Raptor Pharmaceutical Corp. Announces Publication of Results from Phase 2a Trial of DR Cysteamine for Treatment of Cystinosis

NOVATO, California, January 11 /PRNewswire/ --

- Potential to Improve Treatment with Less Frequent Administration and Better Tolerability

Raptor Pharmaceutical Corp. ("Raptor" or the "Company") (Nasdaq: RPTP), today announced the publication of results from a Phase 2a clinical trial of a prototype formulation of its proprietary delayed-release cysteamine bitartrate ("DR Cysteamine") in patients with nephropathic cystinosis ("cystinosis"). Ranjan Dohil, M.D., Professor of Pediatrics at the University of California, San Diego, was lead author of the study to be published in the Journal of Pediatrics and available online at http://www.ncbi.nlm.nih.gov/pubmed/19775699?itool=EntrezSystem2.PEntrez.Pubmed.Pubmed_ResultsPanel.Pubmed_RVDocSum&ordinalpos=1.

(Logo: http://www.newscom.com/cgi-bin/prnh/20071022/NYM074LOGO)

The Phase 2a study demonstrated proof-of-concept for DR Cysteamine, which is Raptor's proprietary, delayed-release, enteric-coated microbead formulation of immediate release cysteamine bitartrate contained in a gelatin capsule. Immediate-release cysteamine bitartrate ("IR Cysteamine") is the current standard of care for treating cystinosis. The results indicated that when given twice daily, the prototype DR Cysteamine formulation was effective at maintaining low white blood cell ("WBC") cystine levels (<2 nmol half-cystine/mg protein) in subjects with cystinosis. Results also indicated that the prototype DR Cysteamine effectively maintained trough WBC cystine levels within a satisfactory range when patients received approximately 60% of the previous total daily dose of IR Cysteamine.

Dr. Dohil stated, "We are seeking to improve tolerability and reduce dosing frequency requirements of IR Cysteamine which have been documented challenges for cystinosis patients, leading to widely reported instances of poor treatment compliance. We believe the results of the Phase 2a study bring us significantly closer to a potential treatment solution for cystinosis patients. The results from our trial indicate prototype delayed-release cysteamine formulation lead to improved tolerability and efficacy when administered twice-daily and at a lower total daily dose than IR Cysteamine. Based on these results, I believe that Raptor's final DR Cysteamine formulation has the potential to improve compliance and long-term treatment outcomes for cystinosis patients."

Based on these Phase 2a results, Raptor conducted a Phase 2b clinical trial using its final commercial formulation of DR Cysteamine and recently announced the following top-line Phase 2b results: DR Cysteamine demonstrated improved tolerability and the potential to reduce total daily dosage and administration frequency compared to IR Cysteamine.

- Pharmacokinetic evaluation showed that DR Cysteamine had a terminal half-life more than three times longer than the terminal half-life of IR Cysteamine.

- Twice-daily DR Cysteamine may achieve the same pharmacodynamic result while using a total daily dose 30% lower than IR Cysteamine administered four times daily.
- No adverse events recorded during the clinical trial were determined by the principal investigator to be possibly or probably related to DR Cysteamine. Nine adverse events recorded in the clinical trial were determined to be possibly or probably related to IR Cysteamine.
- The proprietary, final formulation of DR Cysteamine confirmed earlier clinical trials conducted by Dr. Dohil using an enteric-coated prototype formulation of cysteamine bitartrate, which was funded by the Cystinosis Research Foundation ("CRF").

During the first quarter of 2010, Raptor plans to meet with the Food and Drug Administration ("FDA") and European Medicines Agency ("EMEA") to discuss plans for a repeat-dose, pivotal, Phase 3 clinical trial in cystinosis patients. Upon receiving FDA and EMEA agreements on protocol, Raptor intends to initiate its Phase 3 clinical trial at multiple sites in the US and Europe.

Cystinosis is an inborn metabolic error characterized by the abnormal transport of cystine, an amino acid, out of the lysosomes. Failure to treat cystinosis can cause serious health consequences, including renal failure and resultant kidney transplant, growth failure, rickets, 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 Raptor Pharmaceutical Corp.

Raptor Pharmaceutical Corp. (Nasdaq: RPTP) ("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 a non-opioid solution designed to potentially treat chronic pain.

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.

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 the results of the Phase 2a study bring Raptor significantly closer to a potential

treatment solution for cystinosis patients; that Raptor's DR Cysteamine formulation has the potential to improve compliance and long-term treatment outcomes for this highly unmet medical need; that twice-daily DR Cysteamine may achieve the same pharmacodynamic result while using a total daily dose 30% lower than IR Cysteamine administered four times daily; that Raptor intends to initiate its Phase 3 clinical trial at multiple sites in the US and Europe; and that any of Raptor's clinical and preclinical drug candidates will result in approved therapeutics. 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 at raising funds to continue its development programs; 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; and that Raptor's products may not work as well as hoped or worse, that the Company's products may harm recipients. 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 current report on Form 8-K as filed with the SEC on November 17, 2009 and the joint proxy statement/prospectus on Form S-4 filed with the SEC on August 19, 2009, 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.

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