



News Release

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Dicerna Data Highlighted at ASCO by UCSF Cancer Researcher Frank McCormick During Plenary Session

Novel DsiRNA Approach Shows Promise in Oncology

WATERTOWN, Mass. – June 6, 2010 – 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, today announced that data from the company was highlighted in the scientific plenary discussion at the annual meeting of the American Society of Clinical Oncology (ASCO), which took place June 4-8, 2010 in Chicago. The presentation, which led the General Oncology Special Session on Sunday, June 6, was given by Frank McCormick, Ph.D., FRS, director of the University of California San Francisco Helen Diller Family Comprehensive Cancer Center and a member of Dicerna's scientific advisory board. Notably, Dr. McCormick is the recent recipient of one of ASCO's 2010 Special Awards – the distinguished Science of Oncology Award and Lecture – for his outstanding contributions to translational research in cancer.

Dr. McCormick presented a historical perspective on the pharmaceutical industry's previously unsuccessful attempts to develop small molecule therapies capable of effectively blocking the activation or the downstream effects of important oncogenes, such as *KRAS*, a gene that plays a central role in the growth, differentiation and survival of cells. Mutations in the *KRAS* gene are implicated in multiple cancers, including various leukemias, lung cancer, colorectal and pancreatic tumors. As counterpoint, Dr. McCormick presented data from Dicerna demonstrating that DsiRNA molecules have potent *in vivo* activity and pharmaceutical properties that significantly inhibit *KRAS* gene expression in preclinical models.

“Having looked across the data presented at ASCO, I believe *KRAS* expression is effectively inhibited by Dicerna DsiRNA molecules and that targeting *KRAS* could potentially be an important contribution to cancer treatment,” stated Dr. McCormick. “*KRAS* has proven to be a particularly intractable and ‘undruggable’ oncology drug target using conventional small molecule approaches.”

“Dicerna's DsiRNA molecules are designed to have a distinct early entry point into the gene silencing pathway, resulting in greater potency and longer duration of action than first generation RNAi technologies. We are pleased that the advantages of our next generation RNAi approach

are gaining recognition in the research community, as highlighted by Dr. McCormick during ASCO's plenary session," said Douglas Fambrough, Ph.D., chief executive officer at Dicerna.

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 (RISC). 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

Dicerna Pharmaceuticals is a private, venture-backed RNAi-focused biopharmaceutical company developing novel therapeutic agents and related drug delivery systems in multiple 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 believes that its Dicer Substrate Technology is based on intellectual property that is both broadly enabling and distinct from other IP in the field. Dicerna has exclusive, worldwide rights to the Dicer Substrate Technology and has the sole right to grant sublicenses to the company's portfolio of Dicer Substrate Technology intellectual property. Dicerna has a major alliance with Kyowa Hakko Kirin for DsiRNA pharmaceuticals and drug delivery systems, initially focused on oncology. 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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