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## **Rexahn Reports Data from the Ongoing Phase 2a Clinical Trial of RX-3117 in Advanced Bladder Cancer at the American Society of Clinical Oncology Genitourinary Cancers (ASCO GU) 2018 Annual Meeting**

### **Encouraging Progression Free Survival and Evidence of Tumor Shrinkage Observed in Patients with Advanced Bladder Cancer Who Had Failed on Multiple Prior Treatments Including Immunotherapy and Gemcitabine**

ROCKVILLE, Md., Feb. 12, 2018 (GLOBE NEWSWIRE) -- Rexahn Pharmaceuticals, Inc. (NYSE American:RNN), a clinical stage biopharmaceutical company developing innovative, targeted therapeutics for the treatment of cancer, announced that updated safety and efficacy data from the ongoing Phase 2a clinical trial of RX-3117 in advanced urothelial (bladder) cancer were presented at the American Society of Clinical Oncology Genitourinary Cancers (ASCO GU) 2018 Annual Meeting on February 9, 2018.

The poster presentation reported interim data on the Phase 2a clinical trial of RX-3117 monotherapy for the treatment of advanced bladder cancer. A total of 27 patients with advanced bladder cancer were enrolled into the study. Patients in the study had actively progressing bladder cancer with distant metastases to multiple sites including the liver, lung, lymph nodes and pelvis. Eighty-seven percent of the patients had received two or more prior therapies including gemcitabine (85% of patients) and immunotherapy (67% of patients).

Twenty-one patients were included in the efficacy analysis. Of these patients, 33% experienced progression free survival for two months or more and 19%, or four patients, had disease stabilization for greater than four months with one of these patients having stable disease for 301 days. Four patients had a reduction in tumor size that was equal to or greater than 15%. RX-3117 was safe and well tolerated based on the data from the trial to date.

"The trial is still ongoing, but there is a strong efficacy signal to support further development of RX-3117 in advanced bladder cancer," said Ely Benaim, M.D., Chief Medical Officer, Rexahn. "There is nothing approved for third line treatment of bladder cancer. These patients are usually transferred to palliative and supportive care only, so we are very pleased with the data emerging from this study."

"Patients with advanced bladder cancer, who have already developed resistance to gemcitabine and whose cancer has progressed on immunotherapy, are very difficult to treat," said Dr. Sumanta Pal, Associate Professor at City of Hope Comprehensive Cancer Center, Duarte, California. "It was encouraging to see disease stabilization and evidence of tumor reduction in some patients. RX-3117 also appears to be safe and well-tolerated with no dose limiting side-effects."

The Phase 2a clinical trial is a multicenter, open-label single-agent study of RX-3117 conducted at 5 clinical centers in the United States. Patients initially received 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 progressed. The dosing was increased to four weeks continuous treatment with no rest week for patients for whom RX-3117 was well tolerated. The primary endpoints are an assessment of the progression free survival rate or an objective clinical response and reduction in tumor size.

The interim data from the Phase 2a clinical trial of RX-3117 in advanced bladder cancer was presented on Friday February 9, 2018 in a poster presentation entitled **RX-3117, an Oral Hypomethylating agent to Treat Advanced Solid Tumors: Interim Results from an ongoing Phase 2a study in Advanced Urothelial Cancer (aUC)**.

A copy of the ASCO GU poster can be viewed on the company's website at <https://rexahn.com/cms/media-center/publication/posters/>

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

RX-3117 is a novel, investigational, oral, 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. Because UCK2 is overexpressed in multiple human tumors, but has a very

limited presence in normal tissues, RX-3117 offers the potential for a targeted anti-cancer therapy with an improved efficacy and safety profile. RX-3117 is currently being studied in a Phase 2a clinical trial in combination with Abraxane® (nab-paclitaxel) in first line metastatic pancreatic patients and a Phase 2a clinical trial in patients with advanced or metastatic bladder cancer. It has received Orphan Drug designation for the treatment of pancreatic cancer. Additional information on RX-3117 can be found at: <https://rexahn.com/cms/portfolio/rx-3117/>.

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

Rexahn Pharmaceuticals Inc. (NYSE American:RNN) is a clinical stage biopharmaceutical company dedicated to developing novel, targeted 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, including 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: RX-5902 (Supinoxin™), RX-3117, and RX-0201 (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](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 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 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.

### **Investor contact:**

LifeSci Advisors, LLC  
Ashley Robinson  
617-775-5956  
[arr@lifesciadvisors.com](mailto:arr@lifesciadvisors.com)