



Cystic Fibrosis Foundation Therapeutics and Alnylam Initiate Collaboration to Discover RNAi Therapeutic to Treat Cystic Fibrosis

Cystic Fibrosis Foundation Therapeutics to Provide \$1.5 Million

CAMBRIDGE, Mass. and BETHESDA, Md., Mar 16, 2005 (BUSINESS WIRE) -- Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), the drug discovery and development affiliate of the Cystic Fibrosis Foundation (CFF), and Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY), a leading RNAi therapeutics company, announced today that they have initiated a collaborative program to discover Direct RNAi™ therapeutics for the treatment of cystic fibrosis (CF). Alnylam will apply RNAi technology toward the discovery of short interfering RNAs (siRNAs), the molecules that induce RNA interference, to restore protein function in CF. CFFT is expected to provide Alnylam with \$1.5 million in up front and milestone-driven funding for the discovery effort, along with introductions to world-class CF academic researchers and access to critical research resources.

Cystic fibrosis is a genetic disease affecting approximately 30,000 children and adults in the United States. A defective gene causes the body to produce an abnormally thick, sticky mucus that obstructs the lungs, leading to life-threatening lung infections, and obstructs the pancreas, causing difficulty absorbing food. The median life expectancy has improved from early childhood to the mid-30s today, but many individuals battle lung disease for years.

"Existing drugs for cystic fibrosis focus on the symptoms and complications of the disease, such as preventing infections, reducing the quantity and thickness of secretions in the lungs, and improving breathing and digestion. We hope a potential therapeutic will result from this groundbreaking technology to treat the cause of the disease," said Robert J. Beall, Ph.D., president and CEO of the CF Foundation and CFFT. "We are very pleased to collaborate with Alnylam, a leader in the discovery and development of RNAi therapeutics, to focus on CF."

This genetic disease is marked by defects in a protein, known as the Cystic Fibrosis Transmembrane conductance Regulator (CFTR) protein, required for proper transport of salt and water across the cell membrane. Defects in this protein lead to thick, sticky mucus that clogs the lungs and digestive system. In most patients, potentially functional CFTR protein is made but does not reach the cell surface. CFFT and Alnylam aim to determine whether this protein can be redirected to the cell surface by using a potential RNAi therapeutic to silence specific genes. If so, the goal will be to develop this potential RNAi therapeutic as a treatment for CF patients.

"The opportunity to harness RNAi to treat CF represents an important strategy in the application of our technology for discovering novel medicines and making a difference in patients' lives," said John Maraganore, Ph.D., president and CEO of Alnylam Pharmaceuticals. "The ability to collaborate with a strong foundation, such as the CF Foundation and its affiliate, CFFT, allows us to address this unmet medical need. We believe applying our technology to this disease represents an outstanding opportunity for Alnylam and look forward to bringing the potential fruits of this collaboration to patients."

About the Cystic Fibrosis Foundation and CFFT

The mission of the Cystic Fibrosis Foundation is to assure the development of the means to cure and control CF and to improve the quality of life for those with the disease. CFFT is the non-profit drug discovery and development affiliate of the CF Foundation. CFFT supports and governs activities related to cystic fibrosis (CF) drug discovery through drug development and clinical evaluation. The CF Foundation provides support to fund CFFT's operations, specifically the Therapeutics Development Program. For more information about CF, the CF Foundation or CFFT, call (800) FIGHT CF or visit www.cff.org.

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 Pharmaceuticals, Inc.

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 auto immune diseases. The company's global headquarters are in Cambridge, Massachusetts. For additional information, please visit www.alnylam.com.

Alnylam Forward Looking Statement

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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