



May 14, 2013

## **Tekmira Provides Corporate Update and Announces First Quarter 2013 Results**

### **Conference Call at 4:00 pm Eastern Time Today**

VANCOUVER, British Columbia, May 14, 2013 (GLOBE NEWSWIRE) -- Tekmira Pharmaceuticals Corporation (Nasdaq:TKMR) (TSX:TKM), a leading developer of RNA interference (RNAi) therapeutics, today announced its financial and operating results for the first quarter ended March 31, 2013 and provided a corporate update.

"Last month, we were pleased to present positive data from the dose escalation portion of our TKM-PLK1 Phase I clinical trial conducted in a population of advanced cancer patients with solid tumors. The data generated demonstrated that TKM-PLK1 showed encouraging signs of drug activity with RNAi activity confirmed in tumor biopsy. We were encouraged that two patients enrolled with gastrointestinal carcinoid (neuroendocrine) cancer, both responded to treatment with TKM-PLK1, and building on these results, we plan to initiate a Phase II clinical trial in patients with previously treated gastrointestinal carcinoid (neuroendocrine) cancer in the second half of 2013," said Dr. Mark J. Murray, Tekmira's President and CEO.

"Our successful collaboration with the U.S. Department of Defense's JPM-TMT program is ongoing, with recent modifications to our approximately \$140 million contract, which will support development plans that integrate advancements in our lipid nanoparticle (LNP) formulation and manufacturing technologies, while providing additional funding for the TKM-Ebola program," added Dr. Murray.

"We are actively working to grow the number of collaborators using our technology, including pharmaceutical, biotechnology and agricultural companies. We also anticipate some near-term milestones from existing partners, including Alnylam, which has guided that it will present initial data from its ALN-TTR02 Phase II trial in mid-2013 and is on track to start a Phase III pivotal trial for ALN-TTR02 by the end of 2013. Tekmira expects to receive \$10 million in milestone payments from Alnylam in 2013," stated Dr. Murray.

### **Corporate Update and Highlights**

#### **Tekmira's Products**

##### ***TKM-PLK1, Tekmira's Lead Oncology Therapeutic***

Tekmira's lead oncology product candidate, TKM-PLK1, targets PLK1 (polo-like kinase 1), a protein required for 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.

Results from the dose escalation portion of a Phase I clinical trial were presented at the American Association for Cancer Research (AACR) Annual Meeting held from April 6-10, 2013 in Washington, DC. In this Phase I study, TKM-PLK1 was generally well-tolerated and showed encouraging signs of drug activity with RNAi activity confirmed in tumor biopsy. TKM-PLK1, which employs a unique LNP formulation for oncology applications, was administered to 24 patients at doses ranging from 0.15 mg/kg to 0.90 mg/kg. Dose-limiting toxicities included: one grade 3 transient thrombocytopenia in one patient (at 0.9 mg/kg) and one grade 3 hypoxia/dyspnea in another patient (at 0.9 mg/kg). Based on these data, the maximum tolerated dose is estimated to be 0.75 mg/kg. A 10-patient expansion cohort is currently enrolling patients at 0.75 mg/kg, with data expected later this year.

Patients had a mean of 5.1 prior treatment regimens (range of 1-14). Forty-four percent (4 out of 9) patients evaluable for response, treated at a dose equal to or greater than 0.6 mg/kg, showed clinical benefit. In particular, one patient with progressive, metastatic appendiceal carcinoid (neuroendocrine) cancer had a durable partial tumor response based on RECIST criteria, continuing for more than 10 months. Three other patients achieved stable disease, including one patient with metastatic appendiceal carcinoid (neuroendocrine) cancer, another patient with metastatic colorectal cancer, and a third patient with metastatic adrenocortical carcinoma.

Tekmira expects to initiate a Phase II clinical trial in patients with previously treated gastrointestinal carcinoid (neuroendocrine) cancer in the second half of 2013. Details of the trial design will be disclosed later this year. Tekmira is also evaluating

additional indications for Phase II development and will provide guidance later this year. Tekmira also expects to present data from the expansion cohort of the TKM-PLK1 Phase I clinical trial this year.

### ***TKM-Ebola, Tekmira's Collaboration with the U.S. Department of Defense***

TKM-Ebola, an anti-Ebola viral therapeutic, is being developed under a contract with the U.S. DoD's Joint Project Manager Transformational Medical Technologies (JPM-TMT) Office under a contract valued at approximately \$140 million. Tekmira's contract with the JPM-TMT was recently modified to support development plans that integrate a more potent LNP formulation and advancements in manufacturing technology, including lyophilization, as well as provide for \$6.9 million in additional funding. Tekmira has initiated pre-clinical, chemistry, manufacturing and control studies that support the use of these improvements in the TKM-Ebola program. Tekmira anticipates the completion of these studies and a submission to the FDA in the second half of 2013 in order to support the use of the enhanced product in a Phase I clinical trial. New data from the TKM-Ebola program will be presented at the 15th Annual TIDES Summit: Oligonucleotide and Peptide® Therapeutics from Research through Commercialization taking place in Boston, MA on May 15, 2013.

### ***Other Preclinical Candidates***

Tekmira is currently evaluating several preclinical candidates with potential in diverse therapeutic areas. The Tekmira research team will continue to generate data to support the advancement of the most promising of these targets and expects to nominate the next product candidate for development in 2013.

### **Partners' Products**

Tekmira has granted a license to Alnylam Pharmaceuticals, Inc. to use Tekmira's LNP technology to enable RNAi therapeutic products. Tekmira will receive milestone and royalty payments as these LNP-enabled products are developed and commercialized.

#### ***ALN-TTR02***

Alnylam has guided that initial results from a Phase II clinical trial with ALN-TTR02 — an RNAi therapeutic for the treatment of ATTR that is enabled by Tekmira's LNP technology — are anticipated to be reported in mid-2013. Tekmira is entitled to receive a \$5 million milestone payment when ALN-TTR02 enters a pivotal or Phase III clinical trial, which Alnylam has guided should occur by the end of 2013. Tekmira is eligible to receive royalty payments based on commercial sales of ALN-TTR.

#### ***ALN-VSP***

ALN-VSP, which is a systemically delivered RNAi therapeutic for the treatment of advanced solid tumors with liver involvement, is enabled by Tekmira's LNP technology. Complete results from the ALN-VSP Phase I clinical trial and extension study were published in January 2013 in the journal *Cancer Discovery*. ALN-VSP is partnered with Ascleptis Pharmaceuticals (Hangzhou) Co., Ltd., a privately held U.S.-China joint venture pharmaceutical company, for further advancement in the Chinese market. Tekmira is entitled to receive a US\$5 million milestone payment for enabling ALN-VSP to enter a clinical trial in China, which is expected to occur in 2013. Tekmira is eligible to receive royalty payments based on commercial sales of ALN-VSP.

#### ***ALN-PCS02***

ALN-PCS02, which is an RNAi therapeutic to treat hypercholesterolemia or high levels of cholesterol in the blood, is enabled by Tekmira's LNP technology. In February 2013, Alnylam disclosed an exclusive global alliance with The Medicines Company to advance the ALN-PCS program. Tekmira is eligible to receive royalty payments based on commercial sales of ALN-PCS02.

### ***Marqibo***

Marqibo, which is a liposomal formulation of the chemotherapy drug vincristine — along with two other liposomal chemotherapy products, Alocrest and Brakiva — were licensed from Tekmira to Talon Therapeutics, Inc. in 2006. Talon is responsible for all future development of these products. On August 9, 2012, Tekmira disclosed that Talon received accelerated approval from the FDA for Marqibo® (vinCRISTine sulfate LIPOSOME injection) for the treatment of adult patients with Philadelphia chromosome negative (Ph-) acute lymphoblastic leukemia (ALL) in second or greater relapse or whose disease has progressed following two or more anti-leukemia therapies. Tekmira is eligible to receive royalty payments based on Marqibo's commercial sales.

## **Financial Results**

### **Net income/loss**

For Q1 2013, net loss was \$2.6 million (\$0.18 per common share) as compared to a net loss of \$3.2 million (\$0.25 per common share) for Q1 2012.

## **Revenue**

Revenue was \$2.2 million for Q1 2013 as compared to \$3.6 million for Q1 2012.

Under a DoD contract to develop TKM-Ebola, Tekmira is being reimbursed for costs incurred, including an allocation of overheads, and is being paid an incentive fee. For this contract, Tekmira recorded \$2.0 million in revenue in Q1 2013 as compared to \$3.5 million in Q1 2012.

Contract revenues were higher in Q1 2012 as a Phase 1 clinical trial for TKM-Ebola was initiated and Tekmira began to acquire materials for scaling up its TKM-Ebola drug product manufacturing process. In Q1 2013 various program activities progressed, in particular, animal studies using a new LNP formulation.

## **Research, development, collaborations and contracts expenses**

Research, development, collaborations and contracts expenses were \$4.2 million in Q1 2013 as compared to \$4.1 million in Q1 2012.

TKM-Ebola contract expenses were lower in Q1 2013 than in Q1 2012 for similar reasons as discussed in "Revenue" above. However, in Q1 2013, Tekmira incurred more early stage research expense as work continued on identifying additional drug candidates for development. In Q1 2013, Tekmira also expensed a \$0.2 million license fee payment to Marina Biotech under a license to Marina's UNA technology.

## **General and administrative**

General and administrative expenses were \$0.9 million in Q1 2013 as compared to \$1.8 million in Q1 2012. Q1 2012 general and administrative expenses were higher as they included legal fees incurred in respect of a lawsuit against Alnylam and AICana that was settled in November 2012.

## **Financial guidance**

As disclosed in its 2012 year-end MD&A, Tekmira believes that its current funds on hand, plus expected income, including payments from current licensees, collaborative partners and the DoD will be sufficient to continue product development into 2015. Tekmira expects to have an aggregate balance of cash and cash equivalents and short-term investments of greater than \$35.0 million at the end of 2013.

## **Conference Call Information**

Tekmira will hold a conference call and webcast today (Tuesday, May 14, 2013) at 1:00 pm Pacific Time (4:00 pm Eastern Time) to discuss its first quarter 2013 results and provide a corporate update. A live webcast of the call can be accessed through the Investor section of Tekmira's website at [www.tekmirapharm.com](http://www.tekmirapharm.com). Or, alternatively, to dial into the conference call, please call 914-495-8556 or 1-866-393-1607.

An archived webcast of this conference call will be available on the Tekmira website approximately two hours after the event. Or alternatively, you may access a replay of the conference call available until May 17, 2013 by calling 404-537-3406 or 1-855-859-2056 and referencing conference ID 69658053.

## **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 Alnylam RNAi Technology

Tekmira has licenses to Alnylam RNAi intellectual property for certain siRNA programs.

## 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; estimates of the number of clinical development programs to be undertaken by Tekmira and its product development partners; selection of additional product candidates; timing of release of clinical data; the effects of Tekmira's products on the treatment of cancer, infectious disease, and other diseases; the effects of TKM-PLK1 on the treatment of cancer, including gastrointestinal carcinoid (neuroendocrine) tumors; the expected enrollment, completion and release of data from the expansion cohort of the TKM-PLK1 later this year; the evaluation of additional indications for TKM-PLK1 Phase II development, and guidance thereon; the expected timing of the release of trial design and other details about, and the initiation of, a Phase II clinical trial for TKM-PLK1; the modifications to the TKM-Ebola contract with the U.S. DoD's JPM-TMT office to integrate recent advancements in LNP formulation and manufacturing technology; the initiation of pre-clinical and chemistry, manufacturing and control studies that support the use of the advancements; the completion of these studies and submission to the FDA to support the use of the enhanced product in a Phase I clinical trial, and the timing thereon; the initiation of a Phase I clinical trial for TKM-Ebola; the quantum and timing of funding that may be provided to Tekmira pursuant to the TKM-Ebola contract with the U.S. DoD's JPM-TMT Office; the evaluation of preclinical candidates with data generation thereon to support target selection; the timing and nomination of Tekmira's next product candidate for development; Tekmira's expectations of entering into a separate cross license agreement with AICana, which includes anticipated milestone and royalty payments and an expected agreement for AICana not to compete in the RNAi field for five years, and expected payments upon execution of the cross-license agreement with AICana; the quantum and timing of future milestone royalty payments expected from the ALN-TTR02, ALN-VSP, ALN-PCS02 and other LNP-enabled product development programs of Alnylam; the timing and reporting of initial results from a Phase II clinical trial; the timing of an ALN-TTR02 pivotal or Phase III clinical trial, and related payments to Tekmira; the timing and initiation of a ALN-VSP clinical trial in China; milestones and royalty payments from Alnylam's LNP-enabled products; licenses from Alnylam for the discovery, development and commercialization of RNAi products directed to thirteen gene targets; the use of lipid nanoparticle technology by Tekmira's licensees and expected royalty payments from commercial sales of Tekmira's product development partners; statements about Tekmira's MV-RNA license with Halo-Bio, as well as milestone and royalty payments thereon; statements about Tekmira's Unlocked Nucleobase Analog (UNA) license with Marina, as well as milestone and royalty payments thereon; anticipated royalty payments based on sales of Marqibo; statements with respect to revenue and expense fluctuation and guidance; the quantum and timing of potential funding; statements about Tekmira's cash runway extending into 2015 and estimated cash and cash equivalents at the end of 2013; and estimates of the length of time Tekmira's business will be funded by its anticipated financial resources.

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, infectious disease, or other diseases; the developmental milestones and approvals required to trigger funding for TKM-Ebola from the JPM-TMT program; 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 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 including Alnylam, Talon, the DoD, and others; 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 in the

treatment of cancer, infectious disease, or other diseases; 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; the FDA may not approve the commencement of Tekmira's planned clinical trials or approve the use of Tekmira's products; Tekmira may not complete enrollment or release data from the expansion cohort of the TKM-PLK1 Phase I clinical trial in the expected time frame, or at all; Tekmira may not release details about the TKM-PLK1 Phase II trial design in the timeframe anticipated, or at all; TKM-PLK1 might not enter into Phase II clinical trials in the timeframe anticipated, or at all; there may be no additional indications for Phase II development; the DoD may reduce or cancel certain defense spending, including Tekmira's contract to develop TKM-Ebola, or adversely modify the contract with Tekmira; the FDA may decide that TKM-Ebola "Animal Rule" data is insufficient for approval and require additional pre-clinical, clinical or other studies, refuse to approve TKM-Ebola, or place restrictions on our ability to commercialize TKM-Ebola; Tekmira may not complete the work or studies necessary for the submission of the new LNP formulation for TKM-Ebola to the FDA in the anticipated timeframe, or at all; the FDA may not approve the new LNP formulation for TKM-Ebola; Tekmira may not initiate a new TKM-Ebola Phase I clinical trial in the anticipated timeframe, or at all; expected milestone or royalty payments related to the settlement and licensing agreement between Tekmira and Alnylam may not be received in the quantum and on the timing currently anticipated, or at all; additional exclusive or non-exclusive licenses from Alnylam may not be received as anticipated, or at all; initial results from the Phase II ALN-TTR02 trial may not be reported in the time frame currently contemplated, or at all; a Phase III or pivotal trial for ALN-TTR02 may not start as currently anticipated, or at all; clinical trials for ALN-VSP may not commence as anticipated, or at all; the possibility that Tekmira may not enter into a separate cross license agreement with AICana on the terms currently anticipated, or at all; 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; anticipated payments under contracts with Tekmira's collaborative partners may not be received by Tekmira on a timely basis, or at all, or in the quantum expected by Tekmira; UNAs may not have the effect of increasing stability or reducing off-target effects when incorporated into RNAi drugs; Tekmira may never develop a commercially viable product that uses UNA or MV-RNA technologies, or at all; the possibility that Marqibo may not be accepted in the marketplace or that Talon may not be able to develop adequate marketing and distribution capabilities; the possibility that Tekmira may not receive milestone and royalty payments based on the successful development and commercialization of Talon's Marqibo, Brakiva, and Alocrest product candidates; payments received from third parties may not be sufficient to fund Tekmira's continued business plan as currently anticipated; 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; Tekmira's cash runway may not extend into 2015 as anticipated, and may be substantially less than required to continue current operations; 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 [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.

Marqibo is a U.S. registered trademark of Talon Therapeutics, Inc.

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