Santaris Pharma A/S advances a second drug from its cardiometabolic program, SPC4955, inhibiting apoB, into Phase 1 clinical trials for the treatment of high cholesterol

- ***Phase 1 clinical study to assess safety and tolerability of SPC4955, a drug inhibiting synthesis of apolipoprotein B (apoB), a major protein component involved in the formation of LDL-C or "bad" cholesterol***
- ***High cholesterol is a risk factor for cardiovascular disease and according to the World Health Organization, it is estimated to cause 18% of strokes and 56% of heart disease globally***
- ***In preclinical studies, SPC4955 potently and dose-dependently reduced apoB levels resulting in significant and durable reductions in plasma levels of LDL-C and triglycerides***
- ***Developed using Santaris Pharma A/S Locked Nucleic Acid Drug Platform, SPC4955 is the second to advance into Phase 1 clinical trials from the company's multi-faceted cardiometabolic program aimed at helping patients achieve target LDL-C levels***

Hoersholm, Denmark/San Diego, California, May 11, 2011 — Santaris Pharma A/S, a clinical-stage biopharmaceutical company focused on the research and development of mRNA and microRNA targeted therapies, today announced that it has advanced a second drug from its cardiometabolic program, SPC4955 into Phase 1 clinical trials, for the treatment of high cholesterol. Developed using Santaris Pharma A/S proprietary Locked Nucleic Acid (LNA) Drug Platform, SPC4955 is a mRNA-targeted drug candidate that inhibits apolipoprotein B (apoB), a major protein component involved in the formation of low density lipoprotein cholesterol (LDL-C) or "bad" cholesterol.

Cholesterol is an essential component of all cells and several important hormones, but cholesterol levels that are out of balance or too high overall lead to the formation of atherosclerotic plaques that cause cardiovascular diseases such as heart attacks or strokes. According to the World Health Organization, cardiovascular disease is the number one cause of death globally and high cholesterol is estimated to cause 18% of strokes and 56% of heart disease globally[1].

The randomized, dose-escalation, double-blind, placebo-controlled Phase 1 study will assess safety, tolerability, pharmacokinetics, and pharmacodynamics of SPC4955. The study aims to enroll 30 healthy volunteers who will be randomized to receive either subcutaneous injections of SPC4955 or placebo. In preclinical studies, SPC4955 potently and dose-dependently inhibited the synthesis of apoB resulting in significant and durable reductions in plasma levels of LDL-C and triglycerides[2].

Last week, Santaris Pharma A/S announced it had advanced another drug candidate, SPC5001, a mRNA-targeted drug inhibiting the synthesis of Proprotein Convertase Subtilisin/Kexin type 9 (PCSK9), for the treatment of high cholesterol into Phase 1 clinical trials. PCSK9 is a protein involved in removing low density lipoprotein cholesterol (LDL-C) or "bad" cholesterol from the bloodstream.

"Advancing two drug candidates, SPC4955 and SPC5001, in the space of only a week from our cardiometabolic program into the clinic is a testament to the efficiency of our LNA Drug Platform and Drug Discovery Engine capabilities and represents an important step for patients as multiple approaches are needed to lower high cholesterol levels," said Arthur A. Levin, PhD, Vice President and Chief Development Officer of Santaris Pharma A/S. "SPC4955 aims to inhibit the production and export of LDL-C in the liver while our other clinical candidate SPC5001, increases the clearance of LDL from the blood. Working on two distinct compounds with different target pathways allows the company to address the broad spectrum of patients trying to control their high cholesterol levels."

(more)

In addition to its cardiometabolic programs, in September 2010, Santaris Pharma A/S successfully advanced miravirsen, a lead microRNA drug candidate targeting miR-122, into Phase 2 studies for the treatment of patients infected with the Hepatitis C virus.

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 for a range of diseases including metabolic disorders, infectious and inflammatory diseases, cancer and rare genetic disorders.

With its partners, Santaris Pharma A/S also has a robust product pipeline consisting of mRNA and microRNA drug discovery and development collaborations. These include partnerships with Pfizer, Inc. (delivery of lead candidates against up to 20 targets), miRagen Therapeutics (cardiovascular diseases), Shire plc (rare genetic disorders), GlaxoSmithKline (four viral disease drug candidates) and Enzon Pharmaceuticals (eight cancer targets successfully delivered – three are now in Phase 1 clinical studies).

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.

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[1] World Health Organization - http://www.who.int/healthinfo/statistics/bod_cerebrovasculardiseasestroke.pdf
[2] Santaris Pharma A/S in-house data