

October 10, 2016



Rexahn Pharmaceuticals Presents Preliminary Efficacy Data from Phase Ib/IIa Trial of RX-3117 in Metastatic Pancreatic Cancer and Supinoxin™ Phase I Trial at 2016 European Society for Medical Oncology (ESMO) Congress

RX-3117 showed Preliminary Efficacy in Pancreatic Cancer Patients for Whom Three or More Prior Therapies had been Ineffective

Supinoxin™ was Safe and Well Tolerated in Patients with Advanced and Metastatic Solid Tumors and Showed Preliminary Evidence of Clinical Activity

ROCKVILLE, Md., Oct. 10, 2016 (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 preliminary efficacy data for RX-3117 in an ongoing Phase Ib/IIa clinical trial in patients with metastatic pancreatic cancer and provided an update on the Phase I clinical trial with Supinoxin™ (RX-5902) at the 2016 European Society for Medical Oncology (ESMO) Congress in Copenhagen, Denmark.

“The RX-3117 clinical data are very encouraging, since there are no approved treatments for pancreatic cancer patients who have failed two or more prior therapies. Current options for these patients are usually limited to palliative or best supportive care. Having patients who show responses beyond 4 months in this refractory patient population is certainly very exciting,” said Ely Benaim, M.D., Chief Medical Officer for Rexahn.

“RX-3117 is a novel oral antimetabolite that has shown interesting clinical activity against several cancers, including pancreatic cancer. I am encouraged by the current data and look forward to the additional data being generated in stage 2 of the ongoing Phase Ib/IIa clinical trial in pancreatic cancer patients,” commented Manish A. Shah, M.D., Director, Gastrointestinal Oncology Program, Sandra and Edward Meyer Cancer Center at Weill Cornell Medicine.

RX-3117 Phase Ib/IIa clinical data

The preliminary efficacy data for RX-3117 from an ongoing Phase Ib/IIa clinical trial in metastatic pancreatic cancer were presented on Monday October 10, 2016 in a poster presentation entitled “RX-3117, An Oral Antimetabolite to Treat Advanced Solid Tumors (ST): Phase 1 and Ongoing Phase 2a Results” authored by Drs. Drew Rasco (South Texas

Accelerated Research Therapeutics, San Antonio, TX), Jaime R. Merchan (Sylvester Comprehensive Cancer Center, University of Miami Miller School of Medicine, Miami, FL) and Rexahn Collaborators.

Patients enrolled into stage 1 of the clinical trial had actively progressing disease with 88% 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 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 the current 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. The most frequently reported drug related adverse events were mild to moderate fatigue, diarrhea and decreased white blood cell counts. Stage 1 of the clinical study is still on-going. However, since the predefined efficacy criteria have been achieved, stage 2 of the study has been initiated.

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 ≥ 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.

Supinoxin™ Phase I clinical data

Clinical data from the ongoing Phase I clinical trial with Supinoxin were presented on Monday October 10, 2016 in a poster presentation entitled "Phase 1/2a Study of RX-5902 in Advanced Solid Tumors (ST): an Orally Bioavailable Inhibitor of Phosphorylated p68 and Modulator of β -Catenin Nuclear Translocation" authored by Drs. S. Gail Eckhardt (University of Colorado Cancer Center), W. Larry Gluck (Translational Oncology Research, Greenville, SC), Martin Gutierrez (John Theurer Cancer Center, Hackensack University Medical Center, NJ) and Rexahn collaborators.

The updated results from the ongoing Phase I clinical trial continue to show intriguing evidence of single-agent, clinical activity of Supinoxin. Supinoxin is safe and well tolerated at the doses and dosing schedules tested with no dose limiting toxicities or treatment-related serious adverse events. The most frequently reported drug related adverse events were mild nausea, vomiting and fatigue. Initial signs of clinical activity have been observed in patients with breast, neuroendocrine, paraganglioma, head and neck and colorectal cancers, demonstrating stable disease for up to 880 days. Of these patients approximately 55% had received four or more therapies prior to their enrollment in the Phase I clinical study. The study protocol has recently been amended to target triple negative breast cancer patients in a 2-stage Phase IIa design.

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 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 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. The Phase Ib/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 be receiving 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, 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 ≥ 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. Secondary endpoints include time to disease progression, overall response rate and duration of response, as well as pharmacokinetic assessments and safety parameters.

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

About Supinoxin™ (RX-5902)

Supinoxin™ (RX-5902) is an orally administered, potential first-in-class, small molecule inhibitor of phosphorylated-p68 (P-p68). P-p68, which is selectively overexpressed in cancer cells and is absent in normal tissue, increases the activity of multiple cancer related genes

including cyclin D1, c-jun and c-myc, and plays a role in tumor progression and metastasis. Overexpression of phosphorylated-p68 has been observed in solid tumors, such as melanoma, colon, breast, ovarian and lung tumors. In preclinical studies, Supinoxin has been shown to inhibit proliferation of cells in over 100 different human cancer cell lines, including breast, colon, pancreas, ovarian, and stomach cancers, and showed potent activity in drug-resistant cancer cells. In preclinical animal models, where human cancer cells from breast, ovarian, melanoma, pancreas, or renal tumors were grafted into animals, treatment with Supinoxin resulted in a significant reduction in tumor growth.

Supinoxin is currently being evaluated in a Phase I dose-escalation clinical trial in cancer patients with solid tumors designed to evaluate the safety, tolerability, dose-limiting toxicities and maximum tolerated dose (MTD). Secondary endpoints include pharmacokinetic analysis and an evaluation of the preliminary anti-tumor effects of Supinoxin. This trial is being conducted at three clinical oncology centers in the United States. Each patient has the ability to continue on the drug if no disease progression is seen. Patients are assessed by CT or MRI prior to the start of therapy and after every two cycles of therapy to assess tumor progression.

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; 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.

LifeSci Advisors, LLC
Matthew P. Duffy
(212)-915-0685
matthew@lifesciadvisors.com



Source: Rexahn Pharmaceuticals