



**FOR IMMEDIATE RELEASE**

**Catabasis Pharmaceuticals Announces Positive Top-line Phase 1 Data for  
Hypercholesterolemia Product Candidate CAT-2054**

*--Safety, Tolerability, and Reductions in LDL-C Observed--*

**CAMBRIDGE, MA, August 13, 2015** – [Catabasis Pharmaceuticals](#), Inc. (NASDAQ:CATB), a clinical-stage drug development company built on a pathway pharmacology technology platform, today announced positive top-line Phase 1 clinical trial data for CAT-2054, the Company's product candidate targeting the Sterol Regulatory Element-Binding Protein (SREBP) pathway for the treatment of hypercholesterolemia. CAT-2054 was well-tolerated with no serious adverse events (AEs) observed in either the single or multiple ascending dose arms of the double-blind, randomized clinical trial. In the multiple ascending dose study, decreases in median LDL-C levels of up to 20% were observed at day 21 in healthy volunteers. Importantly, CAT-2054 was also found to be well-tolerated in combination with atorvastatin, the statin drug most commonly used in the treatment of hypercholesterolemia, and there was no evidence for impact of CAT-2054 on the pharmacokinetics of atorvastatin.

"CAT-2054 is an oral agent that targets SREBP, a key regulator of lipid metabolism, which may be an effective means to lower LDL-C. It also has potential effects on other metabolic parameters such as triglycerides, glucose, and liver fat in patients. The Phase 1 data of LDL-C efficacy signal combined with no safety issues support the continued clinical development of CAT-2054," said Evan A. Stein, M.D., Ph.D., Director Emeritus of the Metabolic & Atherosclerosis Research Center, Cincinnati, Ohio.

"Based on these data, we intend to initiate a Phase 2a trial in patients with hypercholesterolemia in the fourth quarter of 2015," said Jill C. Milne, Ph.D., chief executive officer of Catabasis.

The Phase 1 clinical trial was designed to assess the safety, tolerability and pharmacokinetics of single and multiple ascending oral doses of CAT-2054 in 118 healthy volunteers. In the single ascending dose portion of the trial, CAT-2054 was well-tolerated and no serious AEs were reported. No safety signals were observed in laboratory, vital sign or electrocardiogram results following CAT-2054 administration. The observed AEs occurring under fed and fasted conditions at doses up to 500 mg were similar for CAT-2054 and placebo. All reported AEs were mild.

In the multiple ascending dose portion of the Phase 1 trial, healthy volunteers received CAT-2054

or placebo at total daily doses ranging from 100 to 750 mg given orally once or twice per day for 14 days. CAT-2054 was also given concurrently with atorvastatin in one cohort. CAT-2054 was well-tolerated with no serious AEs reported. No safety signals were observed in laboratory, vital signs or electrocardiogram results following CAT-2054 administration, and all subjects completed dosing. At the highest doses, the most common AEs were GI-related, all of which were mild. CAT-2054 was also well-tolerated with no safety signals in subjects receiving atorvastatin. There was no evidence of clinically significant changes in atorvastatin pharmacokinetics when co-administered with CAT-2054. Catabasis plans to submit the Phase 1 data for presentation at an upcoming medical meeting.

### **About CAT-2054**

CAT-2054 is an investigational oral drug initially being developed for the treatment of hypercholesterolemia in patients for whom existing therapies are insufficient. By modulating the SREBP pathway, CAT-2054 may inhibit production of important cholesterol metabolism proteins such as PCSK9, HMG-CoA reductase, ATP citrate lyase and NPC1L1. If approved, CAT-2054 may have the potential to be the first therapy to simultaneously modulate cholesterol synthesis, clearance and absorption.

### **About Catabasis**

Catabasis Pharmaceuticals is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics using its proprietary Safely Metabolized And Rationally Targeted, or SMART, linker technology platform. The Company's SMART linker technology platform is based on the concept of treating diseases by simultaneously modulating multiple targets in one or more related disease pathways. The Company engineers bi-functional product candidates that are conjugates of two molecules, or bioactives, each with known pharmacological activity, joined by one of its proprietary SMART linkers. The SMART linker conjugates are designed for enhanced efficacy and improved safety and tolerability. The Company's focus is on treatments for rare diseases. The Company is also developing other product candidates for the treatment of serious lipid disorders. For more information on the Company's technology and pipeline of drug candidates, 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 and other statements containing the words "believes," "anticipates," "plans," "expects," 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 three months ended June 30, 2015, 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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