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Raptor Pharmaceutical Receives Marketing Authorization for PROCYSBI(R) in European Union

NOVATO, Calif., Sept. 12, 2013 (GLOBE NEWSWIRE) -- Raptor Pharmaceutical Corp. (Nasdaq:RPTP) today announced that the European Commission (EC) has approved PROCYSBI[®] gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate) as an orphan medicinal product for the treatment of proven nephropathic cystinosis for marketing in the European Union (EU). The approval of the marketing authorization application (MAA) by the EC follows the positive recommendation by the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) in June 2013. The orphan medicinal product designation provides a 10-year period of market exclusivity in the EU that starts from the notification date of the EC Decision granting the MAA.

"The EC approval of PROCYSBI brings us a major step closer to providing access to this important new therapeutic option to cystinosis patients in Europe," said Christopher M. Starr, Ph.D., Raptor's chief executive officer. "Active dialogue has begun with a number of EU member states initially as part of a phased introduction of PROCYSBI to patients with this debilitating disease."

PROCYSBI is indicated for the treatment of proven nephropathic cystinosis. Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure. PROCYSBI is taken orally every twelve hours. PROCYSBI was engineered to bypass absorption in the stomach with an extended terminal half-life so that patients experience steady drug levels in their bodies for the full 12-hour dosing period. Randomized controlled clinical trials and extended treatment with PROCYSBI therapy demonstrated consistent and continuous control of white blood cell cystine levels.

Basis for MAA Approval

The EC approval of PROCYSBI was based on data from six clinical trials, including a multi-center randomized, active-controlled Phase 3 trial of 43 patients with nephropathic cystinosis and extension data from that trial. The EC's Committee for Medicinal Products for Human Use (CHMP) concluded that the submitted study data support clinical efficacy of PROCYSBI similar to that of immediate-release cysteamine with a dosing schedule that may promote a better compliance.

Important Product Information from the EU Summary of Product Characteristics (SmPC) of PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate)

PROCYSBI treatment should be initiated under the supervision of a physician experienced in the treatment of cystinosis. PROCYSBI is a cystine depleting agent that has received approval in the EU for the treatment of proven nephropathic cystinosis.

It is contraindicated in patients with a hypersensitivity to any form of cysteamine (mercaptamine) or any of PROCYSBI's excipients, penicillamine and for breast-feeding women.

Severe allergic reactions have been uncommonly seen in patients treated with cysteamine. The most commonly reported side effects are vomiting, nausea, diarrhoea, anorexia, fever, sleepiness, skin rash, abnormal liver function on blood tests, abdominal pain, unpleasant breath and body odor, heartburn and tiredness. Uncommon side effects of cysteamine include skin lesions, bone lesions and joint problems, low blood cell count, seizure, depression, gastro-intestinal problems, and benign intracranial hypertension. Patients should be routinely monitored for development of skin or bone lesions and dosage of cysteamine reduced or stopped upon occurrence. If a severe skin rash develops such as erythema multiforme bullosa or toxic epidermal necrolysis, cysteamine should be discontinued.

Cysteamine has been associated with gastrointestinal ulceration and bleeding. Unusual abdominal symptoms or changes in abdominal symptoms should be medically assessed to exclude the possibility of fibrosing colonopathy, considered as possibly related to enteric-coating.

Central Nervous System (CNS) symptoms such as seizures, lethargy, somnolence, depression, and encephalopathy have been associated with cysteamine. Patients should not engage in potentially hazardous activities until the effects of cysteamine on mental performance are known.

Cysteamine has been associated with reversible leukopenia and abnormal liver function studies. Therefore, blood counts and

liver function should be monitored.

Benign intracranial hypertension (or pseudotumor cerebri (PTC)) and/or papilledema has been associated with cysteamine bitartrate treatment. Physicians should monitor for signs and symptoms of PTC.

For the full PROCYSBI EU Summary of Product Characteristics, please visit www.raptorpharma.com.

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. Left untreated, the disease is usually fatal by the end of the first decade of life. There are an estimated 800 patients living in the EU with cystinosis, and 2,000 worldwide.

Cystine depletion is the primary treatment strategy for nephropathic cystinosis. However, poor adherence to therapy has been a major challenge resulting in poor sustained control of cystine levels, and patients consequently experience poor clinical outcomes, including kidney insufficiency leading to dialysis and kidney transplantation, muscle wasting and in some cases, premature death. Even brief interruptions in daily therapy can permit toxic accumulation of cystine, exposing tissues to renewed, progressive deterioration.

About Raptor Pharmaceutical Corp.

Raptor Pharmaceutical Corp. is a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat rare, debilitating and often fatal diseases. The company's first product, PROCYSBI (cysteamine bitartrate) delayed-release capsules, was approved by the FDA for the management of nephropathic cystinosis, a rare metabolic lysosomal storage disease in April 2013. Raptor's pipeline also includes RP103 in a Phase 2/3 trial for Huntington's disease and a Phase 2 trial in nonalcoholic fatty liver disease in children. PROCYSBI was granted orphan designation for nephropathic cystinosis in the U.S. and EU and RP103 has received U.S. orphan drug designation for Huntington's disease. For additional information, please visit www.raptorpharma.com.

FORWARD-LOOKING STATEMENTS

This document contains forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995. These statements are indicated by words or phrases such as "believes," "expects," "anticipates," "estimates," "plans," "continuing," "ongoing", "projected" and similar words or phrases and relate to future events or our future results of operations or future financial performance, including, but not limited to, statements regarding the introduction of PROCYSBI in EU countries, and orphan exclusivity from the European Commission in the coming months. These statements are only predictions and involve known and unknown risks, uncertainties and other factors, which may cause the Company's actual results to be materially different from these forward-looking statements. Factors which may significantly change or prevent the Company's forward-looking statements from fruition include: that Raptor may be unsuccessful in developing any products or acquiring products; that Raptor's technology may not be validated as it progresses further and its methods may not be accepted by the scientific community; that Raptor is unable to retain or attract key employees whose knowledge is essential to the development of its products; that unforeseen scientific difficulties develop with the Company's process; that Raptor's patents are not sufficient to protect essential aspects of its technology; that competitors may invent better technology; that Raptor's products may not work as well as hoped or worse, that the Company's products may harm recipients; and that Raptor may not be able to raise sufficient funds for development or working capital. As well, Raptor's products may never develop into useful products and even if they do, they may not be approved for sale to the public. Raptor cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they were made. Certain of these risks, uncertainties and other factors are described in greater detail in the Company's filings from time to time with the Securities and Exchange Commission (the "SEC"), which Raptor strongly urges you to read and consider, including: Raptor's transition report for the four months ended December 31, 2012 on Form 10-KT filed with the SEC on March 14, 2013, as amended, and Raptor's Quarterly Report on Form 10-Q filed with the SEC on August 9, 2013, as amended, which is available free of charge on the SEC's web site at <http://www.sec.gov>. Subsequent written and oral forward-looking statements attributable to Raptor or to persons acting on its behalf are expressly qualified in their entirety by the cautionary statements set forth in Raptor's reports filed with the SEC. Raptor expressly disclaims any intent or obligation to update any forward-looking statements.

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