Isis Announces Positive Opinion on European Orphan Drug Designation for ISIS-TTR Rx

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CARLSBAD, Calif., Feb. 14, 2014 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that the Committee for Orphan Medicinal Products (COMP) has adopted a positive opinion recommending ISIS-TTR$_{Rx}$ for designation as an orphan medicinal product for the treatment of ATTR-Amyloidosis to the European Commission (EC). The opinion will be subject to review by the EC, which ultimately grants the decision on orphan drug designation. ATTR-Amyloidosis, or TTR amyloidosis, is a severe and rare genetic disease characterized by progressive dysfunction of peripheral nerve and/or heart tissues. ISIS-TTR$_{Rx}$ is an antisense drug in development with GlaxoSmithKline (GSK), which has an option to exclusively license the ISIS-TTR$_{Rx}$ program. ISIS-TTR$_{Rx}$ is currently being evaluated in a Phase 2/3 study in familial amyloid polyneuropathy (FAP) patients.

(Logo: http://photos.prnewswire.com/prnh/20130807/LA60006LOGO)

"We are encouraged by COMP's continued recognition of the need for new treatments for rare and orphan diseases and the designation of our drugs as potential therapeutic options. Since the beginning of the year, we have received two positive opinions on European Orphan Drug Designation, first for ISIS-APOCIII$_{Rx}$, our novel triglyceride-lowering drug, and now for ISIS-TTR$_{Rx}$," said B. Lynne Parshall, chief operating officer at Isis. "Our Phase 2/3 study for ISIS-TTR$_{Rx}$ is underway with some patients treated for more than six months. FAP is a devastating life-threatening disease and patients with FAP have limited therapeutic options. We remain encouraged about the potential for ISIS-TTR$_{Rx}$ to provide therapeutic benefit for these patients in need."

The COMP, a committee of the European Medicines Agency, adopts an opinion on the granting of orphan drug designation, after which the opinion is submitted to the European Commission for endorsement of the opinion. Orphan drug designation is granted to products designed to diagnose, prevent or treat life-threatening or very serious conditions that affect not more than five in 10,000 persons in the European Union.

Transthyretin amyloidosis is a genetic disease in which the patient inherits a mutant gene that produces a misfolded form of TTR, which progressively accumulates in tissues, impairing their function. In patients with transthyretin amyloidosis, both the mutant and normal forms of TTR can build up as fibrils in tissues, including heart, peripheral nerves, and the gastrointestinal tract. The presence of TTR aggregates interferes with the normal functions of these tissues, and as the TTR protein aggregates enlarge more tissue damage occurs and the disease worsens. The two most common types of TTR amyloidosis are familial amyloid cardiomyopathy, or FAC, which affects more than 40,000 patients worldwide, and familial amyloid polyneuropathy, or FAP, which affects more than 10,000 patients worldwide. ISIS-TTR$_{Rx}$ is an investigational drug that is designed to inhibit the production of all forms of TTR, and could potentially offer an alternative approach to treat all types of transthyretin-related amyloidosis.

ABOUT ISIS PHARMACEUTICALS, INC.
Isis is exploiting its leadership position in antisense technology to discover and develop novel drugs for its product pipeline and for its partners. Isis' broad pipeline consists of 31 drugs to treat a wide variety of diseases with an emphasis on cardiovascular, metabolic, severe and rare diseases, including neurological disorders, and cancer. Isis' partner, Genzyme, is commercializing Isis' lead product, KYNAMRO®, in the United States for the treatment of patients with HoFH. Isis' patents provide strong and extensive protection for its drugs and technology. Additional information about Isis is available at www.isispharm.com.

ISIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT
This press release includes forward-looking statements regarding Isis' collaboration with GlaxoSmithKline, the discovery, development and potential of drugs for severe and rare diseases, and the development, activity, therapeutic potential and safety of ISIS-TTR$_{Rx}$. 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. 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 drugs. 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, 2012, and its most recent quarterly report on Form 10-Q, 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.

SOURCE Isis Pharmaceuticals, Inc.

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