



News Release

Contacts:

For Dicerna:

Martin Williams, SVP & Chief Business Officer
Dicerna Pharmaceuticals
617-621-8097

Michele Rozen
Pure Communications, Inc.
617-953-2214

Dicerna to Present New Data on Dicer Substrate siRNA Molecules at Cambridge Healthtech Institute's Second Annual RNA Interference: From Tools to Therapies Conference

Dicerna to Chair, Moderate and Participate in Several RNAi Conference Sessions

WATERTOWN, Mass. – February 2, 2010 – Dicerna Pharmaceuticals, Inc., 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 the company will present new preclinical data at the Cambridge Healthtech Institute's Second Annual RNA Interference: From Tools to Therapies Conference, as part of Cambridge Healthtech Institute's 17th International Molecular Medicine Tri-Conference 2010. The conference is being held February 3-5, 2010 at the Moscone North Convention Center in San Francisco.

Bob D. Brown, Ph.D., senior vice president of research at Dicerna, will present new preclinical data in a session titled "Dicer Substrate siRNA (DsiRNA) Activity *In Vivo* and Absence of microRNA Disruption" on February 4, 2010 at 5:20 p.m. (PST). The data demonstrates that DsiRNA treatment was sufficient to knockdown target gene expression by more than 95 percent and does not interfere with microRNA (miRNA) expression. Microarray analysis of 598 murine miRNAs was used to quantitate miRNA expression in the liver and spleen from mice treated with DsiRNA versus controls. Data from the miRNA arrays confirm that DsiRNA-directed knockdown of an oncogene target in the liver and spleen had no impact on endogenous miRNA biogenesis. In his presentation, Dr. Brown will discuss the superiority in activity of DsiRNA compared to siRNA *in vitro* and *in vivo* and show data from Dicerna's internal drug delivery program.

"I look forward to presenting these results at the Cambridge Healthtech Institute's Second Annual RNA Interference Conference," stated Dr. Brown. "These exciting conclusions from the miRNA array analysis show that DsiRNA does not impact miRNA biogenesis *in vivo*, and also show profound target knockdown *in vivo* in tumors, indicating the potential to halt disease-causing genes responsible for a vast number of acute conditions such as cancer."

In addition to presenting preclinical data, Dr. Brown will moderate a breakout discussion titled "Progress in Developing RNAi Therapeutics" on February 3, 2010 at 5:20 p.m. (PST) and will

also give chairperson's opening remarks for the morning session track titled "Novel Formulations for RNAi Delivery" on February 5, 2010 at 8:30 a.m. (PST). Also on February 5, Dr. Brown and John Rossi, Ph.D., scientific co-founder and scientific advisory board chair for Dicerna, as well as professor in the Department of Molecular and Cellular Biology and dean, Graduate School of Biological Sciences at City of Hope's Beckman Research Institute, will participate in a panel titled "Do We Understand the Challenges We Face with RNAi Therapeutics?" at 11:00 a.m. (PST).

On that same day, Dr. Rossi will give chairperson's remarks for the afternoon session track titled "Novel Approaches for Targeted Delivery" at 1:00 p.m. (PST) and present a session titled "*In vivo* delivery of Dicer substrate RNAs for treatment of HIV infection" at 1:05 p.m. (PST). In his presentation, Dr. Rossi will discuss the use of aptamers and dendrimers to deliver DsiRNAs *in vivo*. The results obtained demonstrate that DsiRNAs can be delivered in a cell type specific manner with an aptamer, and can be generally delivered with dendrimers to effectively inhibit HIV replication in a humanized mouse model.

"RNAi is a powerful mechanism that can be used for treatment of HIV infection and has many advantages over conventional drugs," said Dr. Rossi. "These data, along with Dr. Brown's data presentation, further validate and reinforce the ability of Dicerna's unique Dicer Substrate Technology platform to address a broad range of potentially life-threatening diseases. The company looks forward to having a strong presence at this year's conference."

About Dicer Substrate RNAi

RNA interference (RNAi) is a key cellular mechanism regulating gene expression in both normal and disease processes. Dicer is a critical enzyme involved in the RNAi gene silencing cascade and acts earlier in the pathway, preparing double-stranded RNA for processing. Dicer then hands off these 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 take advantage of this natural and early entry point. Based on gene sequence rather than protein structure, RNAi therapeutics have the potential to silence disease-causing genes using small synthetic RNA molecules, which will enable the development of new therapies for many acute and chronic diseases. The resulting target gene knockdown reduces gene expression in a way that is highly selective and specific.

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). The company is developing second generation DsiRNA-based therapies that engage the enzyme Dicer within cells, an early step in the gene silencing process, to exploit a natural initiation point for the RNAi cascade. DsiRNA takes advantage of this distinct biological pathway, resulting 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 full portfolio of Dicer Substrate intellectual property. Dicerna has a major alliance with Kyowa Hakko Kirin for DsiRNA pharmaceuticals and drug delivery systems, initially focused on oncology, and a collaboration with Archemix to research

novel conjugates of DsiRNA and aptamers for targeted delivery. Dicerna is based in Watertown, Massachusetts. For more information, please visit www.dicerna.com.

###