Santaris Pharma A/S presents final Phase 2a study results for miravirsen showing dose-dependent, prolonged antiviral activity in Hepatitis C patients

-- Data to be presented in oral presentation at EASL Annual Meeting --

- *Miravirsen is a host-targeted, pan-HCV genotype anti-viral agent and the first microRNA-targeted drug to enter clinical trials*
- *Miravirsen was safe, well tolerated and provided prolonged antiviral activity, with no signs of viral resistance*
- *Miravirsen provided robust, dose-dependent antiviral activity with a mean reduction of 2 to 3 logs from baseline in hepatitis C virus (HCV) RNA (log10 IU/mL) that was sustained well beyond (> 4 weeks) the end of therapy*
- *Four out of nine patients treated at the highest dose with miravirsen became HCV RNA undetectable during the study, providing clinical evidence that miravirsen's unique mechanism-of-action offers the potential for treatment cures when used as monotherapy*
- *Miravirsen's long-lasting suppression of HCV RNA, high barrier to viral resistance, low propensity for drug interactions and favorable tolerability and dosing profiles holds promise as new treatment option given as monotherapy or in combination with direct acting antiviral agents*

Hoersholm, Denmark/San Diego, California, April 19, 2012 — Santaris Pharma A/S, a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, will present final data from a Phase 2a trial showing that miravirsen given as a four-week monotherapy treatment provided robust dose-dependent anti-viral activity with a mean reduction of 2 to 3 logs from baseline in HCV RNA (log10 IU/mL) that was sustained well beyond the end of therapy. These final clinical data are being presented at the International Liver Congress™ 2012, the annual meeting of the European Association for the Study of the Liver (EASL), taking place on April 18 – 22 in Barcelona, Spain.

Clinical data from the Phase 2a study will be presented in an oral presentation on April 20, 2012 at 5:15 p.m. CEST, titled "Final Results: Randomized, Double-Blind, Placebo-Controlled Safety, Anti-Viral Proof-of-Concept Study of Miravirsen, an Oligonucleotide Targeting miR-122, in Treatment-Naïve Patients with Genotype 1 Chronic HCV Infection." Study results demonstrated the following:

- Miravirsen was safe and well tolerated
- Adverse events were generally infrequent, mild and similar between treatment groups
- No dose limiting toxicities or any discontinuations due to adverse events
- Miravirsen was associated with dose-dependent reductions in HCV RNA that were sustained well beyond the end of the four-week dosing period
- Four out of nine patients treated at the highest dose (7 mg/kg) with miravirsen became HCV RNA undetectable with just five weekly doses of miravirsen monotherapy
- No evidence of viral resistance

"These data provide clinical evidence that miravirsen's unique mechanism-of-action offers a high barrier to viral resistance and the potential for cure with monotherapy. Due to its ability in targeting miR-122, miravirsen has the potential to change the way hepatitis C is treated," said Dr. Henk Reesink, Academic Medical Center, University of Amsterdam, who is presenting the data at EASL. "These data show that longer duration of miravirsen monotherapy has the potential to produce sustained virological responses."

The mean of the maximum decline from baseline in HCV RNA (log10 IU/mL) over the 18 week study was 1.2, 2.9, 3.0 in the 3, 5 and 7 mg/kg miravirsen dose groups respectively vs. 0.4 in the placebo group (Table 1).

Table 1: Mean of the Maximum HCV RNA (Log10 IU/mL) Change from Baseline in all 3 Cohorts

Dose Group	Mean Maximum HCV RNA decline IU/mL (SEM)	p-value (t) MIR vs. placebo
Pooled placebo	0.4 (0.4)	---
Miravirsen 3 mg/kg	1.2 (0.6)	0.011
Miravirsen 5 mg/kg	2.9 (1.7)	0.003
Miravirsen 7 mg/kg	3.0 (1.6)	0.002

"We are pleased that the final data set reinforces that miravirsen, the first microRNA-targeted drug to be given to patients, provides long-lasting suppression of HCV RNA, has a high barrier to viral resistance and favorable tolerability and dosing profiles," said Michael R. Hodges, MD, Vice President and Chief Medical Officer at Santaris Pharma A/S. "Miravirsen has the potential to be used in combination with direct acting anti-viral agents as part of an interferon-free, dosing regimen in patients with all types of HCV genotypes."

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) at doses of 3, 5 and 7 mg/kg. Miravirsen was given as a total of 5 weekly subcutaneous injections over 29 days.

Additional data supporting the final results in the miravirsen Phase 2a study will also be presented in posters demonstrating evidence of its high barrier to resistance and favorable dosing schedule:

- "Preclinical characterization of miravirsen (miR), an oligonucleotide targeting miR-122 in the HCV genotype 1B Replicon System" – Søren Ottosen, et al.; April 20, 2012 from 12:30-2 p.m. CEST
- "Pharmacokinetics of miravirsen, a miR-122 inhibitor, predict the prolonged viral load reduction in treatment naïve genotype 1 HCV infected patients" – Robert Persson, et al.; April 21, 2012 from 12:30-1:30 p.m. CEST
- "Sequence analysis of HCV variants from a Phase IIa trial of miravirsen (miR), an oligonucleotide targeting miR-122, in treatment naïve patients with chronic HCV infection" – A.K. Patick, et al.; April 21, 2012 from 12:30-1:30 p.m. CEST

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.

About Hepatitis C

Hepatitis C infection is a viral disease caused by the Hepatitis C virus that leads to 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 have chronic hepatitis C and are 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 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 manufacture, have manufactured and sell products that comprise LNA as active ingredient for studies performed with a view to obtaining marketing approval. 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.

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[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.