Alnylam data supports 'silencing' of gene linked to Parkinson's

Mass High Tech: The Journal of New England Technology

Alnylam Pharmaceuticals [1] Inc. is releasing early data that shows its technology may be able to “silence” a gene associated with Parkinson’s disease.

The Cambridge-based biotechnology company RNA1, along with collaborators at The Parkinson’s Institute and the Mayo Clinic have published their research findings in the journal Public Library of Science (PLoS). The new data shows effective silencing of the alpha-synuclein gene with an RNAi therapeutic, in a pre-clinical trial, testing the technology on a group of non-human primates. Alpha-synuclein is widely believed to play a central role in the development of Parkinson’s disease.

RNAi prevents genes from producing toxic proteins by binding to and destroying targeted messenger RNA molecules to silence the bad gene. Researchers believe shutting off these genes could treat a variety of diseases.

“These new findings add to a growing body of data on the applications of RNAi therapeutics for the treatment of neurodegenerative disorders, such as Parkinson’s disease. Indeed, direct delivery of RNAi therapeutics in the CNS (central nervous system) represents an important component of our overall product development strategy,” said David Bumcrot, director of Research at Alnylam, in a statement. “We remain committed to advancing this promising therapeutic modality to patients.”

Recent research indicates that at least one million people in the United States, and more than 5 million worldwide, suffer from Parkinson’s disease.

SOURCE [2]

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