

May 15, 2014



# Rexahn Pharmaceuticals Reports First Quarter 2014 Financial Results and Pipeline Update

## *Three clinical trials ongoing with key milestones expected in Q4 2014*

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 quarter ended March 31, 2014.

"We continue to enroll patients in each of our three clinical trials as expected, and as planned for," stated Rexahn's Chief Executive Officer, Peter D Suzdak, Ph.D. "By the end of 2014, we expect to achieve key milestones in each trial. In the fourth quarter we expect to have data from our Phase I trial of Supinoxin<sup>™</sup> in cancer patients with solid tumors. We are also scheduled to complete enrollment of patients in our Phase Ib clinical trial of RX-3117 by the end of 2014. During the fourth quarter of this year, we anticipate completing the safety portion of our Phase II trial for metastatic renal cell carcinoma."

### **Pipeline Update:**

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

The Company initiated a Phase I clinical trial of Supinoxin in cancer patients with solid tumors in August 2013, which is ongoing and is expected to be completed in the fourth quarter of 2014. In March, the Company announced the initial results from the trial. The maximum tolerated dose (MTD) of Supinoxin 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) is ongoing. 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.

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

Rexahn initiated a Phase Ib clinical trial of RX-3117 in cancer patients with solid tumors in December 2013. The Company expects to complete patient enrollment for the trial in the fourth quarter of 2014 or early 2015. 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 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 has completed one dose cycle (30 mg) and is in the middle of the second dose cycle (100mg).

## **Archexin®**

Rexahn continues to enroll metastatic renal cell carcinoma patients in its Phase IIa proof-of-concept clinical trial for Archexin. 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 to either Archexin in combination with everolimus or everolimus alone, in a ratio of 2:1. Rexahn plans to complete the initial safety component of this study in the fourth quarter of 2014.

### **Additional Highlights from First Quarter 2014:**

- Presented data on RX-3117 and RX-21101 at the 2014 American Association for Clinical Research Annual Meeting.
- Announced the appointment of Mark P. Carthy to Rexahn's Board of Directors. Mr. Carthy is the Managing Partner of Orion Equity Partners, LLC, a healthcare venture capital management and advisory firm co-founded by Mr. Carthy in 2008.
- Completed a registered direct offering for aggregate gross proceeds of \$20 million. The proceeds of this offering will be used to further research and development of Rexahn's pipeline.

### **Financial Update:**

**Cash Position** - Rexahn's cash and investments totaled \$40.3 million as of March 31, 2014, compared to \$19.0 million as of December 31, 2013. The increase of \$21.3 million was primarily due to the issuance of common stock and the exercise of warrants and stock options to purchase common stock totaling \$23.9 million, which amount was offset by \$2.6 million of net cash used in operating activities. Rexahn expects that its cash and cash equivalents balance as of March 31, 2014 will be sufficient to fund the Company's operations into the first half of 2016.

**R&D Expenses** - Research and development expenses were \$1.3 million for the first quarter of 2014, compared to \$0.6 million for the first quarter of 2013. The increase is primarily attributable to the clinical trials that were ongoing during the three months ended March 31, 2014.

**G&A Expenses** - General and administrative expenses were \$1.4 million for the first quarter of 2014, compared to \$1.2 million for the first quarter of 2013. The increase is attributable to increases in investor relations and financial advisory services relating to the Company's financing activities.

**Net Loss** - Rexahn's net loss was \$14.6 million, or \$0.09 per share, for the first quarter of 2014, compared to a net loss of \$1.5 million, or \$0.01 per share, for the comparable period in 2013. Included in the net loss for the first quarter was a non-cash charge of \$11.7 million due to an adjustment to the fair value of outstanding warrants resulting from the increased stock price of the underlying common stock, as compared to a non-cash gain of \$0.4 million in the first quarter of 2013.

### **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 tumors. 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 small molecule nucleoside analog that is activated (phosphorylated) by the enzyme 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 efficacy and safety profile in cancer patients. RX-3117 also mediates the down regulation 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, 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. 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 unique anti-cancer drug candidate which inhibits the cancer cell signaling protein Akt-1, which is involved in cancer cell growth, survival, angiogenesis, and drug resistance. Rexahn has completed a Phase I clinical trial of Archexin 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 and 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.

The Trout Group LLC  
Tricia Truehart, 646-378-2953  
[ttruehart@troutgroup.com](mailto:ttruehart@troutgroup.com)

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