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## **Rexahn Pharmaceuticals Announces Poster Presentation of RX-3117 Data in Metastatic Pancreatic Patients at the American Society of Clinical Oncology Gastrointestinal Cancers (ASCO GI) 2018 Annual Meeting**

*Encouraging Progression Free Survival and Evidence of Tumor Shrinkage Observed in Patients with Metastatic Pancreatic Cancer Resistant to Gemcitabine who had Failed on Multiple Prior Treatments*

*Rexahn is Progressing Development of RX-3117 in Combination with Abraxane® in Newly Diagnosed Pancreatic Cancer Patients*

ROCKVILLE, Md., Jan. 22, 2018 (GLOBE NEWSWIRE) -- Rexahn Pharmaceuticals, Inc. (NYSE American:RNN), a clinical stage biopharmaceutical company developing innovative, targeted therapeutics for the treatment of cancer, today announced the final data from a Phase IIa Clinical Trial of RX-3117 in metastatic pancreatic cancer were presented at the American Society of Clinical Oncology Gastrointestinal Cancers (ASCO GI) 2018 annual meeting on January 19, 2018.

The poster presentation reported the final data on the Phase IIa study of RX-3117 monotherapy in metastatic pancreatic cancer. A total of 46 patients with metastatic pancreatic cancer were enrolled into the study. Seventy-eight percent of the patients had received two or more prior cytotoxic therapies and 93% of the patients had progressed after receiving gemcitabine therapy.

Forty-three patients were included in the efficacy analysis. Of these, 31% of patients had an increase in progression free survival for two months or more and five patients, or 12%, had disease stabilization for greater than four months. One patient who had three prior treatments for metastatic pancreatic cancer (including gemcitabine) had a partial response ( $\geq$  30% reduction in total tumor volume). There are no published studies on gemcitabine monotherapy in heavily pre-treated pancreatic cancer patients, but the data compare favorably to published data on gemcitabine when used in newly diagnosed patients. Disease stabilization with gemcitabine monotherapy in first line, newly diagnosed metastatic pancreatic cancer patients is reported to be 20% compared to 31% in later stage patients in the present study<sup>1</sup>. RX-3117 was well tolerated.

"The data provide a strong basis for continuing the development of RX-3117 in newly diagnosed metastatic pancreatic cancer patients," said Ely Benaim, M.D., Chief Medical Officer, Rexahn. "It is especially exciting to see evidence of efficacy in patients whose tumors had developed resistance to multiple prior therapies including gemcitabine. We are very pleased with the outcome in this first Phase II study and we will continue to progress development of RX-3117 in combination with Abraxane (nab-paclitaxel) as first line treatment for patients with metastatic pancreatic cancer. This represents the largest segment of the pancreatic cancer patient population and the patients who are likely to benefit most from RX-3117."

"Patients with metastatic pancreatic cancer, who have already developed resistance to gemcitabine and other anticancer treatments, are difficult to treat," said Dr. Vincent Chung, MD, FACP, Associate Clinical Professor at City of Hope Comprehensive Cancer Center, Duarte, California. "Usually they receive supportive and palliative care only. In a heavily pretreated population, we saw both tumor reduction and prolonged stable disease in a meaningful proportion of patients which is encouraging."

The Phase IIa clinical trial was a multicenter, open-label single-agent study of RX-3117 conducted at eight clinical centers in the United States. Patients received 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 progressed.

The data from the completed Phase IIa clinical trial in metastatic pancreatic cancer were presented on Friday January 19, 2018 in a poster presentation entitled ***RX-3117: Activity of an Oral Antimetabolite Nucleoside in Subjects with Pancreatic Cancer — Preliminary Results of Stage II of the Phase IIa Study*** authored by Drs. V. Chung (City of Hope, Duarte, CA), J.R. Merchan (Sylvester Comprehensive Cancer Center, University of Miami Miller School of Medicine, Miami, FL), A.J. Ocean (Weill Cornell Medical College, New York, NY), D.W. Rasco (South Texas Accelerated Research Therapeutics, San Antonio, TX), H.M. Babiker (University of Arizona Cancer Center, Tucson, AZ), I.Oliff (Orchard Healthcare Research, Inc., Skokie, IL), R. K. Paluri (University of Alabama at Birmingham, Birmingham, AL), E. Roman (Lakes Research, Miami Lakes, FL) and Rexahn Pharmaceuticals.

A copy of the ASCO GI poster can be viewed on the company's website at <https://rexahn.com/cms/media-center/publication/posters/>

1. Fernandes BM et al. J Clin Oncol 2017;35:4 suppl. 489

### **About RX-3117**

RX-3117 is a novel, investigational, oral, 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.

Rexahn has previously completed a Phase Ib clinical trial of RX-3117 in patients with solid tumors 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 the Phase Ib study, 12 patients experienced stable disease persisting for up to 276 days and three patients showed evidence of tumor burden reduction. The maximum tolerated dose was identified as 700 mg administered for five consecutive days, followed by 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.

**Pancreatic Cancer:** Rexahn completed a two stage Phase IIa clinical trial of RX-3117 in patients with relapsed or refractory pancreatic cancer. Preliminary data were presented at the European Society of Medical Oncology (ESMO) Congress in October 2016 and the final data were presented at the American Society of Clinical Oncology Gastrointestinal Cancers (ASCO GI) 2018 Annual Meeting as reported here. Patients in the trial received a 700 mg daily oral dose of RX-3117, five times weekly for three weeks in a 28 day cycle for up to eight treatment cycles, or until their disease progresses.

A Phase IIa clinical proof-of-concept study of RX-3117 in combination with Abraxane (nab-paclitaxel) in first line metastatic pancreatic patients is ongoing. The study is an open-label evaluation of the safety and efficacy of RX-3117 in combination with Abraxane (nab-paclitaxel) in patients who have had no prior chemotherapies. The study is a two stage study. The first stage is designed to determine the optimum dose of RX-3117 and Abraxane to be evaluated in the second stage. Up to 50 patients will be enrolled into the second stage of the study and the primary endpoint is progression free survival.

**Advanced and metastatic bladder cancer:** In 2016, Rexahn initiated a two stage Phase IIa clinical trial in advanced and metastatic bladder cancer. The clinical trial is a multicenter, open-label single-agent study of RX-3117 being conducted at 10 clinical centers in the United States. RX-3117 is being administered orally five times weekly on a three weeks on, one week off dosing schedule. The study is following a two-stage design. In the initial stage, 10 patients with advanced and metastatic bladder cancer will be enrolled. If there is an increase in progression free survival of greater than four months in 20% of the patients or a reduction in tumor size, then an additional cohort of patients will be enrolled. At the 2017 European Society for Medical Oncology (ESMO) congress Rexahn presented the initial data from this trial. Four of ten patients treated with RX-3117 exhibited progression free survival of greater than five months and one of these patients is continuing in the study with stable disease at 259 days. Two patients had reductions of 19% and 15% in tumor size. As a result, the study began enrolling an additional 10 patients in this second stage. There were no dose-limiting toxicities identified in the study.

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

Rexahn Pharmaceuticals Inc. (NYSE MKT:RNN) is a clinical stage biopharmaceutical company dedicated to developing novel, targeted 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. Pre-clinical 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-5902 (Supinoxin™), RX-3117, and Archexin®, and a novel nanopolymer-based drug delivery platform technology that may increase the bio-availability of FDA-approved chemotherapies. 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 clinical 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; the ability to transition from our initial focus on developing drug candidates for orphan indications to candidates for more highly prevalent indications; and the expecting timing of results from our clinical trials. 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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