Catabasis Pharmaceuticals and Sarepta Therapeutics Announce a Joint Research Collaboration in Duchenne Muscular Dystrophy

CAMBRIDGE, MA, September 29, 2016 – Catabasis Pharmaceuticals, Inc. (NASDAQ:CATB), a clinical-stage biopharmaceutical company ("Catabasis"), and Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage developer of innovative RNA-targeted therapeutics ("Sarepta"), today announced a joint research collaboration to explore a combination drug treatment approach for Duchenne muscular dystrophy (DMD). The two companies will contribute their respective expertise to study an exon skipping treatment developed by Sarepta, together with an oral NF-kB inhibition treatment developed by Catabasis in a mouse model of DMD.

"We are excited to work with Sarepta on this joint research collaboration, which to our knowledge is the first time two companies are testing a combination of investigational therapies to treat Duchenne. Although we believe edasalonexent (CAT-1004) has the potential to be a disease-modifying monotherapy, we think there is benefit to exploring innovative ways to make the most meaningful difference in this devastating disease", said Jill C. Milne, Ph.D., chief executive officer of Catabasis. "In addition to our continued development of edasalonexent, we are pleased to take the first step via this collaboration to determine if edasalonexent may be complementary to an exon-skipping treatment strategy in the treatment of DMD using a preclinical model."

"We recognize the extreme unmet medical need in DMD and are committed to determining the best treatment strategies for patients affected by Duchenne," said Edward Kaye, M.D., Sarepta's chief executive officer. "We believe exon skipping has the potential to target the underlying genetic cause of the disease by restoring the mRNA reading frame to produce dystrophin in skeletal muscle. We are pleased to initiate activities with Catabasis to evaluate a potential combination treatment approach of exon-skipping and NF-kB inhibition in DMD."

NF-kB inhibition and exon-skipping represent two novel investigational treatment strategies in Duchenne, each with the potential for disease-modifying effects when used as monotherapy. The objective of the joint research is to study the safety and efficacy of combining these two treatment strategies using a mouse model of DMD, including evaluating the potential for additional or synergistic benefits.

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 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.

About Edasalonexent (CAT-1004)

Edasalonexent (CAT-1004) is an oral small molecule that has the potential to be a disease-modifying therapy for all patients affected by Duchenne muscular dystrophy (DMD or Duchenne), regardless of the underlying mutation. Edasalonexent inhibits NF-kB, a protein that is activated in Duchenne and drives inflammation and fibrosis, muscle degeneration and suppresses muscle regeneration. In animal models of DMD, edasalonexent inhibited NF-kB, reduced muscle degeneration and improved muscle regeneration and function, and beneficial effects were observed in skeletal, diaphragm and cardiac muscle. 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. We have previously reported safety, tolerability and reduction in NF-kB activity in Phase 1 trials in adults. We are currently conducting the MoveDMD® trial of edasalonexent in 4-7 year-old boys affected by Duchenne. From Part A of the MoveDMD trial, we have reported that edasalonexent was generally well tolerated with no safety signals observed and we observed successful NF-kB target engagement. Pharmacokinetic results demonstrated edasalonexent plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-kB was observed.

About Sarepta Therapeutics
Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including EXONDYS 51, designed to skip exon 51 and approved under the accelerated approval pathway. For more information, please visit us at www.sarepta.com.

About Duchenne Muscular Dystrophy
DMD is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500-5,000 males worldwide. A devastating and incurable muscle-wasting disease, DMD is associated with specific errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness in the lower limbs spreads to the arms, neck and other areas. Eventually, increasing difficulty in breathing due to respiratory muscle dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and death usually occurs before the age of 30.

Forward-Looking Statements
Any statements in this press release about future expectations, plans and prospects for Catabasis and/or Sarepta, including statements about future clinical trial plans 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. These forward-looking statements include statements regarding the joint research collaboration plans of Sarepta and Catabasis to explore a combination drug treatment approach for DMD and the potential benefit of the products being researched in DMD. 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 Catabasis’ or Sarepta’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 Catabasis’ or Sarepta’s foreseeable and

unforeseeable operating expenses and capital expenditure requirements; other matters that could affect the availability or commercial potential of Catabasis' or Sarepta's product candidates; and general economic and market conditions and other factors discussed in the "Risk Factors" section of each of Catabasis' and Sarepta's Quarterly Report on Form 10-Q for the period ended June 30, 2016, which are each on file with the Securities and Exchange Commission, and in other filings that Catabasis or Sarepta may make with the Securities and Exchange Commission in the future. In addition, the forward-looking statements included in this press release represent Catabasis' and Sarepta's views as of the date of this press release. Each of Catabasis and Sarepta anticipates that subsequent events and developments will cause their respective views to change. However, while either Catabasis or Sarepta may elect to update these forward-looking statements at some point in the future, each company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the views of either Catabasis or Sarepta as of any date subsequent to the date of this release.

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