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Rexahn Pharmaceuticals Presents an Update of the Ongoing Phase IIa Clinical Trial of RX-3117 in Metastatic Pancreatic Cancer at the American Society of Clinical Oncology (ASCO) 2017 Gastrointestinal Cancers Symposium

Evidence of Prolonged Progression Free Survival in Pancreatic Cancer Patients Treated with RX-3117 and for Whom Three or More Prior Therapies had been Ineffective

ROCKVILLE, Md., Jan. 20, 2017 (GLOBE NEWSWIRE) -- Rexahn Pharmaceuticals, Inc. (NYSE MKT:RNN), a clinical stage biopharmaceutical company developing next generation targeted therapeutics for the treatment of cancer, today announced an update on the safety and efficacy of RX-3117 in an ongoing Phase IIa clinical trial in metastatic pancreatic cancer at the American Society for Clinical Oncology (ASCO) 2017 Gastrointestinal Cancer Symposium in San Francisco California.

“The data on progression free survival in metastatic pancreatic cancer patients treated with RX-3117 is very encouraging with 20% of patients exhibiting progression free survival of greater than 5.6 months (with one patient having progression free survival of 7.2 months). A majority of the patients enrolled in the trial have already failed 3 or more prior cancer therapies. Current options for these patients are usually limited to palliative or best supportive care; there are no drugs approved for metastatic pancreatic cancer patients that have failed two or more prior therapies,” said Ely Benaim, M.D., Chief Medical Officer for Rexahn.

“Rexahn’s development strategy for RX-3117 in pancreatic cancer is to continue to develop RX-3117 as monotherapy for patients with metastatic disease who have failed on two or more prior therapies, and also, in parallel, to develop RX-3117 in combination with Abraxane® (paclitaxel protein bound) for patients with metastatic pancreatic cancer who have received no prior chemotherapy treatment. Since there are no drugs approved for patients who have failed two or more therapies, there may be an accelerated regulatory pathway for approval for this patient population, assuming we continue to see efficacy with RX-3117. In addition, during the first quarter, Rexahn plans to initiate a Phase IIa clinical trial of RX-3117 in combination with Abraxane® in newly diagnosed metastatic pancreatic cancer patients. We expect the initial data from this trial will be available in early 2018,” commented Peter D. Suzdak, Ph.D. CEO of Rexahn.

RX-3117 Phase IIa Clinical Data

The updated efficacy data for RX-3117 from an ongoing Phase IIa clinical trial in metastatic pancreatic cancer were presented on Friday January 20, 2017 in a poster presentation entitled “RX3117, An Oral Antimetabolite Nucleoside Shows Activity in Subjects with Pancreatic Cancer. Preliminary Results of Stage 1 of the Phase 2a Study” authored by Drew W. Rasco (South Texas Accelerated Research Therapeutics, San Antonio, Texas), Jaime R. Merchan (Sylvester Comprehensive Cancer Center, University of Miami Miller School of Medicine, Miami, Florida) and Rexahn Collaborators.

Patients enrolled into stage 1 of the clinical trial had actively progressing disease with 55% of them having received 4 or more prior cancer therapies (including 5-FU and gemcitabine-based therapies). These patients would usually be offered palliative or 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 the current study more than 20% of patients treated with RX-3117 exhibited progression free survival of greater than 5.6 months (with one patient having progression free survival of 7.2 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. The most frequently reported drug-related adverse events were mild to moderate fatigue and diarrhea. Patients in Stage 1 of the clinical trial are still being monitored for survival. However, since the predefined efficacy criteria have been achieved, stage 2 of the study has been initiated which entails enrolling an additional 40 metastatic pancreatic cancer patients. An initial data read out from stage 2 of the trial is expected in late 2Q or early 3Q 2017.

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. Based on predefined criteria, if 20% or more of the patients have progression free survival of $\geq$ 4 months, or an objective clinical response rate and reduction in tumor size, then an additional 40 pancreatic cancer patients can be enrolled into stage 2.

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 a 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 is developing RX-3117 for metastatic pancreatic cancer and for muscle-invasive bladder cancer.

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 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 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. The Phase IIa clinical trial is a multi-center study that will evaluate the safety and efficacy of RX-3117 in this target patient population. Patients in the trial will receive 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. The study follows a two-stage design. In stage 1 of the trial, 10 patients with relapsed or refractory metastatic pancreatic cancer were enrolled and 20% of the patients achieved the predefined criteria (progression free survival of $\geq$ 4 months) which triggered the enrollment of an additional 40 pancreatic cancer patients (stage 2). Stage 2 is ongoing and an initial data readout is expected late in 2/3Q 2017.

Rexahn has also initiated the first stage of a Phase Ib/IIa study of RX-3117 in patients with muscle-invasive bladder cancer. The initial readout of the first stage is expected 2/3Q 2017.

Rexahn has received U.S. Food and Drug Administration (FDA) Orphan Drug Designation for RX-3117 for pancreatic cancer.

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. 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: 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.

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 pre-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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