Santaris Pharma A/S advances RNA-targeted drug development candidate against PCSK9, an important new target for the treatment of high cholesterol

- ***Santaris Pharma A/S selects lead research compound SPC5001 targeting PCSK9 to advance into drug development for the treatment of high cholesterol***
- ***PCSK9, an important new target, is a protein involved in regulating LDL or "bad" cholesterol in the blood– a major risk factor for coronary heart disease, heart attack and stroke***
- ***Preclinical data presented at PCSK9 Conference show SPC5001 provided potent, specific and long-lasting inhibition of PCSK9 and lowered LDL cholesterol in primates by up to 74%***
- ***Versatility and broad utility of Santaris Pharma A/S LNA Drug Platform and Drug Discovery Engine critical in developing effective RNA-targeted therapies for a range of diseases***

Hoersholm, Denmark/San Diego, California, April 26, 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 into drug development a discovery research candidate directed against PCSK9 (proprotein convertase subtilisin/kexin type 9), an important new target for the treatment of high cholesterol. Using its proprietary Locked Nucleic Acid (LNA) Drug Platform and Drug Discovery Engine, Santaris Pharma A/S identified and advanced the new drug, SPC5001, in just 18 months.

High cholesterol is a major risk factor for coronary heart disease, heart attack and stroke. According to the World Health Organization, high cholesterol is estimated to cause 18% of strokes and 56% of heart disease, globally and amounts to approximately 4.4 million deaths and 40.4 million disability-adjusted life years[1].

Santaris Pharma A/S presented preclinical data on SPC5001 last month at the PCSK9 Conference "From gene to therapeutics" in Nantes, France where many top pharmaceutical companies gathered to discuss efforts to develop therapies aimed at inhibiting the important new target PCSK9, a protein involved in regulating LDL (low-density lipoprotein) or "bad" cholesterol in the blood.

Data presented by Santaris Pharma A/S scientists showed that SPC5001 provided potent, specific and long-lasting inhibition of PCSK9 and lowered mean LDL cholesterol by 50% in non-human primates with a sustained reduction of 74% in the highest responder. SPC5001 did not change HDL (high-density lipoprotein) levels or the "good" cholesterol in the blood[2]. The preclinical data suggest that SPC5001 has the potential to provide patients with a new treatment option in managing their cholesterol levels.

"There is a lot of interest in PCSK9 as an exciting new target for the treatment of high cholesterol and having demonstrated that SPC5001 effectively lowers LDL or the "bad" cholesterol in preclinical studies, we are quickly moving forward with plans to advance SPC5001 into clinical studies as a potential new treatment to help patients better manage the risks of cardiovascular diseases," said Henrik Ørum, PhD, Vice President and Chief Scientific Officer of Santaris Pharma A/S. "SPC5001 is a testament to the efficiency of our LNA Drug Platform and Drug Discovery Engine as we discovered, optimized and advanced SPC5001 into preclinical development in just 18 months."

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.

(more)

"In addition to advancing SPC5001 into drug development for the treatment of high cholesterol, we remain on track to begin Phase 2 clinical trials of miravirsen (SPC3649) in patients with Hepatitis C in the second half of this year," said Søren Tulstrup, President and Chief Executive Officer, Santaris Pharma A/S. "In our internal programs and partnered programs, the versatility and broad utility of Santaris Pharma A/S LNA Drug Platform and Drug Discovery Engine continues to be critical in developing effective RNA-targeted therapies with high affinity, target specificity and remarkable potency for a range of diseases."

SPC5001 adds to the portfolio of Santaris Pharma A/S drugs in development, including its most advanced compound miravirsen, the International Nonproprietary Name (INN) or generic name for the compound formerly known as SPC3649.

Miravirsen, the first microRNA-targeted drug to enter human clinical trials, is a specific inhibitor of miR-122, a liver-specific microRNA that the Hepatitis C virus requires for replication. Unlike Hepatitis C therapies that directly target the virus, miravirsen works by removing a "helper" molecule (miR-122) that the virus needs in order to make new copies. Miravirsen is being studied in a Phase 1 trial multiple-ascending dose study in healthy volunteers and a Phase 2 study in patients infected with Hepatitis C is expected to begin in the second half of 2010.

Santaris Pharma A/S also has a broad range of drug discovery and development programs with its partners: Shire plc (lead candidates against up to five targets for rare genetic disorders), Pfizer (lead candidates against up to ten targets), GlaxoSmithKline (options to drug candidates from up to four viral disease programs) and Enzon Pharmaceuticals (lead candidates against eight cancer targets successfully delivered).

About Locked Nucleic Acid (LNA) Drug Platform

The LNA Drug Platform utilizing Santaris Pharma A/S proprietary LNA chemistry provides the key to delivering on the promise of RNA-targeted therapies today by addressing these concerns and overcoming 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.

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, infectious and inflammatory diseases, and rare genetic disorders. The Company has strategic partnerships with Enzon Pharmaceuticals, GlaxoSmithKline, Pfizer and Shire plc. As part of its broad patent estate, the Company holds exclusive worldwide rights to all therapeutic uses of LNAs. 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.

Media Contact:
Navjot Rai
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/healthinfo/statistics/bod_cerebrovasculardiseasestroke.pdf
[2] Oral and poster presentation PCSK9 Conference, Locked Nucleic Acid antisense oligonucleotide inhibition of PCSK9, March 11, 2010

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