



October 31, 2016

## **Rexahn Pharmaceuticals Receives a Notice of Allowance from the U.S. Patent and Trademark Office for a Patent Covering the Manufacturing of RX-3117 — A Cancer Cell Specific Anticancer Agent**

### **RX-3117 is Currently in a Phase Ib/IIa Clinical Trial in Patients with Metastatic Pancreatic Cancer**

ROCKVILLE, Md., Oct. 31, 2016 (GLOBE NEWSWIRE) -- Rexahn Pharmaceuticals, Inc. (NYSE MKT:RNN), a clinical stage biopharmaceutical company developing best-in-class therapeutics for the treatment of cancer, today announced that it has received a notice of allowance from the United States Patent and Trademark Office (USPTO) for a patent for claims related to the synthesis of its novel anti-cancer investigational drug candidate, RX-3117. RX-3117 is currently in stage 2 of a Phase Ib/IIa clinical trial in patients with metastatic pancreatic cancer.

"Rexahn is pleased to expand the intellectual property protection for RX-3117, which has shown preliminary efficacy in stage 1 of a Phase Ib/IIa clinical trial in patients with metastatic pancreatic cancer," commented Peter D. Suzdak, Ph.D., Chief Executive Officer of Rexahn. "This latest patent for RX-3117 is an important addition to our overall intellectual property estate and further strengthens our global patent position for RX-3117."

Data from stage 1 of a Phase Ib/IIa clinical trial was recently presented at The European Society for Medical Oncology (ESMO) congress. Patients with relapsed or refractory metastatic pancreatic cancer, 88% of whom had received 4 or more prior cancer therapies, were treated with RX-3117. There are no approved treatments for pancreatic cancer patients who have failed three or more prior therapies and their survival is usually less than 2 months. These patients would usually be offered palliative or best supportive care. In this study more than 20% of patients treated with RX-3117 exhibited progression free survival of greater than 4 months. An additional 20%, for a total of 40%, of the patients exhibited progression free survival of 2.5 months. Based on positive results, stage 2 of the study has been initiated and an additional 40 pancreatic cancer patients are currently being enrolled. Rexahn has received U.S. Food and Drug Administration (FDA) Orphan Drug Designation for RX-3117 for pancreatic cancer.

#### **About RX-3117**

RX-3117 is a novel, investigational small molecule nucleoside compound. Once intracellularly activated (phosphorylated) by UCK2, it is incorporated into the DNA or RNA of cells and inhibits both DNA and RNA synthesis, which induces apoptotic cell death of tumor cells. UCK2 is highly overexpressed in various human cancer cells. Preclinical studies have shown that RX-3117 has shown broad spectrum anti-tumor activity against over 100 different human cancer cell lines and efficacy in 17 different mouse xenograft models including pancreatic, bladder, lung, cervical and colon cancers, as well as gemcitabine resistant cancer cells. Importantly, RX-3117 still retains its full anti-tumor activity in human cancer cell lines made resistant to the anti-tumor effects of gemcitabine.

Rexahn has previously reported the completion of an exploratory Phase I clinical trial of RX-3117 in cancer patients conducted in Europe, to investigate the oral bioavailability, safety and tolerability of the compound. In this study, oral administration of a 50 mg dose of RX-3117 showed an oral bioavailability of 56% and a plasma half-life ( $T_{1/2}$ ) of 14 hours. In addition, RX-3117 appeared to be safe and well tolerated in all subjects throughout the dose range tested.

In June 2016, final results from the Phase Ib clinical trial of RX-3117 were presented at the American Society of Clinical Oncology (ASCO) Annual Meeting showing encouraging evidence of the single agent activity. Patients in the study were heavily pre-treated, and had generally received four or more cancer therapies prior to enrollment. In this study, 12 patients experienced stable disease persisting for up to 276 days and three patients showed evidence of tumor burden reduction. A maximum tolerated dose of 700 mg was identified in the study and will be administered for five consecutive days, with two days off, for three treatment weeks, followed by a week of rest. At the doses tested to date, RX-3117, administered orally, appeared to be safe and well tolerated with a predictable pharmacokinetic profile for an orally-administered route of therapy.

Based on these data, Rexahn initiated a two stage Phase Ib/IIa clinical trial of RX-3117 in patients with relapsed or refractory pancreatic cancer to further evaluate the safety and anti-cancer properties of this compound. Clinical data from stage 1 of the clinical trial was presented at the European Society for Medical Oncology (ESMO) congress in October 2016. The ongoing Phase Ib/IIa clinical trial is a multicenter, open-label single-agent study of RX-3117 being conducted at 10 clinical

centers in the United States. Patients receive a 700 mg daily oral dose of RX-3117, five times weekly on a three weeks on, one week off dosing schedule in a 28 day cycle for up to eight treatment cycles, or until their disease progresses. The study follows a two-stage design. In stage 1 of the trial, up to 10 patients with relapsed or refractory metastatic pancreatic cancer were enrolled who had actively progressing disease with 88% of them having received 4 or more prior cancer therapies. These patients would usually be offered palliative or best supportive care. There are no approved treatments for pancreatic cancer patients who have failed three or more prior therapies and their survival is usually less than 2 months. In this study more than 20% of patients treated with RX-3117 exhibited progression free survival of greater than 4 months. An additional 20%, for a total of 40%, of the patients exhibited progression free survival of 2.5 months. RX-3117 was shown to be safe and well tolerated in this patient group. Based on these data, stage 2 of the study has been initiated and an additional 40 pancreatic cancer patients are currently being enrolled into the study.

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### **About Rexahn Pharmaceuticals, Inc.**

Rexahn Pharmaceuticals Inc. (NYSE MKT:RNN) is a clinical stage biopharmaceutical company dedicated to developing novel, best-in-class therapeutics for the treatment of cancer. The Company's mission is to improve the lives of cancer patients by developing next generation cancer therapies that are designed to maximize efficacy while minimizing the toxicity and side effects traditionally associated with cancer treatment. Rexahn's product candidates work by targeting and neutralizing specific proteins believed to be involved in the complex biological cascade that leads to cancer cell growth. Preclinical studies show that certain of Rexahn's product candidates may be effective against multiple types of cancer, drug resistant cancers, and difficult-to-treat cancers, and others may augment the effectiveness of current FDA-approved cancer treatments. The Company has a broad oncology pipeline that includes three anti-cancer compounds currently in clinical development: RX-3117, Supinixin™, and Archexin®, and a novel nanopolymer conjugate platform technology that targets the delivery of FDA approved chemotherapies directly into cancer tumor cells. For more information about the Company and its oncology programs, please visit [www.rexahn.com](http://www.rexahn.com).

### **Safe Harbor**

To the extent any statements made in this press release deal with information that is not historical, these are forward-looking statements under the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements about Rexahn's plans, objectives, expectations and intentions with respect to cash flow requirements, future operations and products, enrollments in clinical trials, the path of clinical trials and development activities, and other statements identified by words such as "will," "potential," "could," "can," "believe," "intends," "continue," "plans," "expects," "anticipates," "estimates," "may," other words of similar meaning or the use of future dates. Forward-looking statements by their nature address matters that are, to different degrees, uncertain. Uncertainties and risks may cause Rexahn's actual results to be materially different than those expressed in or implied by Rexahn's forward-looking statements. For Rexahn, particular uncertainties and risks include, among others, understandings and beliefs regarding the role of certain biological mechanisms and processes in cancer; drug candidates being in early stages of development, including in preclinical development; the ability to initially develop drug candidates for orphan indications to reduce the time-to-market and take advantage of certain incentives provided by the U.S. Food and Drug Administration; and the ability to transition from our initial focus on developing drug candidates for orphan indications to candidates for more highly prevalent indications. More detailed information on these and additional factors that could affect Rexahn's actual results are described in Rexahn's filings with the Securities and Exchange Commission, including its most recent annual report on Form 10-K and subsequent quarterly reports on Form 10-Q. All forward-looking statements in this news release speak only as of the date of this news release. Rexahn undertakes no obligation to update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.

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