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Catabasis Pharmaceuticals Announces Top-Line Phase 2a Results for CAT-2054

CAMBRIDGE, MA, June 7, 2016 – [Catabasis Pharmaceuticals, Inc.](#) (NASDAQ:CATB), a clinical-stage biopharmaceutical company, today announced top-line results from its Phase 2a clinical trial of CAT-2054 for the treatment of hypercholesterolemia. CAT-2054 is an oral product candidate that inhibits Sterol Regulatory Element-Binding Protein (SREBP), a master regulator of lipid metabolism in the body. The double-blind, placebo-controlled trial assessed the effects of four weeks of treatment with CAT-2054 in patients with hypercholesterolemia on a high-intensity statin. The trial did not meet the primary end point in hypercholesterolemia, reduction in LDL-cholesterol (LDL-C) from baseline. Additional pre-specified analyses included changes in liver function tests (LFTs) to assess potential activity in nonalcoholic steatohepatitis (NASH). In a pre-specified analysis of patients more likely to have NASH, a potentially favorable pattern in LFTs was seen despite relatively low baseline LFT values and short trial duration and needs further investigation. All four doses of CAT-2054 in the trial were generally well tolerated.

In this Phase 2a trial, 153 patients were randomized with 94.8% completing the trial. The most common adverse events (AEs) were nausea, diarrhea and urinary tract infection (9%, 8% and 5% for CAT-2054 versus 3%, 0% and 10% for placebo, respectively), and four patients discontinued because of AEs. There were no treatment-related serious AEs. Catabasis plans to submit the data from this trial for presentation at an upcoming medical meeting.

“While CAT-2054 lowered LDL-C in normal healthy volunteers in its Phase 1 clinical trial, this effect was not observed in this Phase 2a trial in patients with hypercholesterolemia who were also on high-intensity statins,” said Jill C. Milne, Ph.D., Chief Executive Officer of Catabasis. “The trial was well designed and efficiently run, enabling us to quickly determine that we do not expect to invest further in CAT-2054 for hypercholesterolemia. We plan to complete additional analysis of the data and determine the best path forward for CAT-2054 in NASH. Our corporate strategy remains focused on our lead program, edasalonexent (CAT-1004) in Duchenne muscular dystrophy.”

“There is substantial mechanistic support for the role of SREBP in NASH and the recently presented preclinical data with a CAT-2054 analog are compelling. While this trial in a hypercholesterolemic population was not designed to assess NASH signals, the safety and LFTs observations support further exploration in NASH,” said Arun Sanyal, M.D., Professor of Medicine in the Gastroenterology Division of Virginia Commonwealth University Medical Center.

The CAT-2054 Phase 2a trial was a randomized, double-blind, placebo-controlled trial conducted at 31 clinical trial sites that enrolled 153 patients with hypercholesterolemia on statins. After a 4-week run-in on atorvastatin 40 mg, patients were randomized into one of four treatment groups (CAT-2054 250 mg or 400 mg once daily or twice daily) or placebo. The patients in this trial had hypercholesterolemia on statins with LDL cholesterol levels over 70 and less than 190 mg/dL. At baseline, mean LDL levels were 103 mg/dL, 30% of the patients had type 2 diabetes and 60% had metabolic syndrome. After four weeks of dosing with CAT-2054, the maximum LDL-C decrease observed was 7.1% compared to 4.5% with placebo. Additional secondary lipid end points included triglycerides and non-HDL cholesterol and these did not change significantly with treatment. In one pre-specified analysis, in a subset of the patients more likely to have NASH as determined by FIB-4 (Fibrosis-4, a non-invasive scoring system based on several laboratory tests that estimate the amount of scarring in the liver), a potentially favorable pattern in LFTs was seen. In this subset of patients (N=51), from a mean baseline ALT of 26 IU/mL there was a mean reduction in ALT of 3.0 IU/mL across all dosing arms of CAT-2054 compared to a reduction of 0.2 IU/mL with placebo and from a mean baseline AST of 25 IU/mL there was a mean reduction in AST of 2.8 IU/mL for those treated with CAT-2054 compared to an increase of 0.2 IU/mL with placebo.

About CAT-2054

CAT-2054 is an oral small molecule with a novel mechanism of action being developed as a potential treatment for nonalcoholic steatohepatitis (NASH). CAT-2054 inhibits Sterol Regulatory Element-Binding Protein (SREBP), a master regulator of lipid metabolism in the body, which has the potential to reduce liver fat. We have shown in pre-clinical models of NASH that a CAT-2054 analog significantly improves liver inflammation, fibrosis, steatosis and pre-neoplastic lesions. In a Phase 2a trial in patients with hypercholesterolemia, CAT-2054 had no effect on LDL-C levels when co-administered with high intensity statins. In a pre-specified analysis of patients more likely to have NASH, a potentially favorable pattern in liver function tests (LFTs) was seen despite relatively low baseline LFT values and short trial duration and needs further investigation.

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.

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," "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 March 31, 2016, 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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