

November 14, 2013



# Rexahn Pharmaceuticals Reports Third Quarter Financial Results and Pipeline Update

- *Supinoxin™ Phase I clinical trial enters third dosing group; preliminary data expected in the first quarter of 2014*
- *Lead indication, metastatic renal cell carcinoma, chosen for Archexin® Phase IIa trial to begin December 2013*

ROCKVILLE, Md.--(BUSINESS WIRE)-- Rexahn Pharmaceuticals, Inc. (NYSE MKT: RNN) a clinical stage biopharmaceutical company developing best-in-class therapeutics for the treatment of cancer is providing a quarterly update today on its three clinical development programs and financial results.

"We are excited about the progress of the Supinoxin™, RX-3117 and Archexin® clinical development programs. As we continue through the end of 2013, and into the first quarter of 2014, we look forward to updating our shareholders on the initiation of the Phase I clinical trial with RX-3117, the initiation of the Phase IIa clinical trial with Archexin in metastatic renal cell carcinoma, and the initial clinical data from the Phase I clinical trial with Supinoxin," commented Rexahn's Chief Executive Officer Peter D. Suzdak, PhD. "We are also pleased that we were able to strengthen the balance sheet recently which will allow us to increase the momentum for the clinical development of our pipeline."

## **Pipeline Update:**

### ***Supinoxin™ (RX-5902)***

The Phase I dose-escalation clinical trial of Supinoxin (RX-5902) in cancer patients with solid tumors began enrolling patients in August 2013. Patient enrollment in the third dosing group was recently initiated, and Rexahn anticipates updating investors with available clinical data from this trial by the end of the first quarter of 2014. The Phase I trial is designed to evaluate the safety, tolerability, and the maximal tolerated dose of Supinoxin. This evaluation is being conducted in three clinical oncology centers in the United States. Each patient has the ability to continue on the drug up to six cycles of treatment. Patients are assessed by CT or MRI prior to the start of therapy and after every two cycles of therapy to track tumor progression.

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

Rexahn is preparing to initiate a Phase I clinical trial of RX-3117 in cancer patients with solid tumors in December 2013. The design of this study is based partially on the initial data obtained in an exploratory Phase I clinical trial of RX-3117 in cancer patients conducted in Europe in 2012. This upcoming dose-escalation clinical trial will be conducted in multiple

clinical sites in the U.S. for cancer patients with solid tumors. Patients will receive RX-3117 orally three times a week for three weeks followed by one week off. Patients will have the ability to continue on the drug up to eight cycles of treatment. The decision to enroll the next group of patients and escalate the dose will be made after one cycle of treatment, based on safety and tolerability. Patients will be assessed for tumor progression by CT or MRI prior to the start of therapy and after every two cycles of therapy.

Rexahn is in active discussions with potential licensing partners for RX-3117 and will update the financial community as appropriate.

In September 2013, Prof. Dr. Godefridus J. (Frits) Peters, Head Laboratory Medical Oncology, VU University Medical Center, Amsterdam, The Netherlands, published a peer reviewed publication entitled "Metabolism, mechanism of action and sensitivity profile of fluorocyclopentenylcytosine (RX-3117)" in the medical journal, *Investigational New Drugs*.

### ***Archexin***<sup>®</sup>

Following a comprehensive scientific, clinical and business analysis of potential cancer indications for a new Phase IIa clinical trial with Archexin<sup>®</sup>, Rexahn has decided to pursue metastatic renal cell carcinoma (RCC). Metastatic RCC represents an attractive market opportunity with an estimated annual incidence of 90,000 patients world-wide and potential market size of greater than \$6 billion. Metastatic RCC patients receiving standard of care treatment have a poor prognosis with an overall survival of less than 2 years. Rexahn has been working with key opinion leaders to finalize the design of a Phase IIa clinical trial and anticipates initiating the clinical trial in December 2013.

In preclinical studies, Archexin has shown to inhibit the growth of human RCC cells in both tissue cultures and in animal xenograft models. In addition, Archexin may have the ability to prevent the development of resistance to existing therapies for RCC. Rexahn has received the U.S. Food and Drug Administration's Orphan Drug Designation for this indication.

"We've worked with our scientific advisory board and key opinion leaders to select RCC as the optimal first treatment indication to pursue for our Phase IIa trial of Archexin. The combination of strong scientific data, addressable market, unmet clinical need, and our Orphan Drug Designation were all driving factors for choosing this indication," commented Dr. Suzdak. "We are preparing to initiate our trial next month."

### **Financial Update:**

For the nine month period ending September 30, 2013, total operating expenses were \$5.6 million. Rexahn's cash and cash equivalents including marketable securities and restricted cash as of September 30, 2013 totaled approximately \$15.5 million. In addition, on October 16, 2013, Rexahn completed a \$5.3 million registered direct offering for a purchase price of \$0.52 per share. The proceeds of this offering will be used for further research and development of the Company's pipeline.

### **About Supinoxin<sup>™</sup> (RX-5902)**

Supinoxin is an orally administered, first-in-class, small molecule inhibitor of phosphorylated-p68 RNA helicase (P-p68). P-p68, which is selectively expressed 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. Over-expression of P-p68 has been observed in solid tumors such as melanoma, colon, ovarian and lung.

### **About RX-3117**

RX-3117 is a novel small molecule anti-metabolite that is incorporated into DNA or RNA of cells. It inhibits both DNA and RNA synthesis which induces apoptotic cell death of tumor cells. RX-3117 mediates the downregulation of DNA methyltransferase 1 (DNMT1), which is an enzyme responsible for the methylation of cytosine residues on newly synthesized DNA and is a target for anti-cancer therapies. Preclinical studies have shown RX-3117 to be effective in both inhibiting the growth of various human cancer xenograft models, including colon, lung, renal and pancreas, as well as overcoming chemotherapeutic drug resistance.

RX-3117 has demonstrated a broad spectrum anti-tumor activity against 50 different human cancer cell lines and efficacy in 12 different mouse xenograft models. The efficacy in the mouse xenograft models was superior to that of gemcitabine. In addition, RX-3117 still retains its full anti-tumor activity in human cancer cell lines made resistant to the anti-tumor effects of gemcitabine. These findings have either been previously presented at the American Association of Cancer Research Meeting in 2012, or were the subject of a peer reviewed publication released in September 2013. In August 2012, Rexahn reported the completion of an exploratory Phase I clinical trial of RX-3117 in cancer patients conducted in Europe. The trial investigated the oral bioavailability, safety and tolerability of the compound. In this study, oral administration of RX-3117 demonstrated an oral bioavailability of 56% and a plasma half-life ( $T_{1/2}$ ) of 14 hours. In addition, RX-3117 was safe and well tolerated in all subjects throughout the dose range tested.

### **About Archexin®**

Archexin® is a unique anti-cancer drug candidate that inhibits the cancer cell signaling protein Akt-1. The activated form of Akt-1, which is involved in cancer cell growth, survival, angiogenesis, and drug resistance, has shown to be present or elevated in more than 12 different human cancer cell lines, including pancreatic and renal cell carcinoma. By inhibiting Akt-1, Archexin has shown to both inhibit the growth of human renal cell carcinoma cell lines and decrease the growth of human renal cell carcinoma in animal xenograft models. Thus, while Akt-1 is a very specific anti-cancer target it may have broad therapeutic potential across multiple types of cancer. Archexin has completed a Phase I clinical trial in cancer patients with solid tumors and was shown to be safe and well tolerated. The dose-limiting toxicity was a grade 3 fatigue. In a small Phase IIa trial in advanced pancreatic cancer patients, Archexin in combination with gemcitabine was shown to be safe and well tolerated. It demonstrated a preliminary efficacy signal with a median survival of 9.1 months in evaluable patients.

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

Rexahn Pharmaceuticals is a clinical stage biopharmaceutical company dedicated to developing best-in-class therapeutics for the treatment of cancer. Rexahn currently has three clinical stage oncology candidates, Archexin®, RX-3117, and Supinoxin™ (RX-5902), and a

robust pipeline of preclinical compounds to treat multiple types of cancer. Rexahn has also developed proprietary drug discovery platform technologies in the areas of Nano-Polymer-Drug Conjugate Systems (NPDCS), nano-medicines, 3D-GOLD, and TIMES. For more information, 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 future operations and products 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, the difficulty of developing pharmaceutical products, obtaining regulatory and other approvals and achieving market acceptance; the timing and success of clinical testing; the timing of the conduct of clinical testing; the timing of the receipt and disclosure of clinical data and Rexahn's need for and ability to obtain additional financing. 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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