Santaris Pharma A/S advances miravirsen, the first microRNA-targeted drug to enter clinical trials, into Phase 2 to treat patients infected with Hepatitis C virus

- ***Santaris Pharma A/S initiates Phase 2a clinical trial with miravirsen (SPC3649) to assess safety and tolerability in treatment-naïve patients with chronic Hepatitis C***
- ***Miravirsen is the first microRNA-targeted drug to receive Investigational New Drug (IND) acceptance from FDA, paving the way to conduct Phase 2 trials for treatment of Hepatitis C in the United States***
- ***Developed using Santaris Pharma A/S Locked Nucleic Acid (LNA) Drug Platform, miravirsen inhibits miR-122, a microRNA important for Hepatitis C viral replication, thereby significantly reducing the levels of Hepatitis C virus***
- ***Due to unique mechanism-of-action, miravirsen holds promise as new treatment option for Hepatitis C patients, including the 50% of patients not responsive to current standard of care[1]***

Hoersholm, Denmark/San Diego, California, September 22, 2010 — Santaris Pharma A/S, a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced that it has advanced miravirsen (SPC3649), the first microRNA-targeted drug to enter clinical trials, into Phase 2 studies to assess the safety and tolerability of the drug in treatment-naïve patients infected with the Hepatitis C virus (HCV).

Paving the way to conduct the first clinical trials of a microRNA-targeted drug in the United States, Santaris Pharma A/S also received acceptance of its Investigational New Drug (IND) application from the U.S. Food and Drug Administration (FDA). In addition to the United States, the Phase 2a clinical trials will be conducted in the Netherlands, Germany, Poland, Romania, and Slovakia.

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[2]. Approximately 3-4 million Americans are chronically infected with an estimated 40,000 new infections per year[1]. In Europe, there are about 4 million carriers[2]. The current standard of care, pegylated interferon in combination with ribavirin, is effective in only about 50% of those treated[1].

Developed using Santaris Pharma A/S proprietary Locked Nucleic Acid (LNA) Drug Platform, miravirsen is a specific 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 reduced.

"Advancing miravirsen, the first microRNA-targeted drug to enter clinical trials, into Phase 2 studies in patients with Hepatitis C demonstrates Santaris Pharma A/S leadership in developing RNA-targeted medicines," said Arthur A. Levin, Ph.D., Vice President, Chief Development Officer and President, US Operations. "Receiving IND acceptance from the FDA to conduct the first clinical trials with a microRNA-targeted drug in the United States brings Santaris Pharma A/S one step closer to potentially providing a growing number of patients chronically infected with HCV with a more effective and better tolerated treatment option."

The LNA Drug Platform is the only technology with both mRNA and microRNA targeted drugs in clinical trials, reinforcing the broad utility of the platform. The unique combination of small size and very high affinity, which is only achievable with LNA-based drugs, allows this new class of drugs to potently and specifically

(more)

inhibit RNA targets in many different tissues without the need for complex delivery vehicles. LNA-based drugs are a promising new type of therapy that enables scientists to develop drugs to attack previously inaccessible pathways.

"Using our LNA Drug Platform to advance the first microRNA-targeted therapy into human clinical trials was certainly a scientific breakthrough," said Henrik Ørum, PhD, Vice President and Chief Scientific Officer of Santaris Pharma A/S. "We are extremely pleased with the results of the Phase I trials and excited to progress miravirsen into Phase 2 clinical trials. Because of its unique mechanism of action and tolerability profile, miravirsen has the potential to be an effective treatment option for patients with HCV."

The randomized, double-blind, placebo-controlled, ascending multiple-dose Phase 2a study will assess the safety and tolerability of miravirsen and is designed to enroll up to 55 treatment-naïve patients with chronic Hepatitis C virus genotype 1 infection. Secondary endpoints include pharmacokinetics of miravirsen and its effect on viral load. Miravirsen will be given as subcutaneous injections weekly or every other week for four weeks.

Data from Phase 1 clinical studies with miravirsen in healthy volunteers show that the drug is well tolerated. A recent study published in *Science* demonstrated that miravirsen successfully inhibited miR-122 and dramatically reduced Hepatitis C virus in the liver and in the bloodstream in chimpanzees chronically infected with the Hepatitis C virus[3]. Miravirsen provided continued efficacy in the animals up to several months after the treatment period with no adverse events and no evidence of viral rebound or resistance.

In addition to miravirsen, Santaris Pharma A/S has a robust product pipeline targeting mRNAs and microRNAs both internally as well as in partnerships and collaborations with miRagen Therapeutics (cardiovascular diseases), Shire plc (rare genetic disorders), Pfizer (undisclosed therapeutic areas), GlaxoSmithKline (viral disease) and Enzon Pharmaceuticals (oncology).

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 potent single-stranded LNA-based drug candidates against RNA targets, both mRNA and microRNA, for a range of diseases including metabolic disorders, infectious and inflammatory diseases, cancer and rare genetic disorders. 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, which is only achievable with LNA-based drugs, allows this new class of drugs to potently and specifically inhibit RNA targets in many different tissues without the need for complex delivery vehicles. LNA-based drugs are a promising new type of therapy that enables scientists to develop drugs to attack previously inaccessible clinical pathways. The most important features of LNA-based drugs include excellent specificity, providing optimal targeting; increased affinity to targets providing improved potency; and strong pharmacology upon systemic delivery without complicated delivery vehicles.

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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 metabolic 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.

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

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

[3] *Science*. 2010 Jan 8; 327(5962):198-201. Epub 2009 Dec 3

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