

Raptor Pharmaceutical to Enroll Five Additional Patients in Pivotal Phase 3 Clinical Trial of DR Cysteamine for the Potential Treatment of Nephropathic Cystinosis

NOVATO, Calif., Feb. 15, 2011 (GLOBE NEWSWIRE) -- Raptor Pharmaceutical Corp. ("Raptor" or the "Company"), today announced it has reopened enrollment in its Phase 3 clinical trial of its proprietary delayed-release oral formulation of cysteamine bitartrate ("DR Cysteamine") in patients with nephropathic cystinosis ("cystinosis"). The pivotal Phase 3 clinical trial is designed as a study of the safety, tolerability, pharmacokinetics ("PK") and pharmacodynamics ("PD") of DR Cysteamine compared with immediate-release cysteamine bitartrate.

The design of the pivotal Phase 3 clinical trial is a result of discussions with the FDA through which the FDA provided significant guidance on trial protocol design, clinical endpoints, and a statistical analysis plan ("SAP"). The primary endpoint of the trial is the steady-state white blood cell ("WBC") cystine levels of patients taking DR Cysteamine, dosed every twelve hours, compared to immediate-release cysteamine bitartrate, which requires dosing every six hours. At defined intervals during the clinical study, WBC cystine measurements are taken from each patient, one per day, over three day periods. The spread of these daily measurements is used to calculate a statistical value called the intra-patient variance and this value, as defined in the SAP, is used to determine the total number of patients required in the study.

As defined in the SAP, the total number of patients required to complete the study, is determined by an interim statistical analysis of intra-patient variance of the first 20 patients once they had completed the study. Last month, the Company reported completion of enrollment based upon an intra-patient variance report received by the Company, which determined that a total of 30 patients would be required to complete the study. As of February 7, 2011, a total of 31 patients successfully completed the study. Upon final quality control and quality assurance review of the clinical data set, but prior to database lock, it was reported to the Company that the calculation used to determine the intra-patient variance value contained a mathematical error. A repeat of the 20-patient interim analysis of the intra-patient variance using the corrected data resulted in an increase in the required patient number to 36 patients, rather than 30, as previously reported. Therefore, as required in the SAP, the Company is obligated to enroll at least 36 patients in the trial.

"Based on these findings, the Company has directed its clinical sites to reopen enrollment to include at least 5 additional patients," stated Patrice Rioux, M.D., Ph.D., Chief Medical Officer of Raptor. "Our sites report that potential additional patients have already been identified and clinical teams are still in place to enroll and manage these patients through the clinical protocol."

"Although this requirement to enroll additional patients will result in an unanticipated delay of our goal of clinical data reported by the end of March 2011, we are fortunate to have found this error early in our data review," said Christopher M. Starr, Ph.D., CEO of Raptor. "Although we are doing all we can to quickly enroll these additional patients and complete our study, we know that a high quality, reliable data set to support our NDA filing is of prime importance. We anticipate that this will result in reporting top line data from this pivotal clinical trial sometime within the second quarter of 2011."

About Nephropathic Cystinosis

Nephropathic cystinosis is an inborn metabolic error characterized by the abnormal transport of cystine, an amino acid, out of the lysosomes. Poor compliance with current treatments for nephropathic cystinosis can cause serious health consequences, including renal failure and resultant need for a kidney transplant; growth failure; rickets and fractures; photophobia and blindness. Symptom onset typically occurs within the first year of life, when cystine crystals accumulate in various tissues and organs, including the kidneys, brain, liver, thyroid, pancreas, muscles and eyes.

About Cysteamine and DR Cysteamine

DR Cysteamine is Raptor's proprietary enteric-coated, microbead oral formulation of cysteamine bitartrate designed to potentially reduce dosing frequency and reduce gastrointestinal side effects associated with immediate-release cysteamine bitartrate, which is approved for sale by the FDA and EMA to treat nephropathic cystinosis.

In December 2007, Raptor obtained an exclusive, worldwide license from the University of California, San Diego for the development DR Cysteamine for nephropathic cystinosis and cysteamine for other potential indications including Huntington's Disease, NASH and Batten Disease.

About Raptor Pharmaceutical Corp.

Raptor Pharmaceutical Corp. ("Raptor") is dedicated to speeding the delivery of new treatment options to patients by working to improve existing therapeutics through the application of highly specialized drug targeting platforms and formulation expertise. Raptor focuses on underserved patient populations where it can have the greatest potential impact. Raptor currently has product candidates in clinical development designed to potentially treat [nephropathic cystinosis](#), [non-alcoholic steatohepatitis \("NASH"\)](#), [Huntington's Disease \("HD"\)](#), [aldehyde dehydrogenase \("ALDH2"\) deficiency](#), 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 \("RAP"\)](#) and related proteins that are designed to target cancer, neurodegenerative disorders and infectious diseases.

For additional information, please visit 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 Raptor will be able to enroll at least five additional patients in the clinical study; that Raptor will meet its goal of reporting top line data from the Phase 3 clinical trial in the second quarter of 2011; the Raptor will be able to submit a high quality, reliable data set to support its NDA filing; that Raptor believes that twice-daily DR Cysteamine has the potential to be as efficacious as immediate-release cysteamine bitartrate for cystinosis patients, but with a more convenient dosing schedule and potentially improved tolerability; and that Raptor will be able to successfully develop DR Cysteamine 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 filed with the SEC on November 22, 2010; and Raptor's quarterly report on Form 10-Q filed with the SEC on January 14, 2011; 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.