

## **Shire and Santaris extend their strategic alliance to discover and develop LNA-drugs in the rare genetic disease field**

**Hørsholm, Denmark / San Diego, California, August 23<sup>rd</sup>, 2013.** – Santaris Pharma A/S, a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced that its long term partner, Shire has extended the existing partnership in the rare genetic disease space.

Under the terms of the extended agreement, Shire will have the right to nominate additional collaboration targets for drug discovery and development. Santaris will receive an upfront payment and, consistent with the initial agreement, is eligible for research support, pre-clinical, clinical and sales milestones and royalties on each product emerging from the collaboration.

**Henrik Stage, President & CEO at Santaris Pharma A/S stated:** “Our collaboration with Shire is very important to us so we are very pleased with the decision to extend the agreement and allow for more drug discovery and development programs. We believe the LNA drug platform offers a unique opportunity to develop drugs against the rapidly expanding number of disease targets in the rare genetic disorder space”.

**Albert Seymour, VP, Discovery Research, Shire, stated:** “We are delighted with our partnership with Santaris, whose expertise and capabilities in LNA antisense therapy complement Shire’s drug discovery and development strengths. Our hope is that the partnership will eventually translate into novel drugs that will help patients suffering from debilitating rare diseases lead 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 cardiometabolic disorders, infectious and inflammatory diseases, cancer and rare genetic disorders. LNA is also sometimes referred to as BNA (Bicyclic or Bridged Nucleic Acid). 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 RaNA Therapeutics, Bristol-Myers Squibb, miRagen Therapeutics, Shire, Pfizer, GlaxoSmithKline, and Enzon Pharmaceuticals. As part of its broad patent estate, the company holds exclusive worldwide rights to manufacture and sell products that comprise LNA as 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.

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