

# Dicerna Pharmaceuticals (DRNA)

## DRNA Licenses Validated Delivery Technology for DCR-PH1

### Key Takeaway

**Today, DRNA announced it has in-licensed Tekmira's (TKM.CN, NC) lipid nanoparticle (LNP) delivery technology for use with its PH1 development candidate, DCR-PH1. We view this as a positive, as Tekmira's proprietary LNP technology has been validated in humans and manufacturing is in place. As such, we believe this reduces the risk to the future development of DCR-PH1, which is expected to enter Phase 1 studies in 2015. We reiterate our Buy rating and \$48 PT.**

**DRNA In-Licenses Validated Delivery Technology.** DRNA announced a deal with Tekmira, where DRNA will use Tekmira's LNP delivery technology rather than its internally developed EnCore LNP delivery system for the development of DCR-PH1. Recall, EnCore was initially developed by DRNA for delivery in oncology indications and is currently being used in DRNA's MYC and KRAS targeted programs. While DRNA will continue to use EnCore for its oncology programs, the company has in-licensed Tekmira's LNP delivery for use solely in DRNA's PH1 program. The company had been developing DCR-PH1 in parallel with both EnCore and Tekmira's LNP, and has decided to use Tekmira's technology given it has been validated in humans and has manufacturing capabilities fully in place. Notably, Tekmira's LNP technology is used in Alnylam's (ALNY, NC) ALN-TTR02, which is in Phase 3 development for the treatment of TTR Amyloidosis. DRNA will pay Tekmira \$2.5m upfront, with \$22m in potential development milestones, and a mid-single digit royalty rate. We view these as reasonable economics for the risk reduction in using Tekmira's delivery technology.

**DRNA Announces Proprietary Extended Dicer Substrate And Delivery Technology.** DRNA announced that DCR-PH1 now incorporates a new and proprietary extended Dicer substrate RNA molecule (DsiRNA-EX) that will now be delivered via Tekmira's LNP. In the DCR-PH1 DsiRNA-EX molecule, one of the two RNA strands carries a 10-base extension that is thought to improve immunosilencing and stability. DsiRNA-EX is covered by DRNA patents outside of the patents covered under the City of Hope (COH) license, therefore DCR-PH1 and future programs will no longer owe a royalty to COH on sales. Further, DRNA has developed a proprietary DsiRNA-EX conjugate-based delivery system that will be used in lieu of Tekmira's LNP and DRNA's EnCore LNP in DRNA's pipeline of liver-targeted RNAi candidates. This delivery system does not use LNP technology and can be delivered either subcutaneously or intravenously. We see this as a positive, given the competitive landscape and the potential need for flexible administration routes for chronic indications.

**Upcoming Catalysts.** The company will host a R&D webcast on December 15 where we expect to learn more about the DCR-PH1 data using Tekmira's LNP technology, which has been tested in both mice and non-human primates. Further, we look forward to learning more about DRNA's DsiRNA-EX molecules and DsiRNA-EX conjugate-based delivery that will be used in future programs.

**BUY**

Price target \$48.00

Price \$9.50

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## Company Description

Dicerna Pharmaceuticals is a Watertown, MA-based therapeutics company focused on developing RNA interference (RNAi) technologies targeting liver and cancer. Dicerna has partnered two oncology development programs with the global pharmaceutical company Kyowa Hakko Kirin Co., Ltd. (KHK) targeting KRAS and CKAP5. DRNA's unpartnered programs are DCR-PH1 for Type 1 primary hyperoxaluria, a rare orphan disease resulting in renal failure, and DCR-M1711 targeting MYC for solid tumors.

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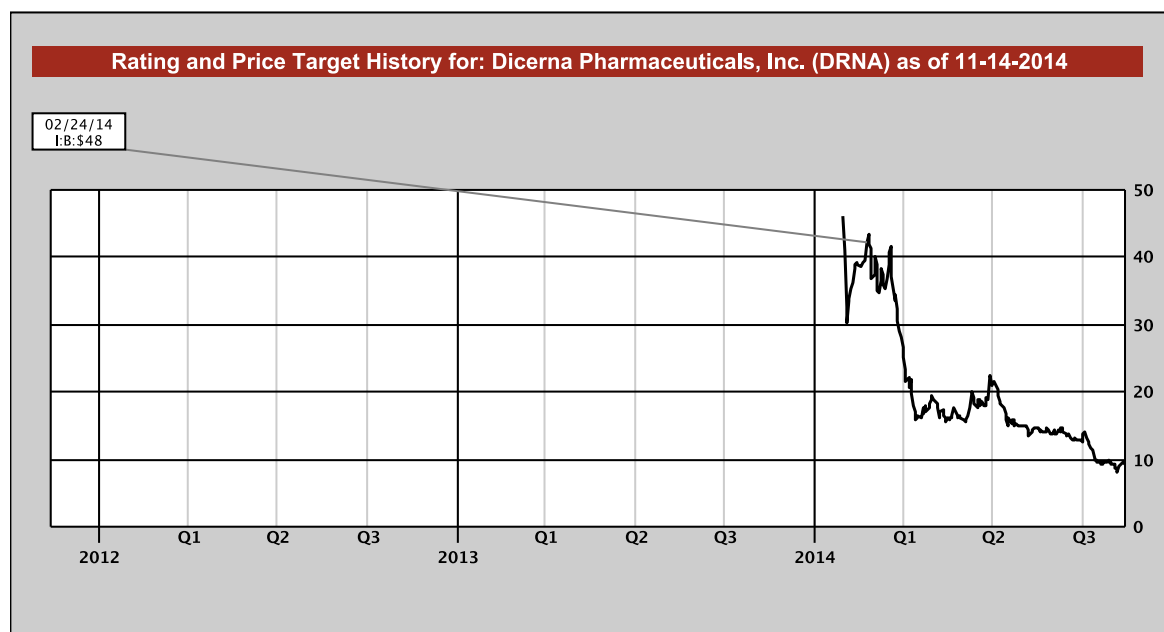
page 2 of 5

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			Count	Percent
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