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Cardiometabolic Health Congress Expert Faculty Evaluate the Clinical Implications of Newly FDA-Approved Treatment for Homozygous Familial Hypercholesterolemia.

BOSTON, Jan. 8, 2013 – The U.S. Food and Drug Administration (FDA) approved **JUXTAPID™ (lomitapide),** a new, first-in-class treatment option for clinical management of patients with homozygous familial hypercholesterolemia (HoFH) on December 28, 2012. The FDA decision prompted the Cardiometabolic Health Congress (CMHC) expert faculty to examine the implications this new treatment option may have on physician practice and patient care.

HoFH, an inherited genetic disorder caused by a mutation to the LDL receptor, is associated with early onset of atherosclerosis and premature cardiac death. Patients with HoFH tend to respond poorly to statin therapy because of low LDL receptor activity. For patients with HoFH who do not respond to statin therapy, LDL apheresis is indicated. This approach is expensive, invasive, and not universally available or effective alone – many patients do not achieve target LDL-C levels and as many as 30 percent of pediatric patients receiving long-term LDL apheresis progress to atherosclerotic disease of the coronary arteries and/or aorta.

Daniel Rader, MD, Cooper-McClure Professor of Medicine and Pharmacology and chief of the Division of Translational Medicine and Human Genetics in the Department of Medicine, the Perelman School of Medicine at the University of Pennsylvania, stated, "The magnitude of LDL-C and apoB reduction seen with lomitapide in patients with HoFH is substantially greater than that achieved with any other pharmacologic therapies in this disease and is expected to change the course of the disease and delay the onset and progression of atherosclerotic cardiovascular disease."

For patients with HoFH, heart and vascular disease often develop in childhood, and the average age of death is approximately 30 years. For this reason, Robert H. Eckel, MD, co-chair of the CMHC and professor of medicine at the University of Colorado Anschutz Medical Campus remarked, "The FDA decision to approve lomitapide provides a new option for patients with

HoFH, who so often develop extensive atherosclerotic cardiovascular disease as children, and remain inadequately treated." Christie Ballantyne, MD, co-chair of the CMHC, chief, Section of Cardiovascular Research and Cardiology at Baylor College of Medicine and director of the Center for Cardiovascular Disease Prevention at Methodist DeBakey Heart and Vascular Center echoed a similar sentiment: "I am excited that we will have a new treatment option available for patients with HoFH."

Sergio Fazio, MD, PhD, the Cornelius Vanderbilt Professor of Medicine and Pathology at Vanderbilt University School of Medicine in Nashville, Tenn., and chief of the Section of Cardiovascular Disease Prevention, cited the significance of the lomitapide approval: "This novel therapy is at the other end of the spectrum of cholesterol-lowering therapies – not a drug for everyone, like the statins, but an extreme intervention for extreme situations. Its mechanism of action is based on the inhibition of MTP, an intracellular protein that moves the lipid droplet to the forming lipoprotein in the liver and intestine. By targeting lipoprotein assembly, lomitapide has the power to reduce cholesterol irrespective of the type of mutation causing FH, since its effect is not based on up-regulation of the LDL receptor."

Convening Oct. 2–5, 2013, in Boston, the CMHC is co-chaired by George L. Bakris, MD; Christie M. Ballantyne, MD; Robert H. Eckel, MD; and Jay S. Skyler, MD, MACP. The CMHC provides the most advanced-level cardiometabolic education encompassing a multitude of risk factors, including cardiovascular disease, obesity, type 2 diabetes, dyslipidemia, atherosclerosis, hypertension, thrombosis, acute coronary syndrome, chronic kidney disease and related comorbidities.

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Disclosures:

Dr. Christie Ballantyne – *Consultant*: Aegerion Pharmaceuticals

Dr. Sergio Fazio – *Consultant*: participated in an advisory meeting sponsored by Aegerion Pharmaceuticals

Dr. Daniel Rader – *Ownership Interest/Shareholder*: Aegerion Pharmaceuticals