Isis Announces Positive Decision on European Orphan Drug Designation for ISIS-APOCIII Rx

January 13, 2014

CARLSBAD, Calif., Jan. 13, 2014 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that the Committee for Orphan Medicinal Products (COMP) has issued a positive opinion for Orphan Drug Designation for ISIS-APOCIII$_{Rx}$ for the treatment of patients with familial chylomicronemia syndrome (FCS). FCS is a rare orphan disease characterized by extremely high triglyceride levels that affects an estimated 3,000 to 5,000 patients worldwide. ISIS-APOCIII$_{Rx}$ is a wholly owned drug that Isis has evaluated in a broad Phase 2 program. In these studies, treatment with ISIS-APOCIII$_{Rx}$ substantially lowered triglycerides in patients with very high to extremely high triglycerides with reductions of more than 1,500 mg/dL observed in FCS patients.

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"FCS is a rare and very serious genetic disorder that is often associated with triglyceride levels higher than 2,000 mg/dL. Orphan drug designation for FCS underscores the need for improved therapies to treat patients with FCS. Because of their extremely high levels of triglycerides, FCS patients are at significant risk of many serious health conditions, including frequent episodes of pancreatitis, which can require hospitalization and be life-threatening. Current treatment options do not reduce triglyceride levels enough to reduce the risk of serious illness in patients with FCS," said Richard Geary, Ph.D., senior vice president of development at Isis. "In our Phase 2 studies patients treated with ISIS-APOCIII$_{Rx}$ achieved statistically significant reductions of apoC-III and triglycerides and statistically significant increases in HDL-cholesterol when ISIS-APOCIII$_{Rx}$ was administered as a single agent or in combination with fibrates in patients with a wide range of incoming triglycerides, including FCS patients. Orphan drug designation for ISIS-APOCIII$_{Rx}$ is an important benchmark as we rapidly move this drug toward Phase 3 initiation this year."

The COMP, a committee of the European Medicines Agency, administers the granting of orphan drug designation. 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.

ISIS-APOCIII$_{Rx}$ is an antisense drug intended to treat patients with severely high triglycerides either as a single agent or in combination with other triglyceride-lowering agents. ISIS-APOCIII$_{Rx}$ targets apoC-III, a protein produced in the liver that plays a central role in the regulation of serum triglycerides. Isis is developing ISIS-APOCIII$_{Rx}$ to treat patients with FCS and patients with severely elevated triglycerides. FCS is a rare genetic disorder associated with extremely high triglyceride levels, often triglyceride levels that are higher than 2,000 mg/dL, that affects an estimated 3,000 to 5,000 patients worldwide. As a result of their severely high triglyceride levels, FCS patients are at risk of many serious health conditions, including recurrent acute pancreatitis that often requires hospitalization, enlargement of the liver and spleen, abdominal pain and eruptive fatty skin lesions. Isis is also developing ISIS-APOCIII$_{Rx}$ for patients with severely elevated triglycerides, greater than 880 md/dL, that affects an estimated 50,000 patients in the United States and Europe. These patients have a high risk of pancreatitis but also have a high risk of type 2 diabetes and cardiovascular disease. For both indications, current therapies do not reduce triglyceride levels enough to decrease the risk of serious health conditions.

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 the discovery, development, activity, therapeutic potential and safety of ISIS-APOCIII$_{Rx}$. 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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