September 14, 2015

Raptor Pharmaceutical Provides Topline Results From Phase 2b CyNCh Study Evaluating RP103 in Pediatric Nonalcoholic Steatohepatitis

NOVATO, Calif., Sept. 14, 2015 (GLOBE NEWSWIRE) -- Raptor Pharmaceutical Corp. (Nasdaq:RPTP) today announced topline results from the Phase 2b CyNCh study, which did not meet its primary endpoint of improving nonalcoholic steatohepatitis (NASH) in children.

The trial evaluated the safety and efficacy of RP103, or cysteamine bitartrate delayed-release capsules, in children with biopsy-confirmed NASH. The study did not achieve its primary endpoint defined as a two-point decrease in NAFLD Activity Score (NAS) and no worsening of fibrosis (p-value = 0.34). There were no differences in adverse events observed in children on RP103 compared to placebo.

The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), part of the National Institutes of Health, sponsored and conducted the CyNCh study through a Cooperative Research and Development Agreement (CRADA) with Raptor. Study investigators intend to submit the data for publication and present topline results at the upcoming American Association for the Study of Liver Diseases (AASLD) meeting being held November 13-17, 2015 in San Francisco.

"While these trial results replicated the serological improvements seen in the earlier Phase 2a study, they did not translate into a measurable effect on histology. We're disappointed with this outcome given the paucity of treatment for these children with NASH. While we'll work closely with the NIDDK to understand the full data set, we do not expect to advance this program based on topline results," said Julie Anne Smith, Raptor's President and CEO. "This clarifies our near-term priorities, which are to maximize the reach of PROCYSBI in nephropathic cystinosis, further the development of RP103 in Huntington's and mitochondrial diseases, prepare for QUINSAIR'S launch and initiate at least one trial in nontuberculous mycobacteria or bronchiectasis. We remain wholly devoted to developing and commercializing transformational treatments for people living with rare diseases."

About CyNCh

CyNCh (**Cy**steamine Bitartrate Delayed-Release for the Treatment of **N**onalcoholic Fatty Liver Disease [NAFLD] in **Ch**ildren) has been sponsored and conducted by the NIDDK. CyNCh was a multi-center, placebo-controlled clinical trial of 169 children ages eight to 17 years with biopsy-confirmed moderate-to-severe NAFLD. Patients were randomized to receive either 600, 750 or 900 mg/day of RP103 or placebo for 52 weeks. The primary objective was to evaluate whether 52 weeks of treatment with RP103 would result in improvement in liver disease severity defined as: (1) a decrease in NAFLD Activity Score of two or more points, and; (2) no worsening of fibrosis. Secondary endpoints included: reduction in serum aminotransferase and gamma-glutamyl transpeptidase; reduction in MRI-determined hepatic fat fraction; changes to markers of oxidation and anti-oxidant status; changes in fasting insulin and glucose; changes in weight, height, body mass index (BMI) and waist circumference; changes in the Pediatric Quality of Life score; changes to any symptoms that patient may have experienced; proportion with a change from a histological diagnosis of definite NASH or indeterminate for NASH to not NASH at end of treatment; individual histological characteristics at end of treatment compared to baseline such as steatosis (fatty liver), lobular inflammation, portal chronic inflammation, ballooning, fibrosis score and stage 1a versus 1b fibrosis; and, change in mean NAS. End of study biopsies were conducted in patients after the 52-week treatment period, with all biopsies centrally scored in a blinded fashion. Further details can be found at https://clinicaltrials.gov/ct2/show/NCT01529268.

About RP103 (cysteamine bitartrate)

RP103 is Raptor's proprietary delayed and extended release oral medication designed to treat the underlying metabolic cause of several rare diseases and disorders including cystinosis and Huntington's disease. RP103 is in clinical development for Huntington's disease and mitochondrial diseases based on a number of proteostatic and antioxidative properties demonstrated in multiple animal models.Â Clinical data were reported from the ongoing Phase 2 CYST-HD study in Huntington's disease in 2014.

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 Quinsair™ (levofloxacin inhalation solution)

Quinsair is a proprietary inhaled formulation of levofloxacin, a fluoroquinolone antibiotic, which is approved in the EU and in Canada for the management of chronic pulmonary infections due to *Pseudomonas aeruginosa* in adult patients with cystic fibrosis. Administration of Quinsair with a high efficiency nebulizer allows for the delivery of high concentrations of active drug directly to the site of infection in approximately five minutes. Quinsair is contraindicated in patients with hypersensitivity to levofloxacin, a history of tendon disorders related to fluoroquinolones, epilepsy, or who may be pregnant or breast feeding. Quinsair's safety was evaluated in two double-blind, placebo-controlled studies and in an active comparator study in which the most frequently reported adverse reactions were cough/productive cough, dysgeusia, and fatigue/asthenia.

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) and mitochondrial diseases including Leigh's 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.

Forward-Looking Statements

This press release 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: ongoing development of Raptor's product candidates; Raptor's plans and timing for regulatory submissions; and anticipated clinical and other milestones. These statements are only predictions and involve known and unknown risks, uncertainties and other factors, which may cause Raptor's actual results to be materially different from these forward-looking statements. Raptor cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they were made. Factors which may contribute to differences in actual results include, among others: the possibility that the acquisition of QUINSAIR may not occur on the anticipated timeline, or at all; market acceptance and sales of PROCYSBI in the U.S. and other territories; Raptor's ability to market and sell QUINSAIR; Raptor's ability to expand the use of RP103 and potentially MP-376 and to receive regulatory approval for other indications; Raptor's reliance on a single active pharmaceutical ingredient supplier for PROCYSBI and other third parties in connection with drug product development; compliance with healthcare regulations, ongoing regulatory requirements and potential penalties; any serious adverse side effects associated with PROCYSBI, QUINSAIR or any other future products and product liability claims; third-party payor coverage, reimbursement and pricing; enacted and future healthcare legislation; Raptor's ability to obtain and maintain orphan drug or other regulatory exclusivity for PROCYSBI, QUINSAIR or any other future products; the integration of European operations with U.S. operations; relationships with key scientific and medical collaborators; intellectual property protection and claims and continued license rights; and Raptor's ability to fund its operations and make required payments on its debt. Certain of these risks, uncertainties and other factors are described in greater detail in Raptor's filings from time to time with the SEC, which Raptor strongly urges you to read and consider, including: Raptor's annual report on Form 10-K for the twelve months ended December 31, 2014 filed with the SEC on March 2, 2015, Raptor's quarterly reports on Form 10-Q for the quarterly periods ended March 31, 2015 and June 30, 2015 filed with the SEC on May 7, 2015 and August 6, 2015, respectively, Raptor's current report on Form 8-K filed with the SEC on September 9, 2015, and other periodic reports filed with SEC, 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 except as may be required by law.

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Source: Raptor Pharmaceutical Corp.

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