CAMBRIDGE, MA, May 1, 2014 – Catabasis Pharmaceuticals Inc. today announced the presentation of preclinical data demonstrating the mechanism of action for CAT-2003. The data were presented by Michael Zimmer, Ph.D., in an oral session at the Arteriosclerosis, Thrombosis and Vascular Biology (ATVB) 2014 Scientific Sessions. The data demonstrate that CAT-2003 inhibits the transcription factor Sterol Regulatory Element Binding Protein (SREBP) in vitro and in vivo, resulting in the reduced expression of Proprotein Convertase Subtilisin/Kexin Type 9 (PCSK9), which normally acts to increase low-density lipoprotein (LDL) cholesterol. CAT-2003 currently is in a Phase 2 clinical trial for the treatment of patients with severe hypertriglyceridemias, including rare chylomicronemia syndromes.

“Our preclinical data demonstrating CAT-2003’s mechanism of action are particularly encouraging, as these data provide proof-of-concept that an oral small molecule can reduce PCSK9 levels through the modulation of SREBP,” said Michael Jirousek, Ph.D., co-founder and chief scientific officer of Catabasis.

The presentation entitled “CAT-2003 is a Novel Small Molecule that Inhibits Proprotein Convertase Subtilisin/Kexin Type 9 Production and Lowers Non-High Density Lipoprotein Cholesterol” was authored by Michael Zimmer, Dominic Picarella, Feng Liu, Pradeep Bista, Diana Lee, Chi Vu and Michael Jirousek. CAT-2003 was shown to inhibit the maturation of SREBP, a key regulator of lipid homeostasis. Inhibiting the maturation of SREBP resulted in a reduction of PCSK9, which correlated with an increase in LDL receptors. In hypercholesterolemia animal models, CAT-2003 lowered LDL and very low-density lipoprotein (VLDL) cholesterol both alone and in combination with the statin atorvastatin.

About CAT-2003

CAT-2003 is an oral, small molecule designed to treat severe hypertriglyceridemias, including rare chylomicronemia syndromes. CAT-2003 increases lipoprotein lipase (LPL) activity and reduces triglycerides by modulating the Sterol Regulatory Element Binding Protein (SREBP) pathway. SREBP is a key regulator of lipid homeostasis. CAT-2003 leverages Catabasis' proprietary SMART Linker technology to enable selective intracellular delivery and synergistic activity of the active components. CAT-2003 currently is in Phase 2 clinical development.

About Catabasis

Catabasis Pharmaceuticals is developing new medicines to treat patients with severe lipid disorders and rare diseases by applying its pathway pharmacology platform. The Company’s mission is to address difficult-to-treat diseases through the simultaneous modulation of multiple targets in a disease pathway. For more information on our technology and pipeline of drug candidates, please visit www.catabasis.com.
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