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Tekmira's Partner Reports Positive Clinical Data for LNP-Enabled Patisiran

Tekmira's LNP Technology Enables Treatment With Favorable Tolerability Profile for a Period of Up to One Year

VANCOUVER, British Columbia, Oct. 14, 2014 (GLOBE NEWSWIRE) -- Tekmira Pharmaceuticals Corporation (Nasdaq:TKMR) (TSX:TKM), a leading developer of RNA interference (RNAi) therapeutics, today reported that Alnylam Pharmaceuticals, Inc. (Nasdaq: ALNY) presented six-month clinical data for the ongoing Patisiran Phase II Open Label Extension (OLE) study in patients with Familial Amyloidotic Polyneuropathy (FAP). Patisiran (ALN-TTR02) is an RNAi therapeutic targeting transthyretin (TTR) in development for the treatment of TTR-mediated amyloidosis (ATTR), which is enabled by Tekmira's lipid nanoparticle (LNP) technology.

"We are pleased with the positive results reported today in Alnylam's patisiran development program, demonstrating sustained knockdown of serum TTR of up to 90% and a favorable tolerability profile out to one year of treatment. This program is enabled by Tekmira's LNP technology and represents the most clinically advanced application of Tekmira's proprietary LNP delivery technology. Our LNP delivery technology underpins the most significant RNAi advances in the field, enabling multiple RNAi products in clinical development in a variety of therapeutic areas to address significant unmet medical needs. Today's announcement reinforces how our LNP technology continues to be the industry gold standard, and is associated with positive outcomes and clinical benefit," said Dr. Mark J. Murray, Tekmira's President and CEO.

Alnylam presented data at the American Neurological Association's 2014 Annual Meeting being held October 12 - 14 in Baltimore. Results showed a mean 0.95 point decrease in modified Neuropathy Impairment Score (mNIS+7) at six months in 19 patients with mNIS+7 data available for the current analysis. This decrease in neuropathy progression compares favorably with the 7 to 10 point increase in mNIS+7 at six months that can be estimated from historical data sets in untreated FAP patients with similar baseline characteristics (Adams *et al., International Symposium on Amyloidosis*, April 2014; Berk *et al., JAMA* 310: 26588-67, 2013; Tafamidis European Medicines Agency Assessment Report, 2011). In addition, patisiran treatment achieved a sustained mean serum TTR knockdown at the 80% target level for over nine months, with an up to 89.6% knockdown achieved between doses. Patisiran was found to be generally well tolerated in this study out to one year of therapy, with no drug-related serious adverse events to date, and all 27 patients enrolled in the study continue to receive drug treatment. Infusion reactions were infrequent, mild and did not result in any discontinuations.

For more detailed information about the newly presented Phase II data for patisiran (ALN-TTR02), please refer to the Alnylam <u>news release</u> dated Oct 13, 2014 and the presentation of these data, which can be found on Alnylam's website at <u>www.alnylam.com</u>.

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 therapeutics, based on RNAi trigger molecules 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 triggers. Tekmira's LNP platform is being utilized in multiple clinical trials by both Tekmira and its partner. 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 RNAi therapeutics 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 whose ratios 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 (LNP) delivery technology to pharmaceutical and biotechnology partners. Tekmira has been working in the field of nucleic acid delivery for over a decade, and has broad intellectual property covering its delivery technology. Further information about Tekmira can be found at www.tekmira.com. Tekmira is based in Vancouver, B.C. Canada

About Alnylam's Patisiran Phase II Open Label Extension Study

Alnylam's ongoing OLE study is treating patients that were previously enrolled in a Phase II study of patisiran in ATTR patients with FAP. The OLE study is an open-label, multi-center trial designed to evaluate the long-term safety and tolerability of patisiran administration. The two-year study has completed enrollment with 27 patients who receive 0.3 mg/kg of patisiran once every three weeks. This study is also measuring a number of clinical endpoints every six months, including mNIS+7 which is an evaluation of muscle weakness, sensory and autonomic function, and nerve conductance, where neuropathy progression leads to an increased score over time. The mNIS+7 measurement is the primary endpoint in the company's Phase 3 APOLLO trial of patisiran in FAP patients.

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," "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 new clinical data for patisiran (ALN-TTR02), progress in our partner's patisiran development program; Phase II Open Label Extension (OLE); and Tekmira's LNP technology which has enabled the development of ALN-TTR02.

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; mRNA is efficiently delivered using Tekmira's LNP; and the use of LNP technology by Tekmira's development partners and licensees; 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 products may not prove to be effective as therapeutic treatments for 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; preclinical 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; 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; 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 10-K for the year ended December 31, 2013 (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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