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Dicerna Announces License Agreement with Tekmira to Advance Dicerna's PH1 Development Program

WATERTOWN, Mass.--(BUSINESS WIRE)-- Dicerna Pharmaceuticals, Inc. (NASDAQ:DRNA), a leading developer of RNA interference (RNAi) therapeutics, today announced a licensing agreement for Dicerna to use Tekmira's proprietary lipid nanoparticle (LNP) technology for delivery of DCR-PH1, Dicerna's investigational product candidate for primary hyperoxaluria type 1 (PH1), a rare, inherited liver disorder that often results in kidney failure, and for which there are no approved therapies.

This announcement follows the successful testing of DCR-PH1 in combination with Tekmira's LNP technology in animal models, including mice and non-human primates. Under the agreement, Dicerna will pay Tekmira $2.5 million upfront, as well as $22 million in potential development milestones, and a mid-single-digit royalty on future PH1 sales.

Tekmira's LNP system has shown in other human clinical studies to provide potent, safe and effective RNA delivery to hepatocytes (liver cells). Licensing Tekmira's LNP will streamline the development path for DCR-PH1 and allows Dicerna to focus its LNP efforts on its oncology pipeline.

"Dicerna is focused on realizing the full clinical potential of our proprietary pipeline of highly targeted RNAi therapies by applying proven technologies," said Douglas Fambrough, Ph.D., Chief Executive Officer of Dicerna. "By drawing on Tekmira's extensive and deep experience with lipid nanoparticle delivery to the liver, the agreement will streamline the development path for DCR-PH1. We look forward to initiating Phase 1 trials of DCR-PH1 in 2015, aiming to fill a high unmet medical need for patients with PH1."

"This new agreement validates our leadership position in RNAi delivery and underscores the significant value we can bring to partners who leverage our LNP technology," said Dr. Mark J. Murray, President and CEO of Tekmira. "Our LNP technology is enabling the most advanced applications of RNAi therapeutics in the clinic. We are excited to be working with Dicerna in advancing a needed, investigational therapeutic for the treatment of PH1."

About RNAi

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.

About Tekmira's LNP Technology

Tekmira 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 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 that 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 Primary Hyperoxaluria Type 1 (PH1)

PH1 is a rare, inherited liver disorder that often results in severe damage to the kidneys. The disease can be fatal unless the patient undergoes a liver-kidney transplant, a major surgical procedure that is often difficult to perform due to the lack of donors and the threat of organ rejection. In the event of a successful transplant, the patient must live the rest of his or her life on immunosuppressant drugs, which have substantial associated risks. Currently, there are no FDA approved treatments for PH1.

PH1 is characterized by a genetic deficiency of the liver enzyme alanine:glyoxalate-aminotransferase (AGT), which is encoded by the AGXT gene. AGT deficiency induces overproduction of oxalate by the liver, resulting in the formation of crystals of calcium oxalate in the kidneys. Oxalate crystal formation often leads to chronic and painful cases of kidney stones and subsequent fibrosis (scarring), which is known as nephrocalcinosis. Many patients progress to end-stage renal disease (ESRD) and require dialysis or transplant. Aside from having to endure frequent dialysis, PH1 patients with ESRD may experience a build-up of oxalate in the bone, skin, heart and retina, with concomitant debilitating complications. While the true prevalence of

primary hyperoxaluria is unknown, it is estimated to be one to three cases per one million people.[1] Fifty percent of patients with PH1 reach ESRD by their mid-30s.[2]

About DCR-PH1

Dicerna is developing DCR-PH1, which is in preclinical development, for the treatment of PH1. DCR-PH1 is engineered to address the pathology of PH1 by targeting and destroying the messenger RNA (mRNA) produced by HAO1, a gene implicated in the pathogenesis of PH1. HAO1 encodes glycolate oxidase, a protein involved in producing oxalate. By reducing oxalate production, this approach is designed to prevent the complications of PH1. In preclinical studies, DCR-PH1 has been shown to induce potent and long-term inhibition of HAO1 and to significantly reduce levels of urinary oxalate, while demonstrating long-term efficacy and tolerability in animal models of PH1.

About Dicerna's Dicer Substrate Technology

Dicerna's proprietary RNAi molecules are known as Dicer substrates, or DsiRNAs, so called because they are processed by the Dicer enzyme, which is the initiation point for RNAi in the human cell cytoplasm. Dicerna's discovery approach is believed to maximize RNAi potency because the DsiRNAs are structured to be ideal for processing by Dicer. Dicer processing enables the preferential use of the correct RNA strand of the DsiRNA, which may increase the efficacy of the RNAi mechanism, as well as the potency of the DsiRNA molecules relative to other molecules used to induce RNAi.

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 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, Canada and Seattle, USA.

About Dicerna

Dicerna Pharmaceuticals, Inc., is a biopharmaceutical company focused on the discovery and development of innovative treatments for rare, inherited diseases involving the liver and for cancers that are genetically defined. The company is using its proprietary RNA interference (RNAi) technology platform to build a broad pipeline in these therapeutic areas. In both rare diseases and oncology, Dicerna is pursuing targets that have been difficult to address using conventional approaches, but where connections between targets and diseases are well understood and documented. The company intends to discover, develop and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners.

Cautionary Note on Forward-Looking Statements

This press release includes forward-looking statements. Such forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements. Applicable risks and uncertainties include that LNP technology may fail to deliver DCR-PH1 to the liver in human beings or otherwise fail to accelerate clinical development, and that clinical trials may not demonstrate the effectiveness of DCR-PH1. Additional risks, including those relating to Dicerna's preclinical research and clinical development and other risks, are identified under the heading "Risk Factors" included in Dicerna's most recent Form 10-Q filing and in other future filings with the SEC. The forward-looking statements contained in this press release reflect Dicerna's current views with respect to future events, and Dicerna does not undertake and specifically disclaims any obligation to update any forward-looking statements.

References

1 Cochat, P, Rumsby, G. Primary hyperoxaluria. The New England Journal of Medicine 2013; 369(7): 649-658.

2 Rare Kidney Stone Consortium. Primary Hyperoxaluria. 2010. Available at: http://www.rarekidneystones.org/hyperoxaluria/physicians.html. Accessed October 14, 2014.

Investors:
Westwicke Partners
Yolanda Taylor, 617-366-6803
yolanda.taylor@westwicke.com
or
Media:
SmithSolve
Alex Van Rees, 973-442-1555 ext. 111

alex.vanrees@smithsolve.com

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