Santaris Pharma A/S obtains exclusive license from Mass General Hospital for intellectual property related to the regulation of miR-33 for the treatment of cardiovascular disorders

- ***miR-33, an important microRNA that regulates high density lipoprotein (HDL) levels or "good" cholesterol, is a promising therapeutic target for raising good cholesterol levels***
- ***High levels of HDL protect against cardiovascular diseases, the number one cause of death globally; by 2030, almost 23.6 million people will die from cardiovascular diseases, mainly from heart disease and stroke***
- ***Data published in Science show that turning off miR-33 raised HDL levels suggesting miR-33 as a novel target in the treatment of cardiovascular and metabolic disorders***
- ***Santaris Pharma A/S, the first company to advance a microRNA-targeted drug into clinical trials, aims to develop LNA-based therapy targeting miR-33 for patients with cardiovascular disease***

Hoersholm, Denmark/San Diego, California, February 28, 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 the Company has obtained an exclusive license from Massachusetts General Hospital (MGH) for intellectual property related to the regulation of miR-33 for the treatment of cardiovascular disorders. Santaris Pharma A/S will utilize its Locked Nucleic Acid (LNA) Drug Platform to develop a LNA-based drug targeting miR-33, an important microRNA that regulates high density lipoprotein (HDL) levels or "good" 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 heart attacks or strokes. According to the World Health Organization, cardiovascular diseases are the number one cause of death globally and by 2030, almost 23.6 million people will die from cardiovascular diseases, mainly from heart disease and stroke[1].

"We are excited to have licensed the intellectual property related to the regulation of miR-33 from Mass General Hospital as it is a promising new target to raise HDL or the 'good' cholesterol in patients who are suffering from cardiovascular diseases," said Søren Tulstrup, President and CEO of Santaris Pharma A/S. "With our leadership position in microRNA drug development having advanced miravirsen into Phase 2 clinical trials and a keen focus on developing cardiovascular therapies, we are well positioned to expand our cardiovascular disease portfolio for the benefit of patients who are in need of new therapies to better control their cholesterol levels."

Last year, MGH researchers published data in *Science* identifying tiny segments of RNA, microRNAs, which play an important role in the body's regulation of cholesterol and lipids[2]. Their study found that the miR-33 family of microRNAs suppresses a protein known to be important for generation of HDL and for the removal of cholesterol from peripheral tissues, including cells that form atherosclerotic plaques. Data show that turning off miR-33 raises HDL levels suggesting miR-33 as a novel target in the treatment of cardiovascular and metabolic disorders.

"Current treatments for such cholesterol abnormalities as low circulating HDL levels are only modestly effective, and there is an urgent need for new therapeutic strategies," said Anders Näär, Ph.D., of the MGH Center for Cancer Research, who led the *Science* study. "Our discovery of miR-33 as a key regulator of HDL has provided a novel therapeutic target for antisense-based technologies to ameliorate cardiometabolic disorders."

(more)

The Santaris Pharma A/S LNA Drug Platform is the only RNA technology with both mRNA and microRNA targeted drugs in clinical trials, demonstrating the broad utility of the proprietary platform. 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. In addition, Santaris Pharma A/S is advancing two mRNA-targeted drugs, SPC5001 targeting PCSK9 and SPC4955 targeting apoB, for the treatment of high cholesterol into Phase 1 in the first half of 2011.

RNA-targeted drugs are a promising new class of therapeutics that are enabling scientists to develop drugs to work through targets thought to be inaccessible to small molecules and monoclonal antibodies. The LNA Drug Platform utilizing Santaris Pharma A/S proprietary single-stranded LNA chemistry may provide the key to delivering on the promise of RNA-targeted therapies today by overcoming the limitation of earlier antisense and siRNA technologies. The unique combination of small size and high affinity achievable with Santaris Pharma A/S LNA technology allows LNA-based drugs to potently and specifically inhibit RNA targets in different tissues without the need for complex delivery vehicles.

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 metabolic 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 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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[2] Najafi-Shoushtari et al. 2010. "MicroRNA-33 and the SREBP Host Genes Cooperate to Control Cholesterol Homeostasis", Science 328: 1566-9.