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Alnylam, Genzyme enter gene silencing deal

23 October 2012 | News | By BioSpectrum Bureau

Alnylam partners with Genzyme to commercialize RNAi therapeutics in Asia

Singapore: Alnylam Pharmaceuticals and Genzyme, a Sanofi company, announced that they have formed an exclusive alliance to develop and commercialize RNAi therapeutics targeting transthyretin (TTR) for the treatment of transthyretin-mediated amyloidosis (ATTR) in Japan and other Asia Pacific countries. ATTR is a rare, debilitating, hereditary disease that damages the nervous system and heart, resulting in a life expectancy of five-to-15 years.

"Our ALN-TTR program holds promise as a breakthrough therapy for the treatment of ATTR, a debilitating orphan disease. As the lead program in our 'Alnylam 5x15' product strategy, we also view this program as a key part of building Alnylam for the future," said Dr John Maraganore, CEO, Alnylam.

"In this important collaboration, Genzyme will advance our ALN-TTR program with their proven capabilities in the Japanese and broader Asian market, while we maintain our plans to develop and commercialize this potential breakthrough medicine in the US, Europe, and rest of world. In addition, a key part of the value proposition in this alliance for Alnylam is the potential for significant royalty payments on sales of products."

Under the terms of the agreement, Genzyme will make an upfront cash payment of $22.5 million to Alnylam. The agreement also includes development milestone payments and tiered royalties expected to yield an effective rate in the mid-teens to mid-

twenties on Genzyme's sales of ALN-TTR products in their territory. In addition, each party will be responsible for the development and commercialization activities in their respective territories.

Recently, Alnylam presented positive clinical results from its ALN-TTR02 Phase I trial demonstrating robust and unprecedented knockdown of serum TTR protein levels of up to 94%; the overall results were highly significant ($p < 0.00001$ by ANOVA). Suppression of TTR, the disease-causing protein in ATTR, was found to be rapid, dose dependent, durable, and specific after just a single dose. The drug was generally safe and well tolerated in this Phase I study. Alnylam is currently enrolling patients in a Phase II multi-dose study of ALN-TTR02 in ATTR patients and aims to initiate a Phase III pivotal study of ALN-TTR02 by the end of 2013.