

# Isis Announces ISIS 333611 Granted Orphan Drug Status for Treatment of ALS

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CARLSBAD, Calif., Dec 13, 2007 /PRNewswire-FirstCall via COMTEX News Network/ -- Isis Pharmaceuticals, Inc. (Nasdaq: ISIS) announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug status to ISIS 333611 for the treatment of an inherited form of amyotrophic lateral sclerosis (ALS), commonly referred to as Lou Gehrig's disease. ALS is a progressive neurodegenerative disease resulting in the loss of muscle control that can lead to death. ISIS 333611, a second-generation antisense drug, is Isis' first drug treating neurodegenerative diseases to enter development and will be administered directly into the cerebral spinal fluid.

"ALS is a terrible disease in need of innovative and efficacious new therapies. We are pleased that ISIS 333611 has been granted orphan drug status for this small but important patient population. In our preclinical studies, we demonstrated that direct delivery of an antisense drug onto the spinal fluid via an implantable pump effectively reduced SOD1, a protein associated with the inherited form of ALS," said C. Frank Bennett, Ph.D., Senior Vice President of Research at Isis Pharmaceuticals, Inc. "Our preclinical success with ISIS 333611 demonstrates the applicability of antisense technology to treat a variety of diseases that affect the central nervous system. In addition, with funding from CHDI, we recently initiated a separate drug discovery program in Huntington's Disease, another fatal neurodegenerative disease."

## ABOUT ISIS 333611

ISIS 333611 is a second-generation antisense drug in development for the treatment of ALS through the inhibition of Cu/Zn superoxide dismutase (SOD1), a molecule that is associated with an inherited, aggressive form of ALS. In preclinical rodent studies, inhibition of SOD1 extended survival of rats that show many features of ALS. The ALS Association and the Muscular Dystrophy Association are providing funding for IND-enabling studies of ISIS 333611.

## About Orphan Drug Designation

The Orphan Drug Act provides for economic incentives to encourage the development of drugs for diseases affecting fewer than 200,000 people in the United States. Orphan drug designation entitles Isis Pharmaceuticals to seven years of U.S. market exclusivity for ISIS 333611 for the treatment of patients with familial ALS.

## ABOUT ISIS PHARMACEUTICALS, INC.

Isis is exploiting its expertise in RNA to discover and develop novel drugs for its product pipeline and for its partners. The Company has successfully commercialized the world's first antisense drug and has 18 drugs in development. Isis' drug development programs are focused on treating cardiovascular and metabolic diseases. Isis' partners are developing antisense drugs invented by Isis to treat a wide variety of diseases. Ibis Biosciences, Inc., Isis' wholly owned subsidiary, is developing and commercializing the Ibis T5000(TM) Biosensor System, a revolutionary system to identify infectious organisms. Isis is a joint owner of Regulus Therapeutics LLC, a joint venture focused on the discovery, development and commercialization of microRNA therapeutics. As an innovator in RNA-based drug discovery and development, Isis is the owner or exclusive licensee of over 1,500 issued patents worldwide. Additional information about Isis is available at <http://www.isispharm.com>.

This press release includes forward-looking statements regarding the development, therapeutic potential and safety of ISIS 333611. Any statement describing Isis' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement, including those statements that are described as Isis' goals. Such statements are subject to certain risks and uncertainties, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such products. Isis' forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Isis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Isis. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Isis' programs are described in additional detail in Isis' annual report on Form 10-K for the year ended December 31, 2006, and its quarterly report on Form 10-Q for the quarter ended September 30, 2007, which are on file with the SEC. Copies of these and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Isis," "Company," "we," "our," and "us" refers to Isis Pharmaceuticals and its subsidiaries.

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