SIGMA-TAU PHARMACEUTICALS, INC. RECEIVES FDA APPROVAL OF CYSTARAN™ (CYSTEAMINE OPHTHALMIC SOLUTION) 0.44% FOR TREATMENT OF CORNEAL CYSTINE CRYSTALS IN CYSTINOSIS PATIENTS

GAITHERSBURG, MD, October 4, 2012 – Sigma-Tau Pharmaceuticals, Inc. announced today that the Company has received approval from the U.S. Food & Drug Administration ("FDA") for CYSTARAN™ (cysteamine ophthalmic solution) 0.44%, a topical ophthalmic therapeutic, developed in partnership with the National Institutes of Health ("NIH"), for the treatment of patients suffering from corneal cystine crystal accumulation as a result of cystinosis. CYSTARAN™ is designated an Orphan Drug in the U.S., and has been granted seven years of market exclusivity.

Cysteamine is a cystine-depleting agent which lowers the cystine content of cells in patients with cystinosis. However, when orally administered, cysteamine does not reach the cornea and is therefore ineffective in reducing the ocular effects of cystinosis. CYSTARAN™ is for topical ophthalmic use and is indicated for the treatment of corneal cystine crystal accumulation in patients with cystinosis. As a result, CYSTARAN™ now represents the only FDA-approved ophthalmic treatment for this condition.

"As a Company dedicated to the development and commercialization of novel therapies that address the unmet medical needs of a wide range of rare disease patients, we are delighted to announce the approval of CYSTARAN™," noted Dave Lemus, Chief Operating Officer of Sigma-Tau. "This new medicine will offer physicians the only FDA-approved treatment for patients with corneal cystine crystal accumulation, many of whom are children and whose lives are seriously impacted by this debilitating chronic condition."

"This is an important advance for children and adults who suffer from cystinosis," stated William A. Gahl, M.D., Ph.D., Clinical Director, National Human Genome Research Institute, NIH. "FDA approval of this drug represents the culmination of a longstanding collaboration among the National Eye Institute, the Eunice Kennedy Shriver National Institute of Child Health and Human Development, the National Human Genome Research Institute and Sigma-Tau Pharmaceuticals. It also has involved invaluable cooperation from cystinosis advocacy groups—the Cystinosis Research Network, the Cystinosis Foundation and the Cystinosis Research Foundation."

The CYSTARAN™ project was supported in part by an Orphan Drug grant from the FDA.

The clinical safety and efficacy of CYSTARAN™ was previously evaluated in controlled clinical trials conducted by the NIH, in approximately 300 patients. Results of these studies support the use of ophthalmic cysteamine as an effective treatment of corneal cystine crystals. The most frequently reported ocular adverse reactions, occurring in ≥ 10% of patients, were sensitivity to light, redness, eye pain/irritation, headache and visual field defects.

CYSTARAN™ is planned to be available in the future through specialty pharmacy distribution channels.

Please click here for full prescribing information for CYSTARAN™.

About Cystinosis

Cystinosis, which affects approximately 300 children and young adults in the U.S. and 2,000 individuals, worldwide, is a rare, genetic lysosomal storage disease, characterized by the abnormal accumulation of the amino acid, cystine. The disease causes cystine crystals to build up in various organs of the body, including the corneas, kidneys, liver, pancreas, muscles, brain and white blood cells. Corneal cystine accumulation can lead to ocular complications such as squinting, foreign body sensations, changes in visual acuity, corneal haziness and photophobia (i.e., sensitivity to light). Other complications of cystinosis include muscle weakness, diabetes, hypothyroidism, difficulty swallowing and rickets.

About Sigma-Tau Pharmaceuticals

Sigma-Tau Pharmaceuticals, Inc. is a U.S. based, wholly owned subsidiary of the sigma-tau Group, and is dedicated to the global development and commercialization of medicines for patients with rare diseases. Sigma-Tau Pharmaceuticals, Inc. is based in Gaithersburg, Maryland. Since 1989, the company's products have been focused on kidney disease, certain genetic disorders and cancers. With more than 7,000 identified rare diseases that affect approximately 30 million patients in the U.S. alone, Sigma-Tau places its considerable scientific resources behind the development and commercialization of compounds that benefit the few. The company has a substantial development program focused on transplant, cancer, inherited genetic disorders, malaria, and other areas of unmet medical need. For more information about the company, visit www.sigmatau.com.

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