



The ALS Association
National Office

News Release

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New ‘Antisense’ Experimental Therapy For ALS Clinical Trial Announced

CALABASAS HILLS, Calif. (October 16, 2009) — A new experimental therapy using an approach known as antisense, in which a drug is designed to shut down the RNA (Ribonucleic acid) that is responsible for the production of disease-causing proteins, is being prepared for a clinical trial in people with a familial form of ALS later this year. The clinical trial follows research funded by The ALS Association through TREAT ALS (Translational Research Advancing Therapy for ALS), our research pipeline that funds and facilitates the development of treatments for ALS based on important laboratory findings.

The research that resulted in the identification of this antisense drug was first funded by The Association in 2003, and has been developed for the clinic through an academic/industry partnership. ALS Association-funded researchers Drs. Don Cleveland Richard Smith and Timothy Miller, in partnership with Isis Pharmaceuticals in Carlsbad, Calif., initiated experiments in a rat model of ALS to determine whether reducing the amount of SOD1 protein may be beneficial in treating the disease.

Initial research in rat ALS disease models demonstrated that the antisense drug inhibited the mutant SOD1 protein, resulting in prolonged life of the rats. Time of treatment for the rats was near onset of symptoms, reflecting the scenario for actual patients who often have definite and even advanced signs of motor neuron loss by the time of ALS diagnosis. Researchers hope that this therapeutic approach will provide a similar therapeutic benefit in people with familial ALS due to mutations in the SOD1 protein. The antisense approach could also prove valuable in treating other neurological disorders, such as Huntington’s disease.

Together with the biotech company Isis, led by Dr. Frank Bennett, Dr. Timothy Miller, Dr. Merit Cudkowicz and Dr. Richard Smith, the team has conducted the necessary research to submit an Investigational New Drug Application with the Food and Drug Administration (FDA) to test this novel approach in people with ALS. The application was recently submitted to the FDA. The Association will provide funding for the clinical trial.

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“This achievement, and the process of taking an idea from the laboratory to the clinic, underscores the importance of The Association’s TREAT ALS pipeline and the financial support provided to the investigators,” commented Senior Vice President, Research and Development Lucie Bruijn, Ph.D. “The development of new treatments is an extremely challenging and costly process. It is only through the support of our generous donors that this type of research is made possible.”

The Association’s research program brings together the best scientific minds from the research and biotech communities to focus on finding the cause of ALS, developing effective treatments, and ultimately, a cure. We are currently funding more than 80 studies around the world, partnering with the best minds in the scientific and biotech communities. To learn more about how you can support The Association’s premier ALS research program and studies such as the antisense clinical trial, visit our website at www.alsa.org.

People interested in learning more about the clinical study should contact the MGH Neurology Clinical Trial Unit at (877) 458-0631 or by email at mghneuroclinicaltrialsunit@partners.org.

The ALS Association is the only non-profit organization fighting Lou Gehrig’s Disease on every front. By leading the way in global research, providing assistance for people with ALS through a nationwide network of chapters, coordinating multidisciplinary care through certified clinical care centers, and fostering government partnerships, The Association builds hope and enhances quality of life while aggressively searching for new treatments and a cure.

For more information about The Association, visit the organization’s Web site at www.alsa.org or call (800) 782-4747.

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