



FOR IMMEDIATE RELEASE

BREAKING NEWS: STRATEGY TO FIND A CURE FOR HUNTINGTON'S DISEASE, LONG-SUPPORTED BY HEREDITARY DISEASE FOUNDATION, IS VALIDATED BY NOBEL PRIZE!

*Congratulations to Andrew Z. Fire and Craig C. Mello,
Scientists Who Discovered RNA Interference
Awarded 2006 Nobel Prize in Physiology or Medicine*

The Nobel Prize:

On October 2, 2006, the Nobel Assembly at Karolinska Institute in Stockholm, Sweden, announced the 2006 Nobel Prize in Physiology or Medicine will be awarded jointly to Andrew Z. Fire, Ph.D., Professor of Pathology and Genetics, Stanford University School of Medicine, Stanford, CA, and Craig C. Mello, Ph.D., Professor of Molecular Medicine and Howard Hughes Medical Institute Investigator, Program in Molecular Medicine, University of Massachusetts Medical School, Worcester, MA.

The Discovery:

Fire and Mello were awarded the Nobel Prize for their discovery of an efficient and generalizable method for selectively turning off the expression of any gene in the nematode worm *C. elegans*. The method is known as RNA interference or RNAi. Fire and Mello published their groundbreaking discovery in the journal, *Nature*, in February of 1998. Since that time, RNAi has been shown to be an effective method for turning off the expression of genes in a wide variety of plant and animal species, including humans. The RNAi technique is now widely used in academic and industrial research laboratories to study the function of genes and plans are underway to develop RNAi-based treatments for a wide variety of diseases, including Huntington's disease (HD).

The Significance for Huntington's Disease:

A gene is comprised of a linear stretch of DNA. When a gene is "expressed," the DNA is first copied into a messenger RNA which is then translated into a linear stretch of amino acids, forming a specific protein. In the vast majority of people with HD, the disease is caused by an inherited difference (the "HD mutation") in the DNA of a specific gene known as the HD gene. The protein generated from expression of an HD gene containing the HD mutation has abnormal properties that are toxic to brain cells, thereby causing the disease. RNAi directed at the HD gene works by turning off the expression of the HD gene, would prevent the production of the toxic protein. Assuming that this can be done effectively and that turning off the expression of the HD gene is safe, RNAi is expected to be a cure for HD.

The Hereditary Disease Foundation's Leadership in RNAi Therapy Development:

Spurred by the excitement generated by Fire and Mello's discovery, the Hereditary Disease Foundation began to organize, champion and support the development of gene-based therapy for HD. In early 2001, the HDF provided the first funding for research aimed at the development of RNAi-based treatments for HD. In 2002, the Foundation held a two-day workshop on RNAi-based treatments with leading experts in the field. (See article in HDF Summer 2003 newsletter by HDF President, Nancy Wexler, entitled "RNA to Our Rescue!!" www.hdfoundation.org/news/Summer03/Summer03_news.pdf and the workshop report: www.hdfoundation.org/workshops/200212Report.php.) More recently, at its biennial symposium "HD 2006: Changes, Advances and Good News (CAG)n" in Cambridge, MA, a full data session presentation was devoted to the latest developments in RNAi therapy development and safety testing.

In addition, in January of 2006, the HDF provided significant research funding to Dr. Beverly Davidson, the Roy J. Carver Professor of Internal Medicine at the University of Iowa College of Medicine and to Dr. Krystof Bankiewicz, Professor of Neurosurgery at the University of California, San Francisco. Drs. Davidson and Bankiewicz are developing methods for delivering RNAi-based therapy to the brain and testing the safety in animal models of an RNAi-based therapeutic product being developed for Huntington's disease by Sirna Therapeutics and Targeted Genetics Corporation (www.sirna.com/wt/page/neurology).

Dr. Davidson had earlier published groundbreaking research on RNAi therapy for HD in the April 19, 2005 issue of the Proceedings of the National Academy of Sciences. The results showed that delivery of RNAi to the brain of mice expressing the abnormal HD gene has a significant beneficial impact on the symptoms and progression of the disease. Dr. Bankiewicz is an expert on delivery of therapeutic treatments into the brain using specially shaped catheters and delivery protocols.

Alnylam Pharmaceuticals Inc., another company focused on the development of RNAi-based therapeutics, is also working on an HD therapy in collaboration with Medtronic, Inc. (www.alnylam.com/collaborations).

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