



Alnylam Reports Progress for Systemic Delivery of RNAi Therapeutics; Results Presented at 2nd International Symposium on Triglycerides and HDL

CAMBRIDGE, Mass.--(BUSINESS WIRE)--July 18, 2005--Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), a leading RNAi therapeutics company, today announced further progress on its research in systemic delivery of RNAi therapeutics targeting apolipoprotein B (apoB), a disease-associated gene in hypercholesterolemia, which it presented at the 2nd International Symposium on Triglycerides and HDL in New York. Alnylam scientists reported an improved dosage regimen for the systemic delivery of short interfering RNAs (siRNAs) that silence the gene for apoB. The siRNAs, the molecules that mediate RNA interference (RNAi), were chemically modified and optimized by Alnylam researchers. Further reported data included the demonstration of siRNA-mediated therapeutic efficacy in an animal model of hypercholesterolemia.

"These data represent important progress in the advancement of systemic RNAi therapeutics towards clinical development," said John Maraganore, Ph.D., President and Chief Executive Officer of Alnylam Pharmaceuticals. "We have been able to demonstrate silencing of an endogenous, disease-associated gene using a chemically modified and optimized siRNA at significantly lower doses than in our previous landmark study."

The new pre-clinical dosing studies in mice used a lower daily dose regimen (5 mg/kg) of the systemically delivered siRNA to achieve a 60% silencing of apoB in the target organ. Overall doses of siRNA in this regimen were 6-fold lower than those published by Alnylam scientists in *Nature* in November 2004. Alnylam's *Nature* article was the first-ever published in vivo demonstration of RNAi-mediated silencing of an endogenous gene in mammals by a method applicable to systemic RNAi therapeutics for human disease. Systemic delivery of siRNAs through the bloodstream potentially enables RNAi therapeutics to be developed for a broad range of diseases.

About RNA Interference (RNAi)

RNA interference, or RNAi, is a naturally occurring mechanism within cells for selectively silencing and regulating specific genes. Since many diseases are caused by the inappropriate activity of specific genes, the ability to silence and regulate such genes selectively through RNAi could provide a means to treat a wide range of human diseases. The discovery of RNAi has been heralded by many as a major breakthrough, and the journal *Science* named RNAi the top scientific achievement of 2002, as well as one of the top 10 scientific advances of 2003.

About Alnylam

Alnylam is a biopharmaceutical company seeking to develop and commercialize novel therapeutics based on RNA interference, or RNAi. Growing from its foundation as the world's first company focused on RNAi therapeutics, the company's leadership in the field of RNAi is supported by its preeminent founders and advisors and its strengths in fundamental patents, technology, and know-how that underlie the commercialization of RNAi therapeutics. Alnylam is developing a pipeline of RNAi products using Direct RNAi™ to treat ocular, central nervous system, and respiratory diseases and Systemic RNAi™ to treat a broad range of diseases, including oncology, metabolic, and autoimmune diseases. The company's global headquarters are in Cambridge, Massachusetts. For additional information, please visit www.alnylam.com.

Alnylam Forward-Looking Statements

Various statements in this release concerning our future expectations, plans, prospects and future operating results constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including risks related to: our approach to discover and develop novel drugs, which is unproven and may never lead to marketable products; our ability to obtain additional funding to support our business activities; our dependence on third parties for development, manufacture, marketing, sales and distribution of our products; the successful development of products, all of which are in early stages of development; obtaining regulatory approval for products; competition from others using technology similar to ours and others developing products for similar uses; obtaining, maintaining and protecting intellectual property utilized by our products; and our short operating history; as well as those risks more fully discussed in the "Certain Factors That May Affect Future Results" section of our most recent Form 10-Q filed with the Securities and Exchange Commission. In addition, any forward-looking statements represent our views only as of today and should not be relied upon as representing our views as of any subsequent date. We do not assume any obligation to update any forward-looking statements.

SOURCE: Alnylam Pharmaceuticals, Inc.

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