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# Preclinical Data Presented at Scientific Symposium Demonstrates That mRNA is Efficiently Delivered Using Tekmira's LNP

VANCOUVER, British Columbia, Oct. 24, 2013 (GLOBE NEWSWIRE) -- Tekmira Pharmaceuticals Corporation (Nasdaq:TKMR) (TSX:TKM), a leading developer of RNA interference (RNAi) therapeutics, announced that data demonstrating its ongoing lipid nanoparticle (LNP) technology innovations, including the enablement of messenger RNA (mRNA), were presented at the 1<sup>st</sup> International mRNA Health Conference taking place in Tubingen, Germany from October 23-24, 2013 by Tekmira's Chief Scientific Officer, Dr. Ian MacLachlan.

"Tekmira's LNP technology represents the most widely adopted delivery technology in RNAi, enabling eight clinical trials and administered to over 200 patients to date. Because LNP can enable a wide variety of nucleic acid payloads, including messenger RNA, we continue to see new product development and partnering opportunities based on our industry-leading delivery expertise," said Dr. Mark J. Murray, Tekmira's President and CEO.

"We believe that mRNA represents a class of nucleic acids with exciting therapeutic applications, particularly in rare diseases; however, effective delivery of mRNA is going to be essential for success. We are excited to be presenting data for the first time demonstrating Tekmira's progress with both liver-directed and tumor- directed LNP formulations that are tailored specifically for mRNA delivery," added Dr. Murray.

In a presentation entitled "Lipid Nanoparticle-Mediated Delivery of Messenger RNA" data were presented that showed highly potent delivery *in vivo* to liver, tumors and other tissues. Some key highlights from the presentation include:

- The data demonstrated that mRNA when encapsulated and delivered using Tekmira's LNP technology can be effectively delivered and expressed in liver, tumors and other specific tissues of therapeutic interest.
- In one study using mRNA with a liver-directed LNP formulation, data showed gene expression levels orders of magnitude greater than other published results with lipid-based delivery technology.
- Tekmira has demonstrated highly efficient delivery in two different tumor models examining both distal tumors and orthotopic liver tumors with gene expression long-lived as compared to other tissues.
- When applying Tekmira's LNP technology to the delivery of mRNA in the liver, data demonstrated mRNA delivery and expression in every single hepatocyte.

## About Messenger RNA (mRNA) and the 1<sup>st</sup> International mRNA Health Conference

Messenger RNA (mRNA) is a class of RNA molecules that contains the genetic information specifying the amino acid sequence of proteins. mRNA is translated in the cell into polymeric chains of amino acids or proteins. The medical use of mRNA to direct the intracellular expression of therapeutic proteins is rapidly evolving into a key area of research and development in biotechnology. Scientists and pharmaceutical companies around the world are developing drugs based on this biomolecule. The first clinical studies are currently ongoing, and innovative therapeutic treatment options for numerous diseases such as cancer, cardiovascular diseases or influenza are becoming more feasible. The 1st International mRNA Health Conference, taking place on Oct. 23-24, will host the world's experts in mRNA from academia and industry, who will present their latest findings to an expert audience. More information can be found at: www.mrna-conference.com.

#### About RNAi and Tekmira's LNP

RNAi therapeutics have the potential to treat a broad 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 therapeutics, such as "siRNAs," require delivery technology to be effective systemically. Tekmira believes its LNP technology represents the most widely adopted delivery technology for the systemic delivery of RNAi therapeutics. Tekmira's LNP platform is being utilized in multiple clinical trials by both Tekmira and its partners. Tekmira's LNP technology (formerly referred to as stable nucleic acid-lipid particles or SNALP) encapsulates siRNAs with high efficiency in uniform lipid nanoparticles that are effective in delivering RNAi therapeutics to disease sites in numerous preclinical models. 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 FDA divisions for use in clinical trials. LNP formulations comprise several lipid components that can be adjusted to suit the specific application.

### **About Tekmira**

Tekmira Pharmaceuticals Corporation is a biopharmaceutical company focused on advancing novel RNAi therapeutics and providing its leading lipid nanoparticle delivery technology to pharmaceutical partners. Tekmira has been working in the field of nucleic acid delivery for over a decade and has broad intellectual property covering LNPs. Further information about Tekmira can be found at <u>www.tekmirapharm.com</u>. Tekmira is based in Vancouver, B.C.

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

This news release contains "forward-looking statements" or "forward-looking information" within the meaning of applicable securities laws (collectively, "forward-looking statements"). Forward-looking statements are generally identifiable by use of the words "believes," "may," "plans," "will," "anticipates," "intends," "budgets," "could," "estimates," "expects," "forecasts," "projects" and similar expressions, and the negative of such expressions. Forward-looking statements in this news release include Tekmira's strategy, future operations, clinical trials, prospects and the plans of management; RNAi (ribonucleic acid interference) product development programs; data demonstrating Tekmira's ongoing lipid nanoparticle (LNP) technology innovations, including the enablement of messenger RNA (mRNA); mRNA is efficiently delivered using Tekmira's LNP; the advancement of products that utilize Tekmira's lipid nanoparticle technology; expectations regarding the advancement of multiple product candidates; the quantum and timing of further clinical data being presented for LNP-enabled products; continued innovation and protection of LNP technology; timing of the initiation of clinical trials and release of clinical data from Tekmira's product candidates; the quantum and timing of potential milestone and royalty payments; and the use of lipid nanoparticle technology by Tekmira's licensees.

With respect to the forward-looking statements contained in this news release, Tekmira has made numerous assumptions regarding, among other things: LNP's status as a leading RNAi delivery technology; the effectiveness of Tekmira's products as therapeutic treatments for diseases, including rare diseases; results in preclinical models are indicative of the potential effect in humans; mRNA is efficiently delivered using Tekmira's LNP; Tekmira's research and development capabilities and resources; FDA approval with respect to commencing clinical trials; the timing and obtaining of regulatory approvals for Tekmira's products; the timing and results of clinical data releases and use of LNP technology by Tekmira's development partners and licensees; the time required to complete research and product development activities; the timing and quantum of payments to be received under contracts with Tekmira's partners; Tekmira's financial position and its ability to execute on its business strategy; and Tekmira's ability to protect its intellectual property rights and not to infringe on the intellectual property rights of others. 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; Tekmira's research and development capabilities and resources may not meet current or expected demand; Tekmira's products may not prove to be effective as therapeutic treatments for diseases, including rare diseases; Tekmira's LNP may not be as efficient of a delivery system for mRNA as currently believed; Tekmira may not obtain and protect intellectual property rights, and operate without infringing on the intellectual property rights of others: Tekmira may face competition from other pharmaceutical or biotechnology companies and the possibility that other organizations have made advancements in RNAi delivery technology that Tekmira is not aware of; pre-clinical and clinical trials may be more costly or take longer to complete than anticipated and may not generate results that warrant future development of the tested drug candidate; the FDA may determine that the design and planned analysis of Tekmira's clinical trials do not adequately address the trial objectives in support of Tekmira's regulatory submissions; Tekmira's development partners and licensees conducting clinical trial, development programs and joint venture strategic alliances may not result in expected results on a timely basis, or at all; future operating results are uncertain and likely to fluctuate; Tekmira may not be able to raise additional financing required to fund further research and development, clinical studies, and obtain regulatory approvals, on commercially acceptable terms or at all; economic and capital market conditions; Tekmira may become subject to product liability or other legal claims for which Tekmira has made no accrual in its financial statements; and the possibility that Tekmira may not have sufficiently budgeted for expenditures necessary to carry out planned activities.

A more complete discussion of the risks and uncertainties facing Tekmira appears in Tekmira's annual report on Form 20-F for the year ended December 31, 2012 (Annual Report), which is available at <u>www.sedar.com</u> or at <u>www.sec.gov/edgar.shtml</u>. 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.

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