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## **TKM-Ebola-Guinea Enters Phase II Clinical Study in Sierra Leone**

### **Patient Recruitment Initiated**

VANCOUVER, British Columbia, March 11, 2015 (GLOBE NEWSWIRE) -- Tekmira Pharmaceuticals Corporation (Nasdaq:TKMR), an industry-leading therapeutic solutions company focused on developing a cure for chronic hepatitis B virus infection (HBV), announced today that TKM-Ebola-Guinea will be evaluated for efficacy in Ebola virus infected patients in Sierra Leone, West Africa. Patient recruitment has been initiated.

The Phase II single arm trial called RAPIDE (Rapid Assessment of Potential Interventions & Drugs for Ebola) is open-label with a concurrent observational study of Ebola virus disease in Sierra Leone. Study results are expected in the second half of 2015.

The University of Oxford, which is the representative of the International Severe Acute Respiratory and Emerging Infection Consortium (ISARIC) is responsible for conducting the Phase II study, with funding provided by the Wellcome Trust.

"We have been working judiciously these last few months to finalize a suitable clinical trial protocol and to receive all the necessary ethics and regulatory approvals to enable TKM-Ebola-Guinea to be clinically evaluated in patients in Sierra Leone," said Dr. Mark J. Murray, Tekmira's President and CEO. "This study will enable us to gather important clinical data that may help us to determine whether TKM-Ebola-Guinea is a promising treatment for patients infected with Ebola virus disease. We expect the results of the study to help inform us on the potential further development of this important therapeutic."

Dr. Murray added, "New cases of Ebola virus disease infections are still occurring, and many people remain infected, which reinforces the critical need for effective therapeutics against this deadly disease. If we are eventually successful in obtaining market approval of TKM-Ebola-Guinea, the company would provide patients a therapeutic option for this terrible disease and in addition this may place the company on a path to receive a Tropical Disease Priority Review Voucher from the US FDA that we can apply to another product in development to support expedited approval."

### **About TKM-Ebola-Guinea, an Anti-Ebola RNAi Therapeutic Targeting Ebola-Guinea**

The Ebola-Guinea strain is known as "Ebola virus Makona" the virus responsible for the current outbreak in West Africa. This strain diverges slightly from the Kikwit strain, which was the original target of TKM-Ebola. The genomic sequence of the Ebola-Guinea strain was determined from several viral isolates and published in the New England Journal of Medicine in October 2014<sup>1</sup>. Tekmira developed a modified RNAi therapeutic, based on the Company's original TKM-Ebola investigational therapeutic, to specifically target Ebola-Guinea. The new product, termed TKM-Ebola-Guinea, is designed to match the genomic sequence exactly, with two RNAi triggers. The ability to rapidly and accurately match the evolving genetic sequences of emerging infectious agents is one of the powerful features of RNAi therapeutics.

### **About the Neglected Tropical Disease Priority Review Voucher Program**

Developed by the U.S. FDA in 2007 under the Food and Drug Administration Amendments Act ("FDAAA"), a Priority Review Voucher (PRV) is an incentive for companies to invest in new drugs and vaccines for neglected tropical diseases. Ebola virus disease was recently added to the PRV program. A PRV entitles the holder to a "priority review" reducing the target review time for a New Drug Application from 10 months to 6 months. Plus, the PRV permits a voucher to be sold—"transferred"—an unlimited number of times, before eventual use.

### **About RNAi and Tekmira's LNP**

RNAi therapeutics have the potential to treat a number of human diseases by "silencing" disease causing genes. The discoverers of RNAi, a gene silencing mechanism used by all cells, were awarded the 2006 Nobel Prize for Physiology or Medicine. RNAi trigger molecules often require delivery technology to be effective as therapeutics. Tekmira believes its LNP technology represents the most advanced and widely adopted delivery technology for the systemic delivery of RNAi triggers. Tekmira's LNP platform is being utilized in multiple clinical trials in various disease areas by Tekmira and its partners. Tekmira's LNP technology (formerly referred to as stable nucleic acid-lipid particles or SNALP) encapsulates RNAi triggers with high efficiency in uniform lipid nanoparticles that are effective in delivering these therapeutic compounds to disease sites. Tekmira's

LNP formulations are manufactured by a proprietary method which is robust, scalable and highly reproducible, and LNP-based products have been reviewed by multiple regulatory agencies for use in clinical trials. LNP formulations comprise several lipid components that can be adjusted to suit the specific application.

### **About Wellcome Trust**

The Wellcome Trust is a global charitable foundation dedicated to improving health. We provide more than £700 million a year to support bright minds in science, the humanities and the social sciences, as well as education, public engagement and the application of research to medicine. Our investment portfolio gives us the independence to support such transformative work as the sequencing and understanding of the human genome, research that established front-line drugs for malaria, and Wellcome Collection, our free venue for the incurably curious that explores medicine, life and art. [www.wellcome.ac.uk](http://www.wellcome.ac.uk).

### **About Oxford University's Medical Sciences Division**

Oxford University's Medical Sciences Division is one of the largest biomedical research centres in Europe, with over 2,500 people involved in research and more than 2,800 students. The University is rated the best in the world for medicine, and it is home to the UK's top-ranked medical school. From the genetic and molecular basis of disease to the latest advances in neuroscience, Oxford is at the forefront of medical research. It has one of the largest clinical trial portfolios in the UK and great expertise in taking discoveries from the lab into the clinic. Partnerships with the local NHS Trusts enable patients to benefit from close links between medical research and healthcare delivery. A great strength of Oxford medicine is its long-standing network of clinical research units in Asia and Africa, enabling world-leading research on the most pressing global health challenges such as malaria, TB, HIV/AIDS and flu. Oxford is also renowned for its large-scale studies which examine the role of factors such as smoking, alcohol and diet on cancer, heart disease and other conditions.

### **About Tekmira**

Tekmira Pharmaceuticals Corporation is a biopharmaceutical company dedicated to discovering, developing and commercializing a cure for patients suffering from chronic hepatitis B infection (HBV). We believe this goal will only be accomplished with multiple agents and multiple mechanisms of action targeting various aspects of the virus in combination. Our strategy is to target the three pillars necessary to develop a curative regimen for HBV, including suppressing HBV replication within liver cells, stimulating and reactivating the body's immune system so that it can mount an effective defense against the virus and, most importantly, eliminating the reservoir of viral genomic material known as covalently closed circular DNA, or cccDNA, that is the source of HBV persistence. Our portfolio of assets includes eight drug candidates for use in combination to develop a cure for HBV.

We also have meaningful non-HBV assets including key development partnerships and product candidates in oncology (TKM-PLK1), anti-viral (TKM-Ebola and TKM-Ebola-Guinea) and metabolic programs (TKM-HTG) that leverage our expertise in RNA interference (RNAi) therapeutics and leading Lipid Nanoparticle (LNP) delivery technology. RNAi and LNP technology have the potential to generate new therapeutics that take advantage of the body's own natural processes to silence disease causing genes, or more specifically, to eliminate specific gene-products, from the cell. Our plan is to maximize the value of these programs while ensuring the primary focus and resource allocation within the company remains weighted on HBV.

### **Forward-Looking Statements and Information**

This press release contains forward-looking statements within the meaning of the Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, and forward looking information within the meaning of Canadian securities laws (collectively, "forward-looking statements"). Forward-looking statements in this press release include statements about an evaluation of TKM-Ebola-Guinea; the timing of expected results of the Phase II clinical (Rapid Assessment of Potential Interventions & Drugs for Ebola) RAPIDE study; the University of Oxford as the conductor of the study; the funding provided by the Wellcome Trust; the ability to gather useful clinical data from the study; market approval of TKM-Ebola-Guinea; receipt and subsequent use of a Tropical Disease Priority Review Voucher; curing HBV through a combination mechanism; and Tekmira's plan to maximize the value of its non-HBV assets while ensuring a primary focus and resource allocation on HBV.

With respect to the forward-looking statements contained in this press release, Tekmira has made numerous assumptions regarding, among other things: the continued ability of the University of Oxford to conduct the Phase II clinical RAPIDE study; the availability of funding from the Wellcome Trust; the availability of patients for the study; the ability to carry out the study effectively and obtain useful data; the continuation of the Neglected Tropical Disease Priority Review Voucher Program; and the continued suitability of Tekmira's asset strategy. While Tekmira considers these assumptions to be reasonable, these assumptions are inherently subject to significant business, economic, competitive, market and social uncertainties and contingencies.

Additionally, there are known and unknown risk factors which could cause Tekmira's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements contained herein. Known risk factors include, among others: the University of Oxford may no longer

be willing or able to conduct the study; the Wellcome Trust may not be willing or able to provide the necessary funding; there may be insufficient patients in Sierra Leone to enroll into the study; the results may be delayed, or not useful in evaluating the efficacy of TKM-Ebola-Guinea; the Neglected Tropical Disease Priority Review Voucher Program may be discontinued; anticipated pre-clinical and clinical trials may be more costly or take longer to complete than anticipated, and may never be initiated or completed, or may not generate results that warrant future development of the tested drug candidate; Tekmira may not receive the necessary regulatory approvals for the clinical development of Tekmira's products; and market shifts may require a change in strategic focus.

A more complete discussion of the risks and uncertainties facing Tekmira appears in Tekmira's Annual Report on Form 10-K and Tekmira's continuous disclosure filings, which are available at [www.sedar.com](http://www.sedar.com) and at [www.sec.gov](http://www.sec.gov). All forward-looking statements herein are qualified in their entirety by this cautionary statement, and Tekmira disclaims any obligation to revise or update any such forward-looking statements or to publicly announce the result of any revisions to any of the forward-looking statements contained herein to reflect future results, events or developments, except as required by law.

## Reference

<sup>1</sup> Baize S., Pannetier D., Oestereich L., et al. "*Emergence of Zaire Ebola Virus Disease in Guinea.*" **New England Journal of Medicine**. October 9, 2014 Vol. 371 No. 15

CONTACT: Investors

Julie P. Rezler

Director, Investor Relations

Phone: 604-419-3200

Email: [jrezler@tekmira.com](mailto:jrezler@tekmira.com)

Media

Please direct all media inquiries to [media@tekmira.com](mailto:media@tekmira.com)