



August 20, 2015

## **Raptor Pharmaceutical Corp. Expands Rare Disease Portfolio With the Acquisition of Quinsair(TM)**

***First Inhaled Fluoroquinolone Therapy for Patients with Cystic Fibrosis***

***Approval Received in the European Union and Canada***

***Expansion of Pipeline with Multiple Late-Stage Orphan Indication Opportunities***

Raptor to Host Conference Call and Webcast Today at 5:30 p.m. EDT/2:30 p.m. PDT

NOVATO, Calif., Aug. 20, 2015 (GLOBE NEWSWIRE) -- Raptor Pharmaceutical Corp. (Nasdaq:RPTP) announced today that it has signed a definitive agreement with Tripex Pharmaceuticals to acquire Quinsair, the first inhaled fluoroquinolone approved for the management of chronic pulmonary infections due to *Pseudomonas aeruginosa* in adults with cystic fibrosis, expanding its portfolio of rare disease therapies.

Quinsair received marketing authorization by the European Commission and Health Canada in March 2015 and June 2015, respectively. Quinsair, a twice-a-day treatment, contains levofloxacin, a proven antimicrobial active against a wide range of gram negative and gram positive bacteria. Raptor plans to launch Quinsair in Europe and Canada in the first half of 2016, and to discuss the path to potential approval in the same indication in the U.S with the FDA in 2016.

In addition to cystic fibrosis, Quinsair has development potential in two additional orphan diseases with significant unmet need, bronchiectasis (BE) and nontuberculous mycobacteria (NTM) lung infections, for which there are currently few therapeutic options. BE is characterized by abnormal dilatation and destruction of lung bronchi and bronchioles due to chronic recurring infection and long-term inflammation which leads to frequent hospitalizations. NTM are a group of microbes that cause severe and recurrent lung infections, often in individuals who are immune-compromised or who have structural lung disease, such as bronchiectasis. Raptor is evaluating Quinsair's potential in these therapeutic indications and intends to initiate clinical programs in 2016 in at least one of the indications.

"The Quinsair acquisition is transformational for Raptor and delivers on our strategic focus to develop and commercialize therapies that bring significant relief to patients and families living with life-threatening diseases," stated Julie Anne Smith, President and CEO of Raptor. "This acquisition expands our portfolio and leverages both our commercial and development expertise in rare diseases. By acquiring Quinsair prior to its launch, we will be able to exclusively shape its commercial strategy and potential in cystic fibrosis and other rare diseases."

"Quinsair is an important new addition to the options that we can offer adult European and Canadian CF patients today," stated Patrick Flume, M.D., Professor of Medicine and Pediatrics at the Medical University of South Carolina. "Since it is a new class of inhaled antibiotics, Quinsair's availability is an important step in addressing an unmet need for the CF community. I'm especially excited about the possibilities to broaden the availability of a drug like this for patients with non-CF bronchiectasis and pulmonary nontuberculous mycobacterial infections, for whom there are limited treatment options."

Under the terms of the agreement, Raptor will pay \$68.4 million upfront, with up to \$34.2 million of the closing consideration payable in Raptor common stock at Raptor's election plus contingent payments of up to \$350 million associated with development, regulatory and commercial milestones, a portion of which is payable in Raptor common stock at Raptor's election, and a single-digit royalty on future global net sales. In addition, Raptor will have single-digit contingent obligations to two additional parties involved in Quinsair's development. Raptor is acquiring exclusive global rights and assets to develop, manufacture and commercialize Quinsair. The transaction is expected to close in the third quarter of 2015, subject to customary closing conditions including expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Act.

"We are extremely excited to be entering into this agreement with Raptor. We strongly believe Raptor has the global presence and experience in successfully developing and launching orphan products to help realize Quinsair's full commercial potential and bring this drug to the patients that need it the most," stated Daniel Burgess, Chief Executive Officer of Tripex Pharmaceuticals.

With this acquisition, Raptor is reiterating its 2015 revenue guidance of \$80 to \$90 million and also maintaining its 2015

guidance for non-GAAP operating expenses, which exclude cost of goods and non-cash expenses, such as stock-based compensation and amortization of transaction-related intangible assets, of between \$115 to \$125 million.

## Conference Call and Webcast Information

Further comments regarding this acquisition will be discussed during a conference call and live audio webcast at 5:30 p.m. EDT (2:30 p.m. PDT) 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 14694258. A live webcast of the conference call will be available online from the investor relations section of the company website at [www.raptorpharma.com](http://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 14694258.

## 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 Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-threatening genetic disease affecting more than 75,000 people in North America, Europe and Australia. CF is caused by a mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. Defective or missing CFTR protein causes poor flow of salt and water into or out of the cell in several organs, including the lungs. This leads to the buildup of abnormally thick secretions that can cause chronic lung infections and progressive lung damage in many patients that eventually leads to death.

## 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), pediatric nonalcoholic steatohepatitis (NASH), 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](http://www.raptorpharma.com).

## Non-GAAP Financial Information and Other Disclosures

Raptor uses non-GAAP financial measures to assess and analyze its operational results and trends and to make financial and operational decisions. Raptor believes non-GAAP financial measures are also useful to investors because they provide greater transparency regarding Raptor's operating performance and exclude non-cash which fluctuate from period to period based on factors that are not within the company's control, such as the company's stock price. In addition, Raptor does not believe that non-cash amortization of transaction-related intangible assets such as those that could occur in connection with the Quinsair acquisition are relevant to assessing Raptor's operating performance or estimating the potential long-term performance of the acquired assets. Non-GAAP operating expenses should not be considered as a substitute for GAAP operating expenses, should only be used to supplement an understanding of Raptor's operating results as reported under GAAP and is unlikely to be comparable with non-GAAP information provided by other companies. Raptor provides operating expense guidance on a non-GAAP basis as projected GAAP operating expense for 2015 is not readily determinable due to the volatility of the company's common stock, which directly affects stock-based compensation expense.

## 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 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 anticipated acquisition of Quinsair and the timing of closing of the acquisition; Raptor's plans to launch Quinsair in Europe and Canada and the timing of launch; Raptor's intention to discuss the path to potential approval in cystic fibrosis in the U.S. with the FDA in 2016; the potential of Quinsair in two additional orphan diseases and Raptor's intention to initiate clinical programs in 2016 for at least one of these

indications; the effect of the acquisition on Raptor and delivery on its strategic focus; expansion of Raptor's portfolio and leverage of its commercial and development expertise; Raptor's ability to shape Quinsair's commercial strategy and potential; 2015 financial guidance; and the impact of the acquisition on Raptor's financial results. 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. 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; Raptor's ability to market and sell Quinsair; market acceptance and sales of PROCYSBI in the U.S. and other territories; Raptor's ability to expand the use of RP103 and potentially Quinsair 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 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 for the twelve months ended December 31, 2014 on Form 10-K filed with the SEC on March 2, 2015, Raptor's quarterly reports for the quarterly periods ended March 31, 2015 and June 30, 2015 on Form 10-Q filed with the SEC on May 7, 2015 and August 6, 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.

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