared to a net loss of \$7.1 million, or \$0.62 per share, for the three months ended June 30, 2019.

### **About Edasalonexent (CAT-1004)**

Edasalonexent (CAT-1004) is an investigational oral small molecule designed to inhibit NF-kB that is being developed as a potential foundational therapy for all patients affected by DMD, regardless of their underlying mutation. In DMD the loss of dystrophin leads to chronic activation of NF-kB, which is a key driver of skeletal and cardiac muscle disease progression. The ongoing global Phase 3 PolarisDMD trial is evaluating the efficacy and safety of edasalonexent for registration purposes. Edasalonexent is also being evaluated in the GalaxyDMD open-label extension trial. In the MoveDMD Phase 2 trial and open-label extension, the Company 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. 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 Phase 3 development for the treatment of Duchenne muscular dystrophy. 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: the potential impact of the COVID-19 pandemic on the Company's business and operations; future clinical trial plans and potential regulatory activities including, among other things, statements about the potential commencement of the Company's planned trial in non-ambulatory patients, the Company's global Phase 3 PolarisDMD trial in DMD to evaluate the efficacy and safety of edasalonexent for registration purposes and related statements, including the anticipated timing for top-line results, steps that have been implemented to address the Covid-19 pandemic and the potential timing for the filing of an NDA; commercialization and supply chain preparations for edasalonexent; edasalonexent potentially being a foundational therapy for DMD patients; plans for an analyst and investor event; and having sufficient cash to fund operations through a potential NDA filing and through the third quarter of 2021, along with other statements containing the words "believes," "anticipates," "plans," "expects," "may" and similar expressions, constitute forward-looking statements within the meaning of applicable securities regulations and laws. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including risks and uncertainties: related to the impact of the COVID-19 pandemic and the effectiveness of the steps we have implemented to address the pandemic, including the use of telehealth visits; inherent in the initiation and completion of clinical trials and clinical development; related to whether the results of earlier stage clinical trials will be predictive of the results of later stage trials; related to the regulatory review and approval process; inherent in the commercialization of marketed products; related to successfully managing the Company's

potential transformation into a fully integrated company; related to competitive products, including those already approved and those in development; inherent in transitioning from a clinical to commercial supply chain, including the ability to enter into long-term agreements with key contract manufacturers, overseeing such manufacturers, and managing inventory, particularly where the Company expects to use sole source manufacturers for the foreseeable future; related to the availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements; related to other matters that could affect the clinical development, regulatory status, availability or commercial potential of the Company's product candidates; and related to general market and economic conditions, as well as the risks and uncertainties discussed in the "Risk Factors" section of the Company's Quarterly Report on Form 10-Q for the period ended June 30, 2020, 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)  
*(Unaudited)*

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
<b>Operating expenses:</b>				
Research and development	\$ 6,750	\$ 5,160	\$ 12,039	\$ 9,357
General and administrative	2,803	2,165	5,555	4,302
Total operating expenses	<u>9,553</u>	<u>7,325</u>	<u>17,594</u>	<u>13,659</u>
Loss from operations	(9,553)	(7,325)	(17,594)	(13,659)
<b>Other income (expense):</b>				
Interest and investment income	60	257	227	483
Other (expense) income, net	(15)	(63)	(93)	7
Total other income, net	<u>45</u>	<u>194</u>	<u>134</u>	<u>490</u>
Net loss	<u>\$ (9,508)</u>	<u>\$ (7,131)</u>	<u>\$ (17,460)</u>	<u>\$ (13,169)</u>
Net loss per share - basic and diluted	<u>\$ (0.53)</u>	<u>\$ (0.62)</u>	<u>\$ (1.03)</u>	<u>\$ (1.24)</u>
Weighted-average common shares outstanding used in net loss per share - basic and diluted	<u>17,967,495</u>	<u>11,505,542</u>	<u>16,933,079</u>	<u>10,600,909</u>

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

	<u>June 30,</u>	<u>December 31,</u>
	<u>2020</u>	<u>2019</u>
<b>Assets</b>		
Cash and cash equivalents	\$ 51,885	\$ 9,899
Short-term investments	2,001	26,345
Right-of-use asset	1,326	2,349
Other current and long-term assets	1,875	3,187
Total assets	<u>57,087</u>	<u>41,780</u>
<b>Liabilities and stockholders' equity</b>		
Current portion of operating lease liabilities	645	1,225
Long-term portion of operating lease liabilities	718	1,028
Other current and long-term liabilities	4,864	3,807
Total liabilities	<u>6,227</u>	<u>6,060</u>
Total stockholders' equity	\$ 50,860	\$ 35,720

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

	<b>Six Months Ended June 30,</b>	
	<b>2020</b>	<b>2019</b>
Net cash used in operating activities	\$ (14,455)	\$ (12,325)
Net cash provided by (used) in investing activities	24,310	(14,229)
Net cash provided by financing activities	31,889	20,875
Net increase (decrease) in cash, cash equivalents and restricted cash	<u>\$ 41,744</u>	<u>\$ (5,679)</u>