



News Release

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Dicerna Pharmaceuticals Announces First DsiRNA Therapeutic Candidate Selected for Development, Triggering \$5 Million Milestone Payment

KHK Exercises Option for Additional Oncology Target

WATERTOWN, Mass. – December 15, 2011 – Dicerna Pharmaceuticals, Inc. (Dicerna), a second generation RNA interference (RNAi) company developing novel therapeutics utilizing its proprietary Dicer Substrate Technology™ and Dicer Substrate siRNA (DsiRNA) molecules, announced today that Kyowa Hakko Kirin Co., Ltd. (TSE: 4151) (KHK) has elected to advance its first collaborative therapeutic oncology candidate from the research stage into formal development studies, triggering a \$5 million milestone payment for Dicerna. In addition, KHK has exercised an option to bring a second oncology target into the collaboration.

In January 2010, Dicerna [announced](#) a research collaboration and license agreement with KHK worth up to \$1.4 billion for the research, development and commercialization of DsiRNA pharmaceuticals and drug delivery systems (DDS) for therapeutic targets in oncology, and in December 2010 [expanded](#) their collaboration into immunologic and inflammatory diseases.

“This is a landmark milestone for Dicerna that provides excellent validation of our DsiRNA technology,” said Douglas M. Fambrough, Ph.D., chief executive officer of Dicerna. “We have reached this point with our partner KHK as a result of the power of the science and the strength of our collaboration. With this program, we have been able to convincingly demonstrate that classically undruggable gene targets can now be effectively drugged, reinforcing the therapeutic potential of the technology. We believe this will be the first of many development programs that result from our Dicer Substrate Technology.”

“Kyowa Hakko Kirin has been pleased with the speed with which we have been able to progress this program into formal development,” said Etsuo Ohshima, Ph.D., managing officer and vice president, head, research division at Kyowa Hakko Kirin. “Working in a very collaborative fashion, our teams have quickly progressed from the identification of highly active DsiRNAs to the unequivocal demonstration of *in vivo* efficacy in a very important tumor model. We are also pleased that this success has been achieved through the combined platform of the DsiRNA molecules and our proprietary DDS technology. This will be a powerful and productive

combination going forward, such that we are also exercising an option with Dicerna to initiate a program on an additional oncology target to enter the research stage immediately.”

The decision to move the first candidate into development triggers the milestone payment to Dicerna. KHK will be responsible for the continuing development of the program, while Dicerna retains a commercialization and profit-share opt-in right. The option exercise by KHK on the additional, new oncology target triggers an undisclosed payment to Dicerna. In addition, the companies continue their collaboration in the inflammation and immunology area.

The nomination for development follows achievement of complete tumor regression by a DsiRNA delivered via KHK’s DDS in an *in vivo* model system of a highly aggressive and difficult to treat human cancer. In the key experiments, repeated multiple times and well-controlled to isolate target specific effects, the current chemical standard of care achieved only a slowing of the tumor growth rate, as was expected. The DsiRNA targets an oncogene that is not druggable by traditional pharmaceutical technologies such as small molecules and antibodies.

“Although our target gene is known to be activated in a large majority of patients with this tumor type, the target has remained beyond the reach of the pharmaceutical industry,” said Bob Brown, Ph.D., senior vice president, research of Dicerna. “Through the use of DsiRNA and the RNAi mechanism, we have demonstrated not only the central role this gene and its product play in this cancer, but also that intervention at this single target can eradicate the disease in animal models.”

About Dicer Substrate RNAi

Dicer is a critical enzyme involved in the RNAi gene silencing cascade and acts as the natural initiation point for this pathway by processing double-stranded RNA so that it can be used for gene silencing. Dicer then delivers these modified small RNA molecules to the mature gene silencing complex. Dicerna’s synthetic Dicer Substrate siRNA (DsiRNA) molecules are 25 or more base pairs in length and are processed by Dicer. By utilizing this distinct early entry point into the pathway, DsiRNA molecules have greater potency and longer duration of action than other RNAi approaches. In addition, DsiRNA molecules have enhanced delivery potential because their structure creates a natural conjugation point for cellular targeting agents.

About Dicerna Pharmaceuticals

Dicerna Pharmaceuticals is a private, venture-backed RNAi-focused biopharmaceutical company developing novel therapeutic agents and related drug delivery systems in oncology and other disease areas based on its proprietary Dicer Substrate Technology™ platform and Dicer Substrate siRNA (DsiRNA) molecules. Dicer Substrate Technology™ is a second generation RNAi approach that results in greater potency, longer duration of action and enhanced delivery potential, differentiating it from other RNAi approaches. Dicerna has a major alliance with Kyowa Hakko Kirin for DsiRNA pharmaceuticals and drug delivery systems focused in oncology, immunology and inflammation. The company also has a partnership with Ipsen to research and develop novel DsiRNA therapeutics with targeted delivery in oncology and endocrinology. Dicerna is based in Watertown, Massachusetts. For more information, please visit www.dicerna.com.

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