Current Agreements

Dealdoc

Collaboration and licensing agreement for lipid nanoparticle delivery technology for use in primary hyperoxaluria type 1 development program

Tekmira Pharmaceuticals
Dicerna Pharmaceuticals

Nov 17 2014

Collaboration and licensing agreement for lipid nanoparticle delivery technology for use in primary hyperoxaluria type 1 development program

Companies:	Tekmira Pharmaceuticals Dicerna Pharmaceuticals
Announcement date:	Nov 17 2014
Deal value, US$m:	24.5 : sum of upfront and milestone payments

Details

Announcement date:	Nov 17 2014
Industry sectors:	Drug delivery Pharmaceutical
Asset type:	Compound Technology
Therapy areas:	Genetic disorders » Rare genetic disorders Metabolic » Liver disease
Technology types:	Drug delivery » Targeted Nanotechnology Small molecules
Deal components:	Collaborative R&D Licensing
Stages of development:	Preclinical Formulation

Financials

Deal value, US$m:	24.5 : sum of upfront and milestone payments
Upfront, US$m:	2.5 : upfront payment
Milestones, US$m:	22 : aggregate development milestones
Royalty rates, %:	n/d : mid-single-digit royalty on future PH1 sales
Semi-quant royalties:	Mid single digit

Termsheet

Tekmira Pharmaceuticals announced a licensing and collaboration agreement with Dicerna Pharmaceuticals.

Tekmira has licensed its proprietary lipid nanoparticle (LNP) delivery technology for exclusive use in Dicerna's primary hyperoxaluria type 1 (PH1) development program.

Dicerna will pay Tekmira $2.5 million upfront and payments of $22 million in aggregate development milestones, plus a mid-single-digit royalty on future PH1 sales.

This new partnership also includes a supply agreement with Tekmira providing clinical drug supply and regulatory support in the rapid advancement of the product candidate.

The agreement announced today follows the successful testing and demonstration of positive results combining Tekmira's LNP technology with DCR-PH1 in pre-clinical animal models.

Dicerna will use Tekmira's third generation LNP technology for delivery of DCR-PH1, Dicerna's Dicer substrate RNA (DsiRNA) molecule, for the treatment of PH1, a rare, inherited liver disorder that often results in kidney failure and for which there are no approved therapies.

Press Release

Tekmira Announces Licensing and Collaboration Agreement With Dicerna

Tekmira's LNP to Enable Dicerna's PH1 Candidate

VANCOUVER, British Columbia, Nov. 17, 2014 (GLOBE NEWSWIRE) -- Tekmira Pharmaceuticals Corporation (Nasdaq:TKMR) (TSX:TKM) a leading developer of RNA interference (RNAi) therapeutics, today announces a licensing and collaboration agreement with Dicerna Pharmaceuticals, Inc. Tekmira has licensed its proprietary lipid nanoparticle (LNP) delivery technology for exclusive use in Dicerna's primary hyperoxaluria type 1 (PH1) development program.

Under the agreement, Dicerna will pay Tekmira $2.5 million upfront and payments of $22 million in aggregate development milestones, plus a mid-single-digit royalty on future PH1 sales. This new partnership also includes a supply agreement with Tekmira providing clinical drug supply and regulatory support in the rapid advancement of the product candidate.

The agreement announced today follows the successful testing and demonstration of positive results combining Tekmira's LNP technology with DCR-PH1 in pre-clinical animal models.

Dicerna will use Tekmira's third generation LNP technology for delivery of DCR-PH1, Dicerna's Dicer substrate RNA (DsiRNA) molecule, for the treatment of PH1, a rare, inherited liver disorder that often results in kidney failure and for which there are no approved therapies.

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

"As a core pillar of our business strategy, we continue to engage in partnerships where our technology improves the risk profile and accelerates the development programs of our collaborators and provides meaningful non-dilutive financing to TKMR," added Dr. Murray.

"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."

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.

AboutTekmira's LNP Technology

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 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 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 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.

Filing Data

Not available.

Contract

Not available.