



Press Release

## Cash position and activity update for H1 2017

- **Solid cash position of €20.3 million at June 30, 2017**
- **CER-209: positive results from Phase I Single Dose Tolerance study for NAFLD and NASH**
- **CER-001: continuation of TANGO, a Phase 3 study in patients with HDL deficiency**

**Toulouse, FRANCE, Ann Arbor, UNITED-STATES, July 20, 2017, 6:00pm (CEST) – Cerenis Therapeutics (FR0012616852 – CEREN – PEA PME eligible)**, an international biopharmaceutical company dedicated to the discovery and development of innovative therapies based on lipid metabolism for treating cardiovascular and metabolic diseases, today announces its cash position at June 30, 2017 and key highlights of the first half of 2017.

### **Solid cash position of €20.3 million at June 30, 2017**

Cash and cash equivalents totaled €20.3 million at June 30, 2017. In line with expectations, Cerenis Therapeutics did not generate any revenue during the first half of 2017, the Company's products being at the Research and Development stage.

In June, the Company was granted non-dilutive funding of €0.75 million in the form of an interest-free innovation loan from Bpifrance. This amount corresponds to the payment of the second tranche of Bpifrance's innovation aid, an interest-free innovation loan totaling €1.5 million. Support from the French public organization is a direct result of the program's second key stage being reached, i.e. the start of the CER-209 drug candidate's Phase 1 clinical trial in Non-Alcoholic Fatty Liver Disease (NAFLD) and Non-Alcoholic Steato-Hepatitis (NASH).

### **CER-209: positive results from Phase I Single Dose Tolerance Study for NAFLD and NASH**

The Phase I Single Dose Tolerance Study, completed in June, has reported an absence of safety and tolerance issues associated with CER-209, as well as pharmacokinetics observations supporting once-daily doses of this drug candidate.

The objective of the Single Dose Tolerance study carried out in the US was to assess the safety, tolerability and pharmacokinetics of CER-209 when taken orally as a single dose. Escalating doses of 1, 3, 10 and 30 mg were tested in 4 cohorts of 6 subjects. In each cohort, four subjects were treated with active study medication and two subjects with a placebo.

The increasing incidence of NAFLD and NASH, now becoming common diseases of the liver, is related to the rise in obesity in the population. NAFLD, a precursor of NASH, is a disorder that is now considered to be the most common liver disease in the western world, affecting 30% of the world's population, according to a publication in the World Journal of Hepatology.

## **CER-001: continuation of TANGO, a Phase 3 study in patients with HDL deficiency**

Cerenis Therapeutics is currently conducting TANGO, a Phase 3 study in patients with HDL deficiency due to defects of genes coding for apoA-I and ABCA1, within the framework of two orphan drug designations granted by the European Medicines Agency (EMA). The results of this study are expected in early 2018. This delay is due to a slower than expected patient recruitment rate mainly due to difficulty finding subjects who have this rare indication and who are willing to make the time commitment necessary to participate in the trial.

TANGO is a Phase 3 clinical study designed to evaluate both the efficacy of CER-001 to regress atherosclerosis and its safety in patients with FPHA caused by an ABCA1 or an apoA-I genetic mutation and who are receiving optimized background lipid-lowering therapy.

Inherited defects in the apoA-I or ABCA1 genes can cause FPHA, a rare syndrome characterized by the absence or severe deficiency of HDL particles in the blood. This means that the body's only natural mechanism for eliminating cholesterol is compromised. These patients thus experience an increased accumulation of cholesterol, particularly in blood vessel walls, which often results in accelerated atherosclerosis and premature cardiovascular disease.

**Dr Jean-Louis Dasseux, founder and CEO of Cerenis Therapeutics,** states: *"The clinical development of CER-209 reached a key milestone during the first half of the year, following the positive results of the Single Dose Tolerance Study. Building on this success, which is reflected in the funding received from Bpifrance, we are looking forward to continuing the development of CER-209 within the framework of a multiple-dose safety and tolerability study. Given its ability to promote the recognition of HDL and the elimination of lipids by the liver, CER-209 has substantial potential for the treatment of NAFLD and NASH, which are part of today's biggest global health issues. In addition, we continue the TANGO Phase 3 study with CER-001 and results are expected in early 2018 taking into account the challenges associated with the recruitment of patients with such a rare disease. Our clinical advances and the Company's financial position give us total confidence in the development prospects of Cerenis, which benefits from a diversified portfolio of drug candidates characterized by clearly distinct mechanisms of action."*

### **Financial agenda:**

2017 half-year results

**September 12, 2017**

### **About Cerenis: [www.cerenis.com](http://www.cerenis.com)**

Cerenis Therapeutics is an international biopharmaceutical company dedicated to the discovery and development of innovative lipid metabolism therapies for the treatment of cardiovascular and metabolic diseases. HDL is the primary mediator of the reverse lipid transport, or RLT, the only natural pathway by which excess lipids is removed from arteries and is transported to the liver for elimination from the body.

Cerenis is developing a portfolio of lipid metabolism therapies, including HDL mimetics for patients with genetic HDL deficiency, as well as drugs which increase HDL for patients with a low number of HDL particles to treat atherosclerosis and associated metabolic diseases including Non-Alcoholic Fatty Liver Disease (NAFLD) and Non-Alcoholic Steato-Hepatitis (NASH).

Cerenis is well positioned to become one of the leaders in the HDL therapeutic market, with a broad portfolio of programs in development.

### About CER-209

CER-209 is the first drug candidate in the category of oral P2Y13 receptor agonists. CER-209 is a specific agonist of the P2Y13 receptor and does not interact with the P2Y12 receptor. In preclinical studies CER-209 promotes HDL recognition by the liver and increases Reverse Lipid Transport (RLT), thereby impacting atherosclerosis regression. Because of the favorable metabolic effects observed in the liver, CER 209 may also offer a new mechanism for the treatment of Non-Alcoholic Fatty Liver Disease (NAFLD) and Non-Alcoholic Steato-Hepatitis (NASH).

### About TANGO clinical trial

TANGO is a Phase 3, multicenter, randomized, 48-week, double-blind, parallel-group, placebo-controlled study to evaluate the efficacy and safety of CER-001 on vessel wall area in thirty patients with genetically defined familial hypoalphalipoproteinemia (apoA-I and ABCA1 deficiencies) and receiving background optimized lipid therapy. Primary endpoint: to evaluate the effect of 24 weeks' treatment with CER-001 on carotid mean vessel wall area (MVWA) compared with placebo using 3TMRI. Secondary endpoints: to evaluate the effect of 8 and 48 weeks' treatment with CER-001 on carotid MVWA compared with placebo using 3TMRI.

### About CER-001

CER-001 is an engineered complex of recombinant human apoA-I, the major structural protein of HDL, and phospholipids. It has been designed to mimic the structure and function of natural, nascent HDL, also known as pre-beta HDL. Its mechanism of action is to increase apoA-I and the number of HDL particles transiently, to stimulate the removal of excess cholesterol and other lipids from tissues including the arterial wall and to transport them to the liver for elimination through a process called Reverse Lipid Transport. SAMBA, the clinical Phase 2 study in patients with hypoalphalipoproteinemia due to genetic defects, has provided important data demonstrating the efficacy of CER-001 in regressing atherosclerosis in several distinct vascular beds. The totality of the data to date indicates that CER-001 performs all of the functions of natural pre-beta HDL particles and has the potential to be the best-in-class HDL mimetic on the market.



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