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## Isis Announces That ISIS-TTR<sub>Rx</sub> Has Been Granted Fast Track Designation For Treatment Of Patients With FAP

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Isis Pharmaceuticals, Inc. announced on 13 December that the US Food and Drug Administration (FDA) has granted ISIS-TTR<sub>Rx</sub> fast track designation for the treatment of familial amyloid polyneuropathy (FAP).

ISIS-TTR<sub>Rx</sub> is an antisense drug in development with GlaxoSmithKline (GSK) for the treatment of transthyretin (TTR) amyloidosis, a severe and rare genetic disease characterised by progressive dysfunction of peripheral nerve and/or heart tissues. Isis and GSK recently amended the clinical development plan and financial terms relating to ISIS-TTR<sub>Rx</sub> to support a registration-directed Phase two/three clinical study on ISIS-TTR<sub>Rx</sub>, which is expected to start this month.

"We are pleased to have received orphan drug status and fast track designation for ISIS-TTR<sub>Rx</sub> for patients with FAP. ISIS-TTR<sub>Rx</sub> is our most advanced drug from our severe and rare disease franchise and represents a significant near-term commercial opportunity for us," said B. Lynne Parshall, chief operating officer and chief financial officer at Isis Pharmaceuticals, Inc. "We look forward to continuing to move ISIS-TTR<sub>Rx</sub> toward the market for patients who have very limited therapeutic options."

ISIS-TTR<sub>Rx</sub> is part of the Isis-GSK strategic alliance to develop RNA therapeutics for rare and infectious diseases. Upon initiation of the Phase two/three study, Isis Pharmaceuticals, Inc. will receive a 7.5 million dollar milestone payment and is eligible to earn an additional 50 million dollars in pre-licensing milestone payments to support the Phase two/three study of ISIS-TTR<sub>Rx</sub>. In addition, Isis Pharmaceuticals, Inc. is eligible to receive regulatory and sales milestones and double-digit royalties on sales of ISIS-TTR<sub>Rx</sub>.

### About ISIS-TTR<sub>Rx</sub>

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 the 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. There are two common types of transthyretin amyloidosis, familial amyloid cardiomyopathy, or FAC, which affects more than 40,000 patients worldwide, and FAP, which affects more than 10,000 patients worldwide. Patients with FAC have TTR build up in the heart muscle and succumb to heart failure five to six years after symptom onset. Patients with FAP have TTR build up in peripheral nerve tissue leading to the loss of nerve function and wasting. ISIS-TTR<sub>Rx</sub> is an investigational drug that is designed to inhibit the production of all forms of TTR, and could offer an alternative approach to treat all types of transthyretin-related amyloidosis.

### About ISIS Pharmaceuticals, Inc.

Isis Pharmaceuticals, Inc. is exploiting its strong position in antisense technology to discover and develop novel drugs for its product pipeline and for its partners. The company's broad pipeline consists of 25 drugs to treat a wide variety of diseases with an emphasis on cardiovascular, metabolic, severe and rare diseases, and cancer. Its partner, Genzyme, plans to commercialise Isis Pharmaceuticals, Inc.'s lead product, KYNAMRO™, in the US and Europe following regulatory approval. Additional information about Isis is available

at [www.isispharm.com](http://www.isispharm.com).

### **Isis Pharmaceuticals, Inc.'s Forward-Looking Statement**

This press release includes forward-looking statements regarding Isis Pharmaceuticals, Inc.'s collaboration with GSK, the discovery, development and potential of drugs for severe and rare diseases, and the development, activity, therapeutic potential and safety of ISIS-TTR<sub>RX</sub>. Any statement describing Isis Pharmaceuticals, Inc.'s goals, expectations, financial or other projections, intentions or beliefs, including the planned commercialisation of KYNAMRO, 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 commercialising drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. Isis Pharmaceuticals, Inc.'s forward-looking statements also involve assumptions that, if they never materialise or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Isis Pharmaceuticals, Inc.'s forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Isis Pharmaceuticals, Inc. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Isis Pharmaceuticals, Inc.'s programmes are described in additional detail in the company's annual report on Form 10-K for the year ended 31 December, 2011, and its most recent quarterly report on Form 10-Q. Copies of these and other documents are available from the company.

SOURCE: Isis Pharmaceuticals, Inc.

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