Santaris Pharma A/S to report new clinical data from miravirsen Phase 2a study to treat Hepatitis C in late-breaking oral presentation at the AASLD 2011 annual meeting

Hoersholm, Denmark/San Diego, California, October 3, 2011 — Santaris Pharma A/S, a clinical-stage biopharmaceutical company focused on the discovery and development of microRNA and mRNA-targeted therapies, today announced the Company will report new clinical data results from the miravirsen Phase 2a proof-of-concept study to treat patients infected with the Hepatitis C virus (HCV) in a late-breaking oral presentation session at the American Association for the Study of Liver Diseases (AASLD) 2011 annual meeting.

Data from the Phase 2a trial showed that miravirsen, the first microRNA-targeted drug to enter clinical trials, provided continuous and prolonged anti-viral activity well beyond the end of active therapy in patients and was well tolerated in patients infected with HCV. These data demonstrate that miravirsen has the potential to be a once-weekly treatment for chronic HCV infection.

The abstract titled, "A Randomized, Double-blind, Placebo Controlled Safety and Anti-viral Proof of Concept Study of Miravirsen, an Oligonucleotide Targeting miR-122, In Treatment Naïve Patients with Genotype 1 Chronic HCV Infection" will be presented in a late-breaking oral presentation on November 7 at 4p.m., at The Liver Meeting® 2011, the 62nd annual meeting of the AASLD, taking place from November 4-8 in San Francisco, California. The abstract is published on the AASLD website at http://aasld2011.abstractcentral.com/login.

The randomized, double-blind, placebo-controlled, ascending multiple-dose Phase 2a study assessed the safety and tolerability of miravirsen in treatment-naïve patients with chronic HCV genotype 1 infection. Patients with chronic HCV genotype 1 infection were enrolled sequentially to one of three cohorts (9 active: 3 placebo per cohort). Miravirsen was given as weekly subcutaneous injections, over 29 days.

Key data that will be presented demonstrate that miravirsen was associated with dose-dependent, prolonged reductions in HCV RNA that continued to fall after the completion of miravirsen therapy (see table 1). In Cohort 2, the mean of the maximum change from baseline in HCV RNA (log10 IU/mL) over the first 10 weeks was -2.710 vs -0.152 (p =0.020) in the miravirsen and placebo groups, respectively. In Cohort 2, five subjects had a greater than or equal to a 2 log10 decrease from baseline to week 10 and one patient became HCV RNA undetectable 10 weeks after the last dose of miravirsen (week 14) without the addition of standard-of-care (SOC). Cohort 3 is ongoing.

Table 1: HCV RNA (Log10 IU/mL) Change from Baseline

	week 2	week 4	week 6	week 8	week 10
placebo	0.319	0.296	0.180	0.054	0.051
miravirsen	-0.134 (p=0.051)	-0.849 (p=0.008)	-1.711 (p=0.009)	-2.423 (p=0.021)	-2.163 (p=0.064)

Developed using Santaris Pharma A/S proprietary Locked Nucleic Acid (LNA) Drug Platform, miravirsen is an inhibitor of miR-122, a liver specific microRNA that the Hepatitis C virus requires for replication. Miravirsen is designed to recognize and sequester miR-122, making it unavailable to the Hepatitis C virus. As a result, the replication of the virus is effectively inhibited and the level of Hepatitis C virus is profoundly reduced.

(more)

About Hepatitis C

Hepatitis C is a viral disease caused by the Hepatitis C virus that leads to swelling (inflammation) of the liver. The World Health Organization estimates about 3% of the world's population has been infected with HCV and that some 170 million are chronic carriers at risk of developing liver cirrhosis and/or liver cancer[1]. Approximately 3-4 million Americans are chronically infected with an estimated 40,000 new infections per year[2]. In Europe, there are about 4 million carriers[1]. Treatment with the current standard of care, pegylated interferon in combination with ribavirin, is effective in only about 50% of those treated[2]. Patients that are not effectively treated have an increased risk for the progression of liver disease. By 2029, total annual medical costs in the United States for people with Hepatitis C are expected to more than double, from $30 billion in 2009 to approximately $85 billion[3].

About microRNAs

MicroRNAs have emerged as an important class of small RNAs encoded in the genome. They act to control the expression of sets of genes and entire pathways and are thus thought of as master regulators of gene expression. Recent studies have demonstrated that microRNAs are associated with many disease processes. Because they are single molecular entities that dictate the expression of fundamental regulatory pathways, microRNAs represent potential drug targets for controlling many biologic and disease processes.

About Locked Nucleic Acid (LNA) Drug Platform

The LNA Drug Platform and Drug Discovery Engine developed by Santaris Pharma A/S combines the Company's proprietary LNA chemistry with its highly specialized and targeted drug development capabilities to rapidly deliver LNA-based drug candidates against RNA targets, both mRNA and microRNA, for a range of diseases including cardiometabolic disorders, infectious and inflammatory diseases, cancer and rare genetic disorders. LNA-based drugs are a promising new class of therapeutics that are enabling scientists to develop drug candidates to work through previously inaccessible clinical pathways. The LNA Drug Platform overcomes the limitations of earlier antisense and siRNA technologies to deliver potent single-stranded LNA-based drug candidates across a multitude of disease states. The unique combination of small size and very high affinity allows this new class of drugs candidates to potently and specifically inhibit RNA targets in many different tissues without the need for complex delivery vehicles. The most important features of LNA-based drugs include excellent specificity providing optimal targeting; increased affinity to targets providing improved potency; and favorable pharmacokinetic and tissue-penetrating properties that allow systemic delivery of these drugs without complex and potentially troublesome delivery vehicles.

About Santaris Pharma A/S

Santaris Pharma A/S is a privately held clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies. The Locked Nucleic Acid (LNA) Drug Platform and Drug Discovery Engine developed by Santaris Pharma A/S combine the Company's proprietary LNA chemistry with its highly specialized and targeted drug development capabilities to rapidly deliver potent single-stranded LNA-based drug candidates across a multitude of disease states. The Company's research and development activities focus on infectious diseases and cardiometabolic disorders, while partnerships with major pharmaceutical companies include a range of therapeutic areas including cancer, cardiovascular disease, infectious and inflammatory diseases, and rare genetic disorders. The Company has strategic partnerships with miRagen Therapeutics, Shire plc, Pfizer, GlaxoSmithKline, and Enzon Pharmaceuticals. As part of its broad patent estate, the Company holds exclusive worldwide rights to all therapeutic uses of LNA. Santaris Pharma A/S, founded in 2003, is headquartered in Denmark with operations in the United States. Please visit www.santaris.com for more information.

(more)

Media Contact:
Navjot Rai
Director, Global Communications
Santaris Pharma A/S
Office: + 1 858 764 7064 ext. 206
Cell: +1 619 723 5450
e-mail: navjot.rai@santaris.com

---

[1] World Health Organization - http://www.who.int/csr/disease/hepatitis/Hepc.pdf

[2] American Association for the Study of Liver Diseases - http://www.aasld.org/patients/Pages/LiverFastFactsHepC.aspx

[3] Institute of Medicine of the National Academies. Hepatitis and liver cancer: a national strategy for prevention and control of hepatitis B and C. Colvin HM and Mitchell AE, ed. Available at: http://www.iom.edu/Reports/2010/Hepatitis-and-Liver-Cancer-A-National-Strategy-for-Prevention-and-Control-of-Hepatitis-B-and-C.aspx.