



Raptor Plans to Advance RP103 in a Registration Study in Huntington's Disease Based on Favorable Treatment Effects at 36 Months in CYST-HD Trial

Conference Call and Webcast Today at 8:00 a.m. EST/5:00 a.m. PST

NOVATO, Calif., December 10, 2015 -- Raptor Pharmaceutical Corp. (Nasdaq: RPTP) today announced 36-month efficacy results from a Phase 2/3 clinical trial evaluating RP103 for the potential treatment of Huntington's disease, called CYST-HD, conducted in collaboration with the Centre Hospitalier Universitaire d'Angers (CHU d'Angers), France. Data from the study revealed that there was a slowing in the rate of change in the Total Motor Score (TMS) component of the Unified Huntington's Disease Rating Scale (UHDRS) at 36 months of treatment favoring earlier RP103 treatment (RP103-RP103) versus delayed start of RP103 treatment (placebo-RP103). The 25% treatment effect in TMS favoring subjects treated with RP103 for the full 36 months as compared to the placebo/RP103 arm, while not statistically significant, is regarded by clinical leaders in the field as clinically meaningful. The TMS data were consistent with observed effects in key functional measures including Total Functional Capacity (TFC) and Independence Scale. There was statistically significant improvement in the Independence Scale.

Huntington's disease is a rare, inherited, neurodegenerative disorder that causes motor, cognitive and behavioral dysfunction, deterioration and premature death. There is no currently approved drug that slows disease progression.

"The 36-month efficacy results from the CYST-HD study are clinically meaningful and suggest that RP103 may play an important role in the treatment of Huntington's disease," said Dominique Bonneau, M.D., Professor of Medical Genetics at the CHU d'Angers and principal investigator for CYST-HD. "These data warrant further assessment as there remains a clear need for new, safe and tolerable therapies to treat this disease."

CYST-HD Results

CYST-HD was a randomized, placebo-controlled, multi-center trial evaluating the long-term efficacy, safety and tolerability of RP103 for the treatment of Huntington's disease. Placebo subjects crossed over to open-label treatment at 18 through 36 months. This phase of the study examined the effect of RP103 in Huntington's disease subjects treated earlier, beginning at Month 0 (RP103/RP103) compared to subjects with a delayed start to treatment, beginning at Month 18 (placebo/RP103). The primary efficacy endpoint for this study was the change from baseline at 18 months in the TMS component of the UHDRS between placebo/RP103 and RP103/RP103-treated subjects. Analysis of this endpoint was also completed in the open-label

phase of the study at 36 months. Key secondary endpoints evaluating function included the UHDRS-TFC and Independence Scale. 88 subjects entered the open-label period and 78 subjects completed 36 months of treatment. The full analyses set included all randomized subjects from Month 0 to Month 36.

An evaluation of the change in the progression of the UHDRS-TMS at Month 36 from baseline in the full analyses set in the trial showed a 25% slower progression [10.0 (1.7) vs. 13.3 (1.8), respectively; $p=0.18$] in patients treated earlier with RP103 relative to those patients on a delayed start. In a completers analysis, these effects were more pronounced with a 35% slower progression [9.2(1.7) vs. 14.1(1.9), respectively $p=0.06$] in the earlier treatment with RP103 relative to those patients on a delayed start. These changes in the TMS were consistent across a number of subscale components of the measure including voluntary movements favoring earlier treatment. These effects on the TMS were consistent with improvements in functional measures including the UHDRS-TFC and the Independence Scale. A 23% slowing in the rate of decline in TFC [-2.0 (0.33) vs. -2.6 (0.35); $p=0.25$] and a 46% slowing in the rate of deterioration on the Independence Scale [-6.9(1.45) vs. -12.7 (1.54); $p=0.008$] was observed, favoring earlier treatment relative to a delayed start of RP103.

“We are excited to be advancing a treatment that has the potential to slow the rate of motor and functional decline in Huntington’s disease patients,” said Julie Anne Smith, Raptor’s President and CEO. “Such a therapy holds promise to be transformational for patients and is perfectly aligned with our strategic focus to develop and commercialize therapies that bring significant relief to patients and families living with life-threatening diseases. We look forward to discussing the 36-month data with the regulatory authorities and to advancing RP103 in a confirmatory study.”

“These promising data along with substantial pre-clinical data support the treatment potential of RP103 in individuals with Huntington disease,” said Christopher Ross, M.D., Ph.D., Professor of Psychiatry, Neurology, Pharmacology and Neuroscience; Director of Division of Neurology; Director of the Baltimore Huntington’s Disease Center, Johns Hopkins University School of Medicine and Co-chair Scientific Affairs Committee of the Huntington Study Group (HSG). “The HSG is excited to partner with Raptor on the future development of RP103 in Huntington disease.”

The safety profile observed for RP103 was generally consistent with what has been previously reported. The most common adverse events included nausea, vomiting, diarrhea, headache and breath odor. Three deaths due to suicide occurred during the open-label period, including two deaths that occurred in subjects in the placebo/RP103 group and one death in the RP103/RP103 group. Deaths due to suicide were generally consistent with background rates, with over 25% of patients with Huntington’s disease attempting suicide at least once and accounting for 5% to 7% of deaths, per published estimates. Suicides have not been observed in any other RP103 clinical trials or in any patients on clinical or commercial drugs.

Regulatory Update

Raptor initiated regulatory discussions with the FDA and the EMA starting in 2014 and most recently received feedback from the EMA through a Scientific Advice procedure. The outcome

of these interactions indicated that an additional study would be required. Raptor has already engaged with the EMA on the confirmatory study design through a Protocol Assistance procedure and the EMA was in agreement with key aspects of the confirmatory study design. Raptor intends to update both regulatory agencies with the 36-month data and to discuss a trial design that would support marketing authorization.

Conference Call and Webcast Information

Further comments regarding this trial will be discussed during a conference call and live audio webcast at 8:00 a.m. EST (5:00 a.m. PST) today. The live call may be accessed by dialing (877) 710-6201 for domestic callers or (616) 548-5611 for international callers and using the conference ID number 4539421. A live webcast of the conference call will be available online from the investor relations section of the company website at www.raptorpharma.com. After the call, a webcast replay will be available on the Raptor website for 90 days. A telephone replay of the call will be available by dialing (855) 859-2056 for domestic callers, or (404) 537-3406 for international callers, and using the conference ID number 4539421.

About CYST-HD

The CYST-HD study enrolled 96 patients who were randomized in a double blind, 1:1 ratio to RP103 or placebo for an initial 18-month treatment period followed by an 18-month open-label treatment with RP103. 89 patients completed the first 18-month phase. The study enrolled Stage 1 patients showing early disease symptoms with a Unified Huntington Disease Rating Scale (UHDRS), Total Motor Score (TMS) ≥ 5 , Total Functional Capacity (TFC) > 10 and a CAG repeat > 38 . The trial was conducted at eight clinical sites throughout France under a collaboration agreement between Raptor and CHU d'Angers. Clinical expenses of the study were covered by a grant from the French government (PHRC 2004-03bis CYST-HD).

The objective of the study was to evaluate the effectiveness, safety and tolerability of RP103 in the treatment of Huntington's disease. The primary endpoint of the study was the change from baseline in the TMS sub-scale of the UHDRS at 18 months of treatment in the placebo- and RP103-treated groups. Due to the duration of the study, patients were permitted to continue taking their normal medication regime which included tetrabenazine, a vesicular monoamine transporter 2 inhibitor.

Data through Month 18 were reported in February 2014. Analysis of all 96 patients enrolled in the trial showed a positive trend towards slower progression of TMS in patients treated with RP103 versus those patients on placebo, the primary endpoint of the study. TMS progression was 32% slower in patients treated with RP103 versus those treated with placebo after 18 months of treatment (4.51 vs. 6.68, respectively; $p=0.19$) in the per protocol population. In 66 patients not taking tetrabenazine, RP103 treatment resulted in a statistically significant slower progression in TMS versus the placebo group (2.84 points vs. 6.78, respectively; $p=0.03$). Adverse events were similar in the two groups and were comparable to what has been observed in other studies in this patient population. RP103 was well tolerated with 48/52 patients experiencing at least one adverse event during the 18-month study versus 38/44 on placebo. There were five patients

treated with RP103 who experienced serious adverse events compared with four patients treated with placebo.

About Huntington's Disease

Huntington's disease is a rare, progressive and hereditary neurological disease. The disease is characterized by uncontrollable movements and mood swings or depression, followed by dementia and premature death 15-20 years after diagnosis. The disease is thought to affect 30,000 patients in the U.S., a comparable number in Europe and 100,000 patients worldwide. There is no currently approved drug that slows the progression of Huntington's disease.

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. RP103 is in clinical development for Huntington's and mitochondrial diseases based on a number of proteostatic and antioxidative properties demonstrated in multiple animal models.

About PROCYSBI® (cysteamine bitartrate) delayed-release capsules

PROCYSBI is a cystine depleting agent that is approved in the U.S. for the management of nephropathic cystinosis in adults and children ages two years and older. It is contraindicated in patients with a hypersensitivity to penicillamine. The most commonly reported side effects are vomiting, abdominal pain/discomfort, headaches, nausea, diarrhea, anorexia/decreased appetite, breath odor, fatigue, dizziness, skin odor and rash. For additional information on PROCYSBI, including full prescribing information, please visit www.procysbi.com.

About Raptor Pharmaceutical

Raptor Pharmaceutical Corp. is a global biopharmaceutical company focused on the development and commercialization of transformative therapeutics for rare, debilitating and often fatal diseases. With its recent acquisition of QUINSAIR, Raptor plans to develop MP-376, the pharmaceutical product known commercially as QUINSAIR, in cystic fibrosis and at least one of bronchiectasis or lung infections associated with nontuberculous mycobacteria. In addition, Raptor is developing RP103, known commercially as PROCYSBI, in multiple therapeutic areas such as nephropathic cystinosis and Huntington's and mitochondrial diseases including Leigh syndrome. Raptor holds several orphan drug designations, including orphan drug exclusivity for nephropathic cystinosis in the U.S. and EU. 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: the potential role and future development of RP103 in the treatment of Huntington's disease;

discussions with regulatory authorities regarding confirmatory clinical trial design; and drug safety of RP103 and ongoing development of Raptor's product candidates. 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: Raptor's ability to develop RP103 in the treatment of Huntington's disease and otherwise expand the use of RP103 and MP-376 and to receive regulatory approval for other indications; Raptor's ability to market and sell QUINSAIR; continued market acceptance and sales of PROCYSBI in the U.S. and other territories; Raptor's reliance on single active pharmaceutical ingredient suppliers for PROCYSBI and QUINSAIR 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; any 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, June 30, 2015 and September 30, 2015 filed with the SEC on May 7, 2015, August 6, 2015 and November 5, 2015, respectively, 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.

COMPANY CONTACT:

Kimberly Lee, D.O.
Vice President, Corporate Strategy and Communications
Raptor Pharmaceutical Corp.
(415) 408-6351

INVESTOR CONTACT:

Westwicke Partners, LLC
Robert H. Uhl
Managing Director
(858) 356-5932
robert.uhl@westwicke.com

MEDIA CONTACT:

Cammy Duong
Canale Communications
(619) 849-5382
cammy@canalecomm.com

###