



Catabasis Pharmaceuticals Reports Second Quarter 2017 Financial Results and Reviews Business Progress

-- Favorable Results Seen in 12-week Edasalonexent Phase 2 MoveDMD[®] Trial in Duchenne Muscular Dystrophy; Phase 3 Clinical Trial Plan Expected Second Half of 2017 --

-- Preclinical Data Support CAT-5571 as a Potential Treatment to Enhance Host Defenses by Restoring Autophagy in Cystic Fibrosis --

CAMBRIDGE, Mass., August 10, 2017 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today reported financial results for the second quarter ended June 30, 2017, and reviewed recent business progress.

“In the second quarter, we presented an important prespecified crossover analysis of data from boys with Duchenne in our Phase 2 edasalonexent trial. We are very excited to see improvements in the rate of decline of muscle function across multiple assessments in boys treated with edasalonexent for 12 weeks. The results are very consistent with and support our earlier analysis of functional assessments in boys treated with edasalonexent for 12 weeks as compared to placebo and further strengthen our confidence in the potential of edasalonexent as a novel treatment for DMD. We continue to advance the open-label extension and expect to share 24-week edasalonexent results in the third quarter as well as announce our Phase 3 clinical trial plan for edasalonexent in the second half of 2017,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis.

Dr. Milne continued, “We are also encouraged by the results of our preclinical studies of CAT-5571, supporting its potential as an oral treatment for cystic fibrosis, and with our progress across our research programs driven by our SMART LinkerSM drug discovery technology.”

Recent and Upcoming Corporate Highlights

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

- In the MoveDMD Phase 2 edasalonexent trial, a crossover analysis of the rates of change across five assessments of muscle function in patients after 12 weeks of treatment compared to off-treatment prior to Phase 2 dosing showed clinically meaningful numerical improvements in rates of decline. The analysis was presented at the American Academy of Neurology 69th Annual Meeting in April and was the second of two prespecified analyses of the 12-week data. The first analysis was presented in March and showed meaningful improvements in assessments of muscle function in boys treated with edasalonexent compared to placebo. Functional assessments have precedence as endpoints in pivotal trials in DMD. The MoveDMD 12-week Phase 2 results are consistent with the therapeutic goal of treatment, to delay the predictable, sequential loss of function in DMD. There were no safety signals and edasalonexent was well tolerated in this study.
- The open-label extension of the MoveDMD trial is progressing as planned and results from 24 weeks of edasalonexent treatment are expected to be announced in the third quarter

of 2017. All boys participating in the open-label extension have now moved to the higher 100 mg/kg/day edasalonexent treatment group. Pending IRB approval, the open-label extension will be extended for an additional 52 weeks so that participating boys can continue to receive edasalonexent.

- Catabasis expects to announce the Phase 3 clinical trial plan for edasalonexent in DMD in the second half of 2017.
- The first boy in the MoveDMD trial who is amenable to exon 51 skipping treatment has started EXONDYS 51™ treatment along with edasalonexent in the open-label extension. The Catabasis and Sarepta joint research collaboration previously showed increased dystrophin expression in the *mdx* mouse with edasalonexent in combination with an exon-skip modality. Edasalonexent may have the potential to increase dystrophin levels in combination with dystrophin-targeted therapies.

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

- CAT-5571 demonstrated in preclinical studies improved intracellular clearance of bacterial pathogens that are clinically important in CF, as reported at the European Cystic Fibrosis Society Conference in June. This activity has the potential to improve lung function by reducing the intracellular load of multiple types of bacteria, including the pathogens, *Pseudomonas aeruginosa* and *Burkholderia cenocepacia*, which are the leading cause of morbidity and mortality for patients with CF. CAT-5571 restores autophagy, a host defense mechanism, which is known to be impaired in CF. Catabasis expects to initiate a Phase 1 trial for CAT-5571 in 2018.

Second Quarter 2017 Financial Results

Cash Position: As of June 30, 2017, Catabasis had cash and cash equivalents of \$29.4 million, compared to \$31.8 million in cash, cash equivalents and available-for-sale securities as of March 31, 2017. Catabasis expects that its current operating plan provides for cash to fund operations through August, 2018. Net cash used in operating activities for the three months ended June 30, 2017 was \$5.7 million, compared to \$8.8 million for the three months ended June 30, 2016. Net cash used in operating activities for the six months ended June 30, 2017 was \$13.8 million, compared to \$18.0 million for the six months ended June 30, 2016.

R&D Expenses: Research and development expenses were \$4.5 million for the three months ended June 30, 2017, compared to \$6.8 million for the three months ended June 30, 2016 and \$9.9 million for the six months ended June 30, 2017, compared to \$13.3 million for the six months ended June 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 June 30, 2017, compared to \$2.6 million for the three months ended June 30, 2016 and \$4.8 million for the six months ended June 30, 2017, compared to \$5.3 million for the six months ended June 30, 2016. The decrease in general and administrative expenses was primarily attributable to headcount reductions.

Operating Loss: Loss from operations was \$6.9 million for the three months ended June 30, 2017, compared to \$9.4 million for the three months ended June 30, 2016, and \$14.7 million for the six months ended June 30, 2017, compared to \$18.6 million for the six months ended June 30, 2016.

Net Loss: Net loss was \$7.0 million, or \$0.32 per share, for the three months ended June 30, 2017, compared to a net loss of \$9.4 million, or \$0.61 per share, for the three months ended June 30, 2016. Net loss for the six months ended June 30, 2017 was \$14.9 million, compared to \$18.9 million for the six months ended June 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 second quarter 2017 financial results.

Participant Toll-Free Dial-In Number: (877) 388-2733
Participant International Dial-In Number: (541) 797-2984
Pass Code: 47693255

Please specify to the operator that you would like to join the “Catabasis Second 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. 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- κ B, a protein that is activated in DMD and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. We are currently conducting the MoveDMD trial, a three-part clinical trial investigating the safety and efficacy of edasalonexent in boys enrolled at ages 4 – 7 affected with DMD (any confirmed mutation). The third part of the trial, an open-label extension with edasalonexent, is ongoing. 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.

About CAT-5571

Catabasis is developing CAT-5571 as a potential oral treatment for CF with potential effects on both the cystic fibrosis transmembrane conductance regulator (CFTR) and on the clearance of multiple types of bacteria including *Pseudomonas aeruginosa*. CAT-5571 is a small molecule that activates autophagy, a process that maintains cellular homeostasis and host defense mechanisms, and is known to be impaired in CF. Catabasis has observed in preclinical studies that CAT-5571, in combination with lumacaftor/ivacaftor, enhances cell-surface trafficking and function of CFTR with the F508del mutation. Catabasis has also observed that CAT-5571 enhances the clearance of *P. aeruginosa* infection in preclinical models of CF.

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 LinkerSM 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.

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 our plans to identify, develop and commercialize novel therapeutics based on our SMART Linker drug discovery platform, our plans to continue to evaluate data from the open-label extension of our MoveDMD[®] clinical trial of edasalonexent for the treatment of DMD, our plans for ongoing and planned clinical trials for edasalonexent and other product candidates, whether conducted by us or by any future collaborators, including the timing of initiation of these trials and of the anticipated results, 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; availability and timing of results from preclinical studies and clinical trials; 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 period ended June 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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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>2017</u> | <u>2016</u> | <u>2017</u> | <u>2016</u> |
| Operating expenses: | | | | |
| Research and development | \$ 4,519 | \$ 6,818 | \$ 9,917 | \$ 13,254 |
| General and administrative | 2,400 | 2,578 | 4,763 | 5,348 |
| Total operating expenses | <u>6,919</u> | <u>9,396</u> | <u>14,680</u> | <u>18,602</u> |
| Loss from operations | (6,919) | (9,396) | (14,680) | (18,602) |
| Other (expense) income: | | | | |
| Interest expense | (127) | (220) | (276) | (463) |
| Interest and investment income | 44 | 80 | 83 | 133 |
| Other expense, net | 28 | 91 | 23 | 69 |
| Total other expense, net | <u>(55)</u> | <u>(49)</u> | <u>(170)</u> | <u>(261)</u> |
| Net loss | <u>\$ (6,974)</u> | <u>\$ (9,445)</u> | <u>\$ (14,850)</u> | <u>\$ (18,863)</u> |
| Net loss per share - basic and diluted | <u>\$ (0.32)</u> | <u>\$ (0.61)</u> | <u>\$ (0.73)</u> | <u>\$ (1.23)</u> |
| Weighted-average common shares outstanding used in net loss per share - basic and diluted | <u>21,796,194</u> | <u>15,373,964</u> | <u>20,452,200</u> | <u>15,354,740</u> |

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

| | <u>June 30,</u> | <u>December 31,</u> |
|--|------------------|---------------------|
| | <u>2017</u> | <u>2016</u> |
| Assets | | |
| Cash and cash equivalents | \$ 29,369 | \$ 23,596 |
| Available-for-sale securities | - | 14,931 |
| Total assets | <u>30,816</u> | <u>40,209</u> |
| Liabilities and stockholders' equity | | |
| Current portion of notes payable, net of discount | 3,278 | 3,243 |
| Notes payable, net of current portion and discount | 831 | 2,479 |
| Total liabilities | <u>9,266</u> | <u>11,123</u> |
| Total stockholders' equity | <u>\$ 21,550</u> | <u>\$ 29,086</u> |

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

| | Six Months Ended June 30, | |
|--|----------------------------------|-------------|
| | 2017 | 2016 |
| Net cash used in operating activities | \$ (13,785) | \$ (17,976) |
| Net cash provided by (used in) investing activities | 14,901 | (18,971) |
| Net cash provided by (used in) financing activities | 4,657 | (1,555) |
| Net increase (decrease) in cash and cash equivalents | \$ 5,773 | \$ (38,502) |