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## **Tekmira Presents TKM-PLK1 Data at 6th Annual North American Neuroendocrine Tumor Society (NA-NETS) Conference**

*New Data Shows Positive Chromogranin-A Biomarker Results in GI-NET Patient*

*Phase I/II Clinical Trial with TKM-PLK1 Ongoing in Patients with Advanced Gastrointestinal Neuroendocrine Tumors (GI-NET) or Adrenocortical Carcinoma (ACC)*

VANCOUVER, British Columbia, Oct. 4, 2013 (GLOBE NEWSWIRE) -- Tekmira Pharmaceuticals Corporation (Nasdaq:TKMR) (TSX:TKM), a leading developer of RNA interference (RNAi) therapeutics, today announced that it presented additional TKM-PLK1 data at the *6th Annual NET Conference* hosted by the North American Neuroendocrine Tumor Society (NA-NETS) to be held in Charleston, South Carolina from October 4-5, 2013.

"We are pleased to present data at this conference reaching researchers and physicians treating patients who have been diagnosed with neuroendocrine tumors. We have an ongoing Phase I/II clinical trial with TKM-PLK1, with current enrollment targeting patients with either advanced Gastrointestinal Neuroendocrine Tumors (GI-NET) or Adrenocortical Carcinoma (ACC), initiated following encouraging drug activity observed in these indications during Phase I," said Dr. Mark J. Murray, Tekmira's President and CEO.

Tekmira has enrolled a total of 36 patients in a population of advanced cancer patients with solid tumors. Doses ranged from 0.15 mg/kg to 0.90 mg/kg during the dose escalation portion of the trial, with the maximum tolerated dose (MTD) of 0.75 mg/kg administered to the expansion cohort. A total of 174 doses were administered with a mean number of 5.3 doses per patient (range of 1-31 doses). No dose dependent changes in liver function tests were observed.

Forty percent (6 out of 15) of patients evaluable for response, treated at a dose equal to or greater than 0.6 mg/kg, showed clinical benefit. Of the 36 patients enrolled, three out of the four ACC patients (75%) treated with TKM-PLK1 achieved stable disease, including one patient who saw a 19.3% reduction in tumor size and is still on study receiving TKM-PLK1. Of the two GI-NET patients enrolled, both experienced clinical benefit: one patient had a partial response based on RECIST criteria, and the other GI-NET patient achieved stable disease and showed a greater than 50% reduction in Chromogranin-A (CgA) levels, a key biomarker used to predict clinical outcome and tumor response.

"Lowered levels of Chromogranin-A (CgA) are linked to improved survival and favorable response to treatment in neuroendocrine patients. We are encouraged that a GI-NET patient who achieved stable disease showed a greater than 50% decline in CgA levels. We intend to collect CgA data in our ongoing TKM-PLK1 Phase I/II clinical trial and expect to have results from this completed trial by mid-2014. If supported by the data, we will commence a pivotal trial in GI-NET in 2014," added Dr. Murray.

### **About Chromogranin-A (CgA)**

There have been recent advances in neuroendocrine biomarkers, including research that demonstrates elevated CgA levels correlate with disease burden and poor outcomes, providing evidence that baseline and serial CgA levels may be used to monitor clinical outcome.

### **About the TKM-PLK1 Phase I/II Clinical Trial**

The ongoing TKM-PLK1 Phase I/II clinical trial, which is currently targeting GI-NET and ACC patients for enrollment, is a multi-center, single arm, open label study designed to measure efficacy using RECIST and tumor biomarkers for GI-NET patients, as well as to evaluate TKM-PLK1's safety, tolerability and pharmacokinetics. TKM-PLK1, which employs a unique lipid nanoparticle (LNP) formulation for oncology applications, will be administered weekly with each four-week cycle consisting of three once-weekly doses followed by a rest week. It is expected that approximately 20 patients with advanced GI-NET or ACC tumors will be enrolled in this trial, with a minimum of 10 GI-NET patients to be enrolled.

### **About TKM-PLK1**

Tekmira's lead oncology product candidate, TKM-PLK1, targets polo-like kinase 1 (PLK1), a protein involved in tumor cell proliferation and a validated oncology target. Inhibition of PLK1 expression prevents the tumor cell from completing cell division, resulting in cell cycle arrest and death of the cancer cell. PLK1 has been a target of interest for years, and evidence that patients with elevated levels of PLK1 in their tumors exhibit poorer prognosis and survival rates has been documented in the medical literature. By using an RNAi approach and exploiting its naturally occurring mechanism of action, Tekmira can potentially overcome the limitations of other approaches and effectively silence PLK1.

### **About Gastrointestinal Neuroendocrine Tumors (GI-NET)**

Neuroendocrine tumors (NETs) refers to a group of unusual and complex cancers that affect neuroendocrine cells, with those arising in the gastrointestinal tract referred to as GI-NET. It is estimated that there has been a four-fold increase in the incidence of NETs between 1973 and 2004. Approximately 55,000 people are living with GI-NET in the United States. There is a poor prognosis for advanced metastatic NETs, with 25% of patients surviving less than one year. The treatment of patients with gastrointestinal neuroendocrine tumors remains a challenge, and currently there are no approved anti-cancer drugs or treatments indicated specifically for GI-NET.

### **About Adrenocortical Carcinoma (ACC)**

Adrenocortical Carcinoma (ACC) is a rare cancer that forms in the outer layer of tissue of the adrenal gland (a small organ on top of each kidney that makes steroid hormones, adrenaline, and noradrenaline to control heart rate, blood pressure, and other body functions). Even with appropriated diagnosis and treatment, most patients will develop recurrence and succumb to ACC because of the underlying tumor biology, the difficulty of achieving a complete resection, and the lack of effective systemic therapies.

### **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 [www.tekmirapharm.com](http://www.tekmirapharm.com). 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 statements about Tekmira's strategy, future operations, clinical trials, prospects and the plans of management; RNAi (ribonucleic acid interference) product development programs; the results of the Phase I clinical trial with TKM-PLK1; the effects of Tekmira's products on the treatment of cancer, including gastrointestinal neuroendocrine tumors (GI-NET) and adrenocortical carcinoma (ACC); the expected completion and release of data from a Phase I/II clinical trial with TKM-PLK1, which will enroll patients with advanced GI-NET or ACC tumors; and, the expected timing of the commencement of a pivotal trial in GI-NET in 2014.

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 a treatment for cancer, including gastrointestinal neuroendocrine tumors (GI-NET) and adrenocortical carcinoma (ACC); results in preclinical models are indicative of the potential effect in humans; 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 time required to complete research and product development activities; 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: the completion of, and timing of the release of the data from, the Phase I/II clinical trial with TKM-PLK1 may not occur as anticipated, or at all; TKM-PLK1 may not enter a pivotal trial in GI-NET within the timeframe anticipated, or at all; Tekmira's ability to obtain and protect intellectual property rights, and operate without infringing on the intellectual property rights of others; Tekmira's research and development capabilities and resources will not meet current or expected demand; Tekmira's products may not prove to be effective in the treatment of cancer, including GI-NET or ACC; the possibility that other organizations have made advancements in RNAi delivery technology that Tekmira is not aware of; the FDA will not approve the commencement of Tekmira's planned clinical trials or approve the use of Tekmira's products and generally, difficulties or delays in the progress, timing and results of clinical trials; 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; pre-clinical and clinical trials may be more costly or take longer to complete than anticipated; pre-clinical or clinical trials may not generate results that warrant future development of the tested drug candidate; and the possibility that Tekmira has not 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 [www.sedar.com](http://www.sedar.com) or at [www.sec.gov/edgar.shtml](http://www.sec.gov/edgar.shtml). 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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