Press Release

Cerenis Therapeutics reports two positive Phase II studies for HDL mimetic CER-001 at EAS

Toulouse, FRANCE, Ann Arbor, UNITED STATES, June 2, 2014

- CER-001 proven to enhance Reverse Lipid Transport, facilitating elimination of cholesterol from the body
- Statistically significant reduction in carotid artery Mean Vessel Wall Area in two distinct clinical populations, as measured by Magnetic Resonance Imaging
- CER-001 complementary to LDL-c-lowering treatment and may represent a new chronic therapy on top of standard of care for patients with Familial Hypercholesterolemia and Familial Primary Hypoalphalipoproteinemia
- CER-001 well tolerated

TOULOUSE, France and ANN ARBOR, Michigan, June 2nd 2014 - Cerenis Therapeutics, the biopharmaceutical company, today announced that two of its Phase II studies, SAMBA and MODE (Modifying Orphan Disease Evaluation), with CER-001, an engineered human apoA-I-containing pre-β HDL mimetic, met their primary clinical endpoints in patients with Familial Primary Hypoalphalipoproteinemia (FPHA) and Homozygous Familial Hypercholesterolemia (HoFH), respectively.

Data are being presented at the Late Breaker Session at the European Atherosclerosis Society (EAS) Meetings in Madrid, Spain on June 2, 2014.

For the EAS Interactive Programme and link to the abstracts, please visit: www.eas.kenes.com.

SAMBA clinical trial:
Proof-of-Mechanism data will be presented at the EAS from the SAMBA Phase II efficacy and safety trial in patients with Familial Primary Hypoalphalipoproteinemia (FPHA), a rare syndrome of severe HDL deficiency caused by mutations in the genes responsible for HDL synthesis /maturation and characterized by accelerated atherosclerosis.

This pharmacokinetic/pharmacodynamic trial, conducted by Principal Investigator, Erik S.G. Stroes, MD, PhD of the Department of Vascular Medicine at the Academic Medical Center (AMC) in Amsterdam, The Netherlands, enrolled 7 FPHA patients in an open-label single-arm active-treatment study and assessed the efficacy of infused CER-001 engineered human apoA-I-containing pre-β HDL particles in reconstituting the endogenous Reverse Lipid Transport in individuals who have defects in the natural HDL pathway and facilitate elimination of cholesterol from the body.

The data from patients receiving CER-001 treatment on top of optimized standard of care LDL-c-lowering therapy showed that CER-001 performed the four steps of the Reverse Lipid Transport pathway: CER-001 increased cholesterol mobilization and esterification in the HDL fraction, and one month of treatment with 9 doses of CER-001 resulted in a statistically significant reduction in carotid artery Mean Vessel Wall Area, as measured by Magnetic Resonance Imaging (3T-MRI). CER-001 was well-tolerated.

Specific data from the study will be presented on Monday June 2, 2014 at 3:45pm in Madrid.

Dr. Stroes commented, "The results of this study support the future use of CER-001 for chronic administration aiming to reduce the elevated cardiovascular risk in low HDL patients with a high unmet clinical need."

MODE clinical trial:

Cerenis also reported that data from the MODE (Modifying Orphan Drug Evaluation) trial, a Phase II efficacy and safety study in patients with Homozygous Familial Hypercholesterolemia (HoFH), a rare disease of markedly elevated LDL-cholesterol (bad cholesterol) levels caused by genetic defects in the LDL-receptor pathway and characterized by premature atherosclerosis. Data will also be presented as a late-breaking clinical trial at the EAS.

The open-label single-arm active-treatment study in 23 patients with homozygous FH met the prespecified primary clinical endpoint in the modified Intention to Treat population, demonstrating a statistically significant reduction in carotid artery Mean Vessel Wall Area, as measured by Magnetic Resonance Imaging (3T-MRI), after 6-months of bi-weekly CER-001 treatment on top of optimized LDL-c-lowering therapy, including apheresis. CER-001 was well-tolerated.

Specific data from the study will be presented on Monday June 2, 2014 at 4:00pm in Madrid.

Dr. Kees Hovingh, the Principal Investigator of the MODE study, commented, "These data indicate that HDL therapy is complementary to LDL-c-lowering treatment and may represent a new therapy for addressing the unmet medical need in FH patients."

John F. Paolini, MD, PhD, FACC, Chief Medical Officer of Cerenis, said, “CER-001 has been shown to offer an important benefit for patients suffering from accelerated cardiovascular disease caused by genetic defects at both ends of the spectrum of cholesterol homeostasis. The demonstrated safety profile observed in all studies performed thus far supports the use of CER-001 for chronic treatment in both these rare patient populations.”

Jean-Louis Dasseux, PhD, MBA, and CEO of Cerenis, concluded: “In all preclinical and clinical studies to date, CER-001 has been shown to perform the functions of natural HDL and the steps of the Reverse Lipid Transport Pathway leading to elimination of cholesterol from the body with a high degree of potency. The clinical benefit observed in these genetically challenged patients is further evidence supporting HDL therapy in the broader treatment of atherosclerosis. We are committed to continuing the development of CER-001 in these important rare disease clinical **indications.”**

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