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## **Esperion Therapeutics Announces Positive Top-Line Results from Phase 2 Clinical Study of Novel Oral Therapy in Patients with Hypercholesterolemia and a History of Statin Intolerance**

### **Company Plans to Initiate Phase 2b Study in Statin Intolerant Patients in the Fourth Quarter of 2013**

Plymouth, Mich., June 7, 2013 -- Esperion Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on developing and commercializing first-in-class, oral, low-density lipoprotein cholesterol (LDL-C) lowering therapies for the treatment of hypercholesterolemia, today announced positive top-line results from a Phase 2a clinical study of its lead product candidate ETC-1002 in patients with hypercholesterolemia with a history of intolerance to two or more statins. The study met its primary endpoint, demonstrating that ETC-1002 lowered LDL-C by an average of 32 percent and was well tolerated. These data will be submitted for presentation at a future medical meeting and for publication in a peer-reviewed journal.

"I view this level of LDL-C reduction as comparable to what is seen with mid-dose statins," said Paul D. Thompson, M.D., Director of Cardiology, Cardiovascular Research at Hartford Hospital, and Professor of Medicine at the University of Connecticut. "These patients have very limited therapeutic options. Therefore, a well-tolerated medication that significantly lowers LDL-C could benefit this underserved patient population."

Esperion plans to initiate a Phase 2b clinical study of ETC-1002 that will include patients with hypercholesterolemia and a history of statin intolerance by the end of 2013. The Company expects that the Phase 2b study will be designed to evaluate multiple doses of ETC-1002 in a parallel group design of up to 12 weeks in duration with ezetimibe, a common treatment for statin intolerance, as a comparator. Therapies most often prescribed for patients with hypercholesterolemia and a history of statin intolerance have reported average LDL-C lowering of up to 18 percent in pivotal clinical studies. The goal of the Phase 2b study is to demonstrate comparable tolerability with superior efficacy of ETC-1002 to ezetimibe for the treatment of patients with hypercholesterolemia who are intolerant to two or more statins due to muscle-related adverse events.

#### **Phase 2a Study in Patients with Hypercholesterolemia and a History of Statin Intolerance**

This Phase 2a proof-of-concept clinical study was designed to evaluate the LDL-C lowering efficacy, safety and tolerability of ETC-1002 compared with placebo in patients with hypercholesterolemia and a history of intolerance to two or more statins. Study participants were dosed for eight weeks starting at 60 mg for two weeks, followed by 120 mg, 180 mg and 240 mg for two weeks each (or placebo only for eight weeks).

A total of 56 patients were evaluated in the study. A total of 37 patients were randomized to receive ETC-1002 and 19 patients received placebo. Thirty-one (84 percent) ETC-1002 patients and 15 (79 percent) placebo patients completed eight weeks of treatment. Three patients in the placebo group withdrew from the study for muscle related reasons while no patients in the ETC-1002 group withdrew for the same reasons. ETC-1002 lowered LDL-C by an average of 32 percent compared with an LDL-C reduction of 3 percent in the placebo group ( $p < 0.0001$ ).

High sensitivity C-reactive protein (hsCRP), a recognized marker for inflammation, was significantly reduced after eight weeks of ETC-1002 therapy.

Adverse event rates overall were comparable between the ETC-1002 and placebo groups with muscle-related adverse events similar between groups as well. No serious adverse events (SAE) were observed among placebo patients, and one unrelated SAE occurred in the ETC-1002 treatment group. No patients in the ETC-1002 treated group discontinued the study because of myalgia (muscle pain or weakness).

#### **About Statin Intolerance**

According to the USAGE survey, an academic study of approximately 10,000 current and former statin users published in 2012 in the Journal of Clinical Lipidology, approximately 12 percent of patients on statins discontinue therapy, and 62 percent of these patients cited side effects as the reason for discontinuation. More than 86 percent of patients who discontinued therapy because of side effects cited myalgia as the primary reason for discontinuing their statin. Based on these data, it is estimated that approximately 6 percent of statin users, or more than 2 million adults in the United States, ceased therapy because of

myalgia and are therefore considered to be statin intolerant. Poor statin adherence can be associated with worse cardiovascular outcomes.

### **About ETC-1002**

ETC-1002 is a first-in-class, orally available, once-daily LDL-C lowering small molecule therapy designed to lower levels of LDL-C and to avoid many of the side effects associated with many of the existing LDL-C lowering therapies. In the liver, ETC-1002 targets inhibition of ATP citrate lyase (ACL), a key enzyme in the cholesterol biosynthetic pathway, and activates a complementary enzyme, 5'-adenosine monophosphate-activated protein kinase (AMPK).

Esperion is currently conducting a Phase 2a clinical study of ETC-1002 in combination with statin therapy in patients with hypercholesterolemia. Top-line results are expected in the third quarter of 2013.

Phase 2 clinical studies of ETC-1002 conducted to date have demonstrated clinically meaningful LDL-C reductions of up to 43 percent, as well as reductions comparable to statins in levels of hsCRP, a key marker of inflammation associated with cardiovascular disease. In clinical research to date, ETC-1002 has been well tolerated. To date, one serious adverse event, considered unrelated to ETC-1002, has been observed in 275 patients treated with ETC-1002 at doses of up to 240 mg.

### **About Esperion Therapeutics**

Esperion Therapeutics, Inc. is a biopharmaceutical company focused on the research, development and commercialization of therapies for the treatment of patients with elevated levels of low-density lipoprotein cholesterol (LDL-C) and other cardiometabolic risk factors. ETC-1002, Esperion's lead product candidate, is a unique, first-in-class, orally available, once-daily LDL-C lowering small molecule therapy designed to lower levels of LDL-C and to avoid many of the side effects associated with existing LDL-C lowering therapies. ETC-1002 is targeted for statin intolerant patients with elevated levels of LDL-C. For more information, please visit [www.esperion.com](http://www.esperion.com).