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RNA Medicines for the 21st Century

## **RaNA Therapeutics, Inc. and Santaris Pharma A/S announce agreement to develop RNA-targeted medicines that selectively activate protein expression**

- RaNA Therapeutics licenses rights to utilize Santaris Pharma A/S proprietary Locked Nucleic Acid (LNA) Drug Platform to discover and develop RNA-targeted medicines that selectively activate protein expression against up to 10 of RaNA's proprietary RNA targets for the treatment of human diseases
- RaNA will apply single-stranded LNA-based technology to develop drug candidates in the fields of rare genetic disorders, inflammation, oncology, metabolic diseases and neurodegenerative diseases

**Hørsholm, Denmark/San Diego, California & Cambridge, Massachusetts, July 8, 2013** — Santaris Pharma A/S and RaNA Therapeutics, Inc. announced today that they have signed an agreement whereby RaNA gains access to Santaris' Locked Nucleic Acid (LNA) technology to develop RNA-targeted medicines. Under the terms of the agreement, Santaris will license its proprietary Locked Nucleic Acid drug technology to RaNA for the development of LNA-based drug candidates against up to 10 RaNA proprietary RNA targets. The license granted by Santaris includes access to proprietary chemistry capable of delivering potent single-stranded LNA-based drug compounds. RaNA will apply the LNA chemistry along with its proprietary technologies for selective protein activation to develop RNA-targeted medicines. Santaris will receive an upfront payment and is eligible for target nomination payments, development milestones and royalties on each product.

Henrik Ørum, CSO & VP Business Development of Santaris Pharma A/S, said "Santaris Pharma's LNA drug platform provides the means to discover and develop novel drugs that potently and specifically bind RNA-targets in previously inaccessible clinical pathways. Usually, the objective is to inhibit the target to avoid protein synthesis, but in RaNA's case the LNA drugs will be developed to selectively activate protein expression. As such, our agreement with RaNA is yet another example of how we seek to leverage the broad applicability of our drug platform through partnerships with the pharmaceutical industry."

"We are delighted to gain access to the Santaris LNA chemistry to augment our drug development programs," said Arthur M. Krieg, MD, President and CEO of RaNA Therapeutics. "We believe the LNA



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chemistry has important properties to contribute to RaNA's proprietary approach to selectively express therapeutic proteins."

### **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 chemistries 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 Bristol-Myers Squibb, 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 RaNA Therapeutics, Inc.**

RaNA Therapeutics is pioneering the discovery of a new class of medicines that target RNA to selectively activate protein expression within the body's own cells. RaNA's novel drugs work by targeting specific genes to precisely upregulate the expression of beneficial proteins. The company's leadership leverages preeminent founders and advisors in the field, and the strength of its fundamental patents, technology, and know-how that underlie the discovery and development of therapeutics that selectively activate RNA. RaNA is applying its proprietary technology to rapidly advance a pipeline of novel drugs for diverse therapeutic areas, with an initial emphasis in the fields of rare genetic disorders, inflammation, oncology, metabolic diseases and neurodegenerative diseases. For more information about the company and its drug platform, please visit [www.ranarx.com](http://www.ranarx.com).

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