

*Raptor Pharmaceutical Co. reports that its Phase II trial for delayed release cysteamine for Huntington's Disease patients is on schedule for its previously announced summer 2010 start. The trial will take place in France. A Phase I trial conducted in the U.S. 2005 by Dr. David Dubinsky and Carolyn Gray found that a dose of 20 mg/kg per day of cysteamine was tolerable in people with Huntington's disease.*

*In 2006, research by Frédéric Saudou, Sandrine Humbert, and colleagues showed that cysteamine is neuroprotective in HD mice by increasing brain derived neurotrophic factor (BDNF) release from the cells in the brain. These compounds increase BDNF through two mechanisms. One is through an increase in the level of the heat shock DnaJ-containing protein 1b (HSJ1b), which is known to be lowered in HD patients. The other is through the inhibition of tissue transglutaminase. Lower levels of BDNF have been found in the brains of HD patients and this is thought to be a major pathology in the disease.*

*In addition, Raptor has acquired the rights to the transglutaminase inhibitors developed at the Weizmann Institute of Science in Israel and Niigata University in Japan. The company will explore the potential of these transglutaminase inhibitors in the treatment of Huntington's Disease.*

*- Marsha L. Miller, June 3, 2010*

## **Raptor Pharmaceutical Licenses Intellectual Property Related to Huntington's Disease From the Weizmann Institute of Science and Niigata University**

### **Phase II Clinical Trial Scheduled to Begin Summer**

#### **2010 Lawrence Steinman, M.D. Added to Raptor Advisory Board**

NOVATO, Calif., June 2, 2010 (GLOBE NEWSWIRE) -- Raptor Pharmaceutical Corp. ("Raptor" or the "Company") (Nasdaq:RPTP), announced that the Company has acquired an exclusive worldwide license to intellectual property related to the potential treatment of Huntington's Disease from the Weizmann Institute of Science in Israel and Niigata University in Japan. In addition, Raptor has added Professor Lawrence Steinman, M.D., an inventor on the Weizmann patent, to its Advisory Board.

The Weizmann and Niigata patents cover the use of transglutaminase inhibitors, a class of molecules chemically similar to cysteamine, in the potential treatment of Huntington's Disease and other neurological disorders. These patents add to Raptor's portfolio of intellectual property related to its programs utilizing DR Cysteamine, Raptor's proprietary formulation of delayed-release cysteamine bitartrate, licensed exclusively, with worldwide rights, from the University of California, San Diego ("UCSD").

Ted Daley, President of Raptor's Clinical Division, stated, "This exclusive license covering the Weizmann and Niigata patents significantly strengthens and expands our proprietary position as the compounds claimed in these patents are closely related to our

lead clinical compound DR Cysteamine. Additionally, this strategic move to enhance our intellectual property position coincides with our planned clinical trial of DR Cysteamine in collaboration with The Centre Hospitalier Universitaire d'Angers ("CHU d'Angers") for the potential treatment of Huntington's Disease, as well as our future plans to explore potential treatments for multiple indications utilizing cysteamine and DR Cysteamine."

Huntington's Disease is a rare, progressive, and hereditary neurological disease that often leads to death within 15 to 20 years after diagnosis. The disease is thought to affect as many as 20,000 patients in the U.S. There is no currently available drug that targets the defective gene believed to cause Huntington's Disease, which results in the degeneration of certain nerve cells in the brain. The disease is characterized by uncontrollable movements and mood swings or depression, followed by dementia.

Raptor plans to initiate a Phase II clinical trial this summer of DR Cysteamine in patients with Huntington's Disease under a previously announced collaboration agreement with CHU d'Angers of France. The Company is also developing DR Cysteamine as a potential treatment for nephropathic cystinosis ("cystinosis") and non-alcoholic steatohepatitis ("NASH").

Dr. Patrice Rioux, Raptor's Chief Medical Officer stated: "This clinical trial is intended to build on preclinical work published by Drs. Sandrine Humbert and Frederic Saudou from the Curie Institute in France. Their work demonstrated a potential mechanism for cysteamine in an in vivo preclinical Huntington's Disease model that showed increased brain and blood levels of brain-derived neurotrophic factor ("BDNF"), a growth factor known to be deficient in Huntington's Disease patients, through its inhibition of transglutaminase, a key regulating enzyme in BDNF production."

In October 2008, Raptor entered into an agreement with CHU d'Angers to participate in a Phase II clinical study in Huntington Disease patients. The study is sponsored by CHU d'Angers and will be largely funded by a grant from the French government. Raptor will provide the clinical trial materials for the study and has regulatory and commercial rights to the clinical data generated in the study. CHU d'Angers recently received ethics committee approval to begin the clinical trial. The study will be performed in eight clinical sites throughout France in a 96 patient, placebo-controlled, 18-month trial, followed by an open-label trial with all placebo patients rolling onto DR Cysteamine and all non-placebo patients continuing on DR Cysteamine for up to another 18 months. The primary end point of the trial will be based upon the Unified Huntington's Disease Rating Scale ("UHDRS"). Raptor was granted Orphan Drug Designation in the U.S. by the FDA for cysteamine as a potential treatment for Huntington's Disease in May 2008 and is in the process of applying for Orphan Drug Designation with the European Medicines Agency ("EMA").

Separately, Raptor announced that Dr. Steinman will join the Company's Advisory Board. Dr. Steinman is a leading researcher in neurological disorders including Huntington's Disease and Multiple Sclerosis ("MS"), and currently serves as the George A. Zimmermann Professor of Neurology and Neurological Sciences, Pediatrics and

Genetics at Stanford University. He is a member of the Institute of Medicine of the National Academy of Sciences. He was also co-founder of Bay Hill Therapeutics and Neurocrine Biosciences. The addition of Dr. Steinman to the Advisory Board brings world-class expertise in the development of CNS drugs. He has developed two antigen specific therapies, using DNA vaccines, for MS and Type 1 diabetes. He was senior author on the seminal 1992 Nature article that reported the key role of a particular integrin in brain inflammation which led to the development of the drug Tysabri.

Dr. Steinman said, "I'm excited to join the Raptor team and work with them to examine the possible benefits of DR Cysteamine in the treatment of Huntington's Disease. In previous animal studies published in the journal, Nature Medicine, the team that I worked with found that in a Huntington's Disease preclinical model those given cysteamine had fewer tremors and other abnormal movements and reduced weight loss, as compared to the untreated subjects. Additionally, cysteamine appeared to work differently than other Huntington's Disease drugs, and it may be capable of adding to the benefits of existing therapies. This could be a powerful option in the treatment of Huntington's Disease."

### **About Cysteamine and DR Cysteamine**

Immediate-release cysteamine bitartrate is approved for sale by the FDA and EMEA to treat cystinosis, a rare, genetic lysosomal storage disease. DR Cysteamine is proprietary enteric-coated, microbead formulation of cysteamine bitartrate in gelatin capsules designed to potentially reduce dosing frequency and gastrointestinal side effects.

Raptor obtained an exclusive, worldwide license from UCSD for the development of cysteamine and DR Cysteamine for a number of potential indications including Huntington's Disease, cystinosis, NASH and Batten Disease. Raptor recently presented positive Phase IIa clinical trial data of cysteamine in NASH patients and recently announced positive Phase IIb clinical trial data in cystinosis patients. Raptor is working with the FDA to finalize a Special Protocol Assessment in the coming weeks and plans to initiate its pivotal Phase III study of DR Cysteamine in cystinosis patients shortly thereafter.

### **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](http://www.raptorpharma.com).