August 17, 2015

Raptor Pharmaceutical Receives FDA Approval of Expanded Label for PROCYSBI(R) to Treat Children Aged 2-6 Years With Nephropathic Cystinosis

NOVATO, Calif., Aug. 17, 2015 (GLOBE NEWSWIRE) -- Raptor Pharmaceutical Corp. (NASDAQ:RPTP) today announced that the U.S. Food and Drug Administration (FDA) approved the expanded use of PROCYSBI® (cysteamine bitartrate) delayed-release capsules to treat children two to six years of age with nephropathic cystinosis. The approved supplement was based on efficacy and safety data from an ongoing long-term extension study in which a cohort of children aged 2 to 6 years were enrolled and treated with PROCYSBI for 12 months. Additionally, data submitted as part of this supplement supported the long-term maintenance of white blood cell cystine levels and renal function in all age groups studied during extended treatment with PROCYSBI. PROCYSBI is now approved for the treatment of nephropathic cystinosis in adult and in pediatric patients 2 years of age and older in the U.S. Please see the full prescribing information at www.PROCYSBI.com.

"Strict adherence to cystine depleting therapy from as early an age as possible is critical to maintaining kidney function and leading longer, healthier lives for patients with cystinosis," said Craig Langman, M.D., The Isaac A. Abt, M.D. Professor of Kidney Diseases, Feinberg School of Medicine and Head of Kidney Diseases at Ann & Robert H. Lurie Children's Hospital of Chicago and the lead investigator on the extension trial. "The data in the two to six year old group confirm the maintenance of stable kidney function over time. Not only will this help these young patients with cystinosis, but the 12 hour dosing schedule for PROCYSBI could be a significant benefit to the caregivers of these young children."

"We are extremely pleased that younger patients and families living with cystinosis will now have access to PROCYSBI," said Krishna Polu, M.D., Chief Medical Officer of Raptor. "We appreciate the support of the cystinosis community, families, caregivers, and patients who participated in the trials that led to the initial and expanded approval of PROCYSBI. We remain committed to advancing the science and understanding of cystinosis to help patients and caregivers take more control of their lives."

"Caregivers of younger individuals with cystinosis will now have another treatment option to discuss with their medical professional teams," stated Jeff Larimore, President of the Cystinosis Research Network. "Every opportunity provided to improve treatment adherence is applauded by the cystinosis community."

PROCYSBI was initially approved in 2013. Physicians can prescribe PROCYSBI by calling RaptorCares at 1-855-888-4004. RaptorCares provides individualized services to help patients access PROCYSBI through education, support, extensive case management and a commitment to the principle that no eligible U.S. patient with nephropathic cystinosis will be denied access to PROCYSBI based on inability to pay.

About Nephropathic Cystinosis

Nephropathic cystinosis comprises 95% of diagnosed cases of cystinosis, a rare, life-threatening metabolic lysosomal storage disorder that causes toxic accumulation of cystine in all cells, tissues, and organs in the body. Elevated cystine leads to progressive, irreversible tissue damage and multi-organ failure, including kidney failure, blindness, muscle wasting and premature death. Nephropathic cystinosis is typically diagnosed in infancy and requires lifelong therapy.

Strict adherence to therapy is crucial to improve outcomes, as even brief delays in cysteamine dosing may allow cystine to return to toxic levels. Left untreated, the disease is usually fatal by the end of the first decade of life. There are an estimated 500 patients living in the United States with cystinosis, and 2,000 worldwide.

About PROCYSBI® (cysteamine bitartrate) delayed-release capsules

PROCYSBI is the first cystine depleting agent that can be given every 12 hours that is approved in the U.S. for the management of nephropathic cystinosis in adults and children 2 years of age and older. It is contraindicated in patients with a hypersensitivity to cysteamine or penicillamine. The most commonly reported side effects are vomiting, nausea, abdominal pain, breath odor, diarrhea, skin odor, fatigue, rash, and headache. For additional information on PROCYSBI, including full prescribing information, please visit www.procysbi.com.

About Raptor Pharmaceutical

Raptor Pharmaceutical Corp. is a global biopharmaceutical company focused on the development and commercialization of life-altering therapeutics that treat rare, debilitating and often fatal diseases. The company is engaged in multiple therapeutic areas such as nephropathic cystinosis, Huntington's disease (HD), pediatric nonalcoholic steatohepatitis (NASH), and mitochondrial diseases including Leigh syndrome. Raptor holds several orphan drug designations, including orphan drug exclusivity for nephropathic cystinosis in the U.S. and EU, and orphan drug designation for HD in the U.S. and EU. Raptor holds intellectual property for the use of cysteamine in HD and other neurodegenerative disorders including Parkinson's disease and Rett syndrome. For additional information, please visit www.raptorpharma.com.

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