sigma-tau PHARMACEUTICALS, INC.
Rare dedication

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SIGMA-TAU PHARMACEUTICALS LAUNCHES CYSTARAN™ FOR THE TREATMENT OF CORNEAL CYSTINE CRYSTAL ACCUMULATION IN PATIENTS WITH CYSTINOSIS

GAITHERSBURG, MD, May 1, 2013 – Sigma-Tau Pharmaceuticals, Inc. (Sigma-Tau), a part of the Sigma-Tau Group Rare Disease Franchise, announced today the availability of CYSTARAN™ (cysteamine ophthalmic solution) 0.44%, the first and only FDA-approved therapy for the treatment of corneal cystine crystal accumulation in patients with cystinosis. Sigma-Tau developed CYSTARAN in partnership with the National Institutes of Health (NIH) and in cooperation with the Cystinosis Foundation, the Cystinosis Research Foundation, and the Cystinosis Research Network. The U.S. Food and Drug Administration (FDA) approved CYSTARAN in October 2012, and the product has also been granted Orphan Drug status.

In connection with its product launch, Sigma-Tau has established the CYSTARAN Hotline which is administered by Accredo Specialty Pharmacy and staffed by pharmacists, registered nurses and specialists who are trained to coordinate the delivery of CYSTARAN directly to patients, provide reimbursement support, and offer pharmacy services. Patient assistance programs for CYSTARAN are also available, including co-pay assistance for eligible patients and access to CYSTARAN therapy for uninsured or under-insured patients. Patients, caregivers and physicians in the United States and Puerto Rico can access the CYSTARAN Hotline at 1-800-440-0473, or by visiting the Accredo website, www.Accredo.com.

"The CYSTARAN launch marks an important milestone for Sigma-Tau and further demonstrates our proven ability to develop and deliver novel therapies for patients suffering from a wide range of rare diseases," noted Dave Lemus, Chief Executive Officer of Sigma-Tau. "Sigma-Tau is committed to ensuring comprehensive access for all cystinosis patients with corneal crystal accumulation, and we feel especially privileged to be able to offer this critical new therapy which will make a positive impact on these patients' daily lives."

"For the hundreds of children and adults in the United States who suffer from corneal cystine crystal accumulation as a result of cystinosis, the commercial availability of CYSTARAN provides access to a critically needed therapy," commented Craig B. Langman, MD, the Isaac A. Abt, MD Professor of Kidney Diseases and Head of Kidney Diseases at the Ann & Robert H. Lurie Children's Hospital of Chicago and the Feinberg School of Medicine of Northwestern University. "We need to make sure that physicians and patients managing cystinosis understand the risk of eye complications and are aware of the availability of an FDA-approved therapy with documented safety and effectiveness."

CYSTARAN is available as a sterile ophthalmic solution containing 6.5 mg/mL of cysteamine hydrochloride equivalent to 4.4 mg/mL of cysteamine (0.44%). For full prescribing information for CYSTARAN, see www.cystaran.com.

Safety: 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.

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About Cystinosis

Cystinosis, a genetic disease which affects approximately 2,000 individuals worldwide, is a rare, life-threatening condition in which the body accumulates the amino acid cystine (a building block of proteins) within cells. Excess cystine leads to formation of crystals that can build up and damage cells throughout the body. Cystinosis slowly destroys numerous vital organs including the kidneys, liver, eyes, muscles and the brain. Other complications of cystinosis include muscle weakness, diabetes, hypothyroidism, difficulty in swallowing and rickets. 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). The clinical safety and efficacy of CYSTARAN has been evaluated in controlled clinical trials conducted by the NIH in approximately 300 patients.

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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