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# Rexahn Pharmaceuticals Reports Fourth Quarter and Full-Year 2013 Financial Results and Recent Highlights

- *Supinoxin™ Phase I clinical trial enters fourth dosing group; preliminary data announced in March 2014*
- *Archexin® Phase IIa clinical trial initiated in metastatic renal cell carcinoma*
- *RX-3117 Phase Ib clinical trial initiated*

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 an overview of its three clinical development programs and financial results for the year ended December 31, 2013.

“Last year was a year of transition for Rexahn as we re-focused our efforts to build a pipeline of oncology assets and raised a significant amount of capital to support the clinical development of our three programs,” commented Rexahn’s Chief Executive Officer, Peter D. Suzdak, Ph.D. “We are very excited about the progress of the Supinoxin™, RX-3117, and Archexin® clinical development programs, and we believe that 2014 will be a transformational year for Rexahn with clinical data expected from all three programs. I look forward to updating our shareholders on the upcoming milestones as appropriate and appreciate their support.”

## **Pipeline Update:**

### ***Supinoxin™ (RX-5902)***

A Phase I dose-escalation clinical trial of Supinoxin (RX-5902) in cancer patients with solid tumors began enrolling patients in August 2013. The study is still ongoing and the maximum tolerated dose (MTD) has not yet been achieved. Three dosing cycles have been completed (25, 50 and 100 mg), and no drug related adverse events have been reported. The fourth dosing cycle (150 mg) has been initiated. Two patients have received two cycles of treatment, and one patient has received six cycles of treatment. Pharmacokinetic analysis has shown that Supinoxin displays dose-proportional exposure and an estimated oral bioavailability of 51%. The pharmacokinetic profile of Supinoxin is similar to what has been seen in preclinical studies. Rexahn expects to complete this trial and announce final results in the fourth quarter of 2014.

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

Rexahn initiated a Phase Ib clinical trial of RX-3117 in cancer patients with solid tumors in

January 2014. The Phase Ib trial is a multi-center dose-escalation study which will evaluate the safety, tolerability, dose-limiting toxicities and MTD of RX-3117 in cancer patients with solid tumors. Secondary endpoints will include characterizing the pharmacokinetic profile of RX-3117 and evaluating the preliminary anti-tumor effects of RX-3117. Rexahn expects to complete patient enrollment of the RX-3117 Phase I clinical trial in the fourth quarter of 2014 or early 2015.

### **Archexin®**

Rexahn initiated a Phase IIa Archexin proof-of-concept clinical trial in patients with metastatic renal cell carcinoma (RCC) in January 2014. Rexahn has previously received orphan drug designation for this indication. The trial is a multi-center study designed to evaluate the efficacy of Archexin in combination with everolimus (Afinitor®) to treat metastatic RCC patients and will be conducted in two stages. The first stage will be dose ranging, with up to three cohorts of three RCC patients to determine its MTD in combination with everolimus. Once the MTD has been determined, thirty RCC patients will be randomized for treatment with either Archexin in combination with everolimus or everolimus alone, in a ratio of 2:1. Rexahn plans to complete the initial component of this trial in the fourth quarter of 2014.

### **Additional Highlights from 2013:**

- Announced the appointment of Dr. Peter D. Suzdak as Chief Executive Officer. Prior to joining Rexahn, Dr. Suzdak was Chief Scientific Officer of Corridor Pharmaceuticals and co-Founder, founding Chief Executive Officer and Chief Scientific Officer of Cardioxyl Pharmaceuticals.
- Presented mechanism of action data for RX-5902 at the American Association for Cancer Research (AACR) Annual Meeting in April.
- In-licensed two novel drug delivery platforms, Nano-Polymer-Drug Conjugate Systems (NPDCS) and Lipid-Coated Albumin Nanoparticle (LCAN). These technologies target the delivery of chemotherapeutic agents directly into cancerous tumors.

### **Financial Update:**

For the year ended December 31, 2013, total operating expenses were \$8.0 million. Rexahn's cash and investments totaled \$19.0 million as of December 31, 2013, as compared to \$14.7 million on December 31, 2012. The increase of \$4.3 million was primarily due to \$12.3 million from the issuance of common stock and the exercise of stock warrants and options, offset by \$8.0 million of net cash used in operating activities.

On July 26, 2013, Rexahn completed a \$5.7 million registered direct offering of common stock and warrants at an offering price of \$0.50 per unit. On October 16, 2013, Rexahn completed a \$5.3 million registered direct offering of common stock and warrants at an offering price of \$0.52 per unit. Additionally, on January 15, 2014, Rexahn completed a \$20.0 million registered direct offering of common stock and warrants at an offering price of \$1.05 per unit. The proceeds of this offering will be used for further research and development of Rexahn's pipeline. As of March 21, 2014, Rexahn's cash and investments totaled approximately \$40.3 million (unaudited).

## **About Supinoxin™ (RX-5902)**

Supinoxin (RX-5902) is an orally administered, potential 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. In preclinical studies, Supinoxin has been shown to inhibit proliferation of cancer cells in 18 human cancer cell lines including breast, colon, pancreas, ovarian, and stomach cancers, and showed potent activity in drug-resistant cancer cells. In an animal model, where human cancer cells from melanoma, pancreas, renal or ovarian cancers were grafted into animals, treatment with Supinoxin resulted in a significant reduction in tumor growth.

## **About RX-3117**

RX-3117 is a nucleoside analog that is activated (phosphorylated) by Uridine Cytidine Kinase (UCK) and inhibits both DNA and RNA synthesis which induces apoptotic cell death of tumor cells. UCK is overexpressed in multiple human tumors, but has a limited presence in normal tissues. This unique specificity for cancer cells may lead to an improved safety profile in cancer patients. RX-3117 also mediates the downregulation of DNA methyltransferase 1 (DNMT1), an enzyme responsible for the methylation of cytosine residues on newly synthesized DNA and also a target for anticancer 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, and 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 will be the subject of a peer reviewed publication to be published in early 2014. In August 2012, Rexahn 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 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 potential best-in-class anti-sense drug candidate that specifically inhibits the cancer cell signaling protein Akt-1. Archexin is the only specific inhibitor of Akt-1 in clinical development. 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 exhibit a longer survival benefit in the human renal cell carcinoma animal xenograft model. We believe that, while Akt-1 is a very specific anti-cancer target, Archexin 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<sup>®</sup>, RX-3117, and Supinoxin<sup>™</sup> (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 marketing success of Rexahn's licensees or sublicensees; the success of clinical testing; 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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