



## Raptor Pharmaceutical Receives Notices of Allowance for Key European Patent Applications Protecting Its Cysteamine Portfolio

### Patent Claims Cover Use of Delayed-Release Cysteamine in Cystinosis

NOVATO, Calif., April 12, 2012 (GLOBE NEWSWIRE) -- Raptor Pharmaceutical Corp. ("Raptor" or the "Company") (Nasdaq:RPTP), today announced that the European Patent Office has issued a Notice of Allowance for a key patent covering the use of enteric-coated, delayed-release ("DR") oral formulations of cysteamine bitartrate, including Raptor's proprietary microbead formulation, RP103, as well as other formulations of cysteamine and cystamine, as outlined below:

#### Application No. / Patent

No.: 07 762 690.1 / 1919458

Issued Notice of Allowance:

March, 14, 2012

Patent Title:

"Enterically Coated Cystamine, Cysteamine and Derivatives Thereof."

Expected to Cover:

Use of DR Cysteamine for administration to patients for the potential treatment of cystinosis, including nephropathic cystinosis

Expected Initial Term:

20 years from the filing date; expiring January 26, 2027

Raptor holds exclusive, worldwide licenses to this and other related patent applications, which are owned by the Regents of the University of California, and are based on work performed at the University of California, San Diego ("UCSD"). In 2011, counterpart patents were granted in the US directed to DR cysteamine compositions, including RP103, and methods of use for any indication, including nephropathic cystinosis.

"Having submitted a Marketing Authorization Application for RP103 for the potential treatment of nephropathic cystinosis to the European Medicines Agency, this notice of allowance gives the Company key additional IP protection in Europe," commented Ted Daley, President of Raptor.

Patent application 07 762 690.1 covers the use of a composition of cysteamine or cystamine, regardless of the specific formulation, that provides increased delivery to the small intestine with pharmacokinetic benefits that allow for twice daily dosing in the potential treatment of cystinosis, including nephropathic cystinosis. Raptor believes that its proprietary formulation of cysteamine may provide significant therapeutic and compliance advantages compared to the presently marketed, immediate-release cysteamine bitartrate for the potential treatment of nephropathic cystinosis. In July 2011, Raptor announced that its Phase 3 clinical trial of RP103 (Cysteamine Bitartrate Delayed-release Capsules) met the sole primary endpoint and there were no unexpected serious safety concerns attributable to RP103 experienced by patients in the trial. The trial was conducted at three clinical sites in the U.S. and five clinical sites in Europe. In March, 2012 Raptor filed a Marketing Authorization Application ("MAA") with the European Medicines Agency ("EMA"), as well as a New Drug Application ("NDA") with the US Food and Drug Administration ("FDA"), for RP103 for the potential treatment of nephropathic cystinosis.

#### About Nephropathic Cystinosis

Nephropathic cystinosis, an orphan disease, is estimated to effect a population of 2,000 patients worldwide, including 500 patients in the U.S. and 800 patients in Europe. Cystinosis patients have inherited a defective cystine transporter gene, which results in body-wide cellular toxicity resulting from the abnormal buildup of the amino acid cystine in the lysosomes. Cystinosis is usually diagnosed in the first year of life and requires lifelong therapy. Cystine crystals accumulate in various tissues and organs, including the kidneys, brain, liver, thyroid, pancreas, muscles and eyes. Left untreated, the disease is fatal by the first decade of life. RP103 reduces cellular toxicity by continuously removing cystine from the lysosome.

#### About Cysteamine and RP103

RP103 is Raptor's proprietary delayed and extended release oral medication designed to potentially treat the underlying metabolic cause of cystinosis. RP103 is an enteric coated, microbead formulation of cysteamine bitartrate that has been formulated to be sprinkled onto food for administration to patients too young to take oral capsules. Raptor has been granted orphan product designation for RP103 by the EMA and FDA.

In December 2007, Raptor obtained an exclusive, worldwide license from the University of California, San Diego for the development of RP103 for nephropathic cystinosis and for cysteamine for other potential indications including Huntington's Disease, currently in a Phase 2/3 clinical trial in France, and non-alcoholic steatohepatitis ("NASH") currently in a Phase 2b clinical trial in the US.

### **About Raptor Pharmaceutical Corp.**

[Raptor Pharmaceutical Corp.](#) (Nasdaq:RPTP) ("Raptor") seeks to research, produce, and deliver medicines that improve life for patients with severe, rare disorders. Raptor currently has product candidates in clinical development designed to potentially treat [nephropathic cystinosis](#), Non-[alcoholic](#) Steatohepatitis ("NASH"), [Huntington's](#) Disease ("HD"), [aldehyde dehydrogenase deficiency](#) ("ALDH2"), and [thrombotic](#) disorder.

Raptor's preclinical programs are based upon bioengineered novel drug candidates and drug-targeting platforms derived from the human [receptor-associated protein](#) and related proteins that are designed to target cancer and infectious diseases.

For additional information, please visit [www.raptorpharma.com](http://www.raptorpharma.com).

The Raptor Pharmaceutical Corp. logo is available at <http://www.globenewswire.com/newsroom/prs/?pkgid=7180>

### 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 relate to future events or our future results of operation or future financial performance, including, but not limited to the following statements: that this notice of allowance adds key additional IP protection in Europe; that Raptor's proprietary formulation of cysteamine may provide significant therapeutic and compliance advantages compared to the presently marketed, immediate-release cysteamine bitartrate for the treatment of nephropathic cystinosis; and that Raptor will be able to successfully develop RP103 or any of its other product candidates. 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 annual report on Form 10-K, as amended by Form 10-K/A, filed with the SEC on November 11, 2011 and December 19, 2011, respectively; and Raptor's quarterly report on Form 10-Q filed with the SEC on April 9, 2012; all of which are 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.

CONTACT: Trout Group (investors)

Lauren Glaser

(646) 378-2972

[lglaser@troutgroup.com](mailto:lglaser@troutgroup.com)

EVC Group (media)

Janine McCargo

(646) 688-0425

[jmccargo@evcgroup.com](mailto:jmccargo@evcgroup.com)



Source: Raptor Pharmaceutical Corp.

News Provided by Acquire Media