

## **Santaris Pharma A/S and miRagen Therapeutics Inc. expand worldwide strategic alliance to discover and develop LNA-drugs against disease causing microRNAs**

Hørsholm, Denmark / Boulder, Colorado USA, January 8, 2013. — Santaris Pharma A/S and miRagen Therapeutics, Inc. today announced that they have expanded their existing partnership in the field of microRNA therapeutics.

Under the expanded agreement, miRagen obtains a broad non-exclusive license in the miRNA therapeutics field for therapeutics research and world-wide exclusive rights to research, develop and commercialize LNA drugs against up to six additional microRNA targets that have been shown to be important in human disease areas of high unmet need. This brings the total number of microRNA targets under the agreement to ten. Santaris Pharma A/S will receive a combination of cash and equity in consideration for the licenses as well as clinical milestones and royalties on products emerging from the alliance. Financial terms were not disclosed.

Henrik Ørum, CSO and VP, Business Development at Santaris Pharma A/S stated: “Our collaboration with miRagen is an important part of our corporate strategy in the microRNA therapeutics field, so we are very pleased with our collective decision to expand the relationship. MicroRNA therapy is an exciting new branch of human medicine where the LNA drug platform has already provided proof-of-concept in phase2 clinical studies in HCV patient using Santaris’ miRNA-122 inhibitor, Miravirsen.”

William S. Marshall, President and CEO of miRagen stated: “Our partnership with Santaris has been very productive, and we are excited to expand the relationship to additional microRNA targets in our portfolio. Our goal is to identify important disease causing microRNAs and to deploy the world’s best technologies to create therapeutic development candidates against diseases with high unmet medical need. We hope these new programs will translate into novel drugs that will help patients suffering from serious diseases live longer and better lives.”

### **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 infectious and inflammatory diseases, cardiometabolic disorders, cancer and rare genetic disorders. LNA-based drugs are a promising new class of therapeutics that are enabling scientists to develop drug candidates that target 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 drug 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 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 contain LNA as an 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](http://www.santaris.com) for more information.

#### **About miRagen Therapeutics, Inc.**

miRagen Therapeutics, Inc. is a biopharmaceutical company focused on the discovery and development of innovative microRNA-based therapeutics in disease areas with high unmet medical need. The company leverages insights from leading laboratories to evaluate and advance high-potential therapies for its own pipeline or in conjunction with strategic partners. For several cardiovascular disease programs, miRagen has partnered with Les Laboratoires Servier, a leading European pharmaceutical company in the area of cardiovascular disease. miRagen retains all rights for the Servier-partnered programs in the United States and Japan. With its commercial and academic alliances, miRagen strives to harnesses the power of microRNA biology and chemistry by translating discoveries into breakthrough therapies that improve human health. For more information, please visit [www.miragenrx.com](http://www.miragenrx.com).

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