

Reason for report:

**FLASH NOTE****BIOTECHNOLOGY****New LNP Licensing Agreement Positive for DRNA; Additional Validation for TKMR**

• **Bottom Line:** TKMR (MP) and DRNA (OP) today announced a licensing agreement for DRNA to use TKMR's lipid nanoparticle (LNP) technology for delivery of DCR-PH1, DRNA's product candidate for primary hyperoxaluria type 1 (PH1). We view the agreement positively for DRNA, since it significantly derisks human delivery of DCR-PH1. We believe TKMR's LNP technology currently represents a highly validated delivery technology for RNAi payloads and by in licensing TKMR's technology, DRNA avoids risks around developing its own liver-targeted LNP platform in our view. We believe the deal comes at attractive financial terms for DRNA who remains on track to initiate a Phase I trial in PH1 in 2015. Longer term, we think innovation in the area of subcutaneous delivery will likely surpass less convenient intravenous LNP-mediated RNAi. Similar to ALNY (OP), DRNA's future liver targeted programs will be based on a proprietary subcutaneous conjugate-mediated delivery technology. In addition, DRNA also disclosed a new payload strategy ("DsiRNA-EX") to be highlighted at an upcoming R&D webcast on 12/15 which we think could bypass existing IP estates.

S&amp;P 500 Health Care Index:

777.26

**Companies Highlighted:**  
ALNY, DRNA, TKMR

• **DRNA announced a licensing agreement with TKMR to advance its PH1 development program.** Under the agreement, DRNA will use TKMR's 3rd Gen. lipid nanoparticle (LNP) technology for delivery of DCR-PH1 (primary hyperoxaluria type 1 (PH1)). DRNA will pay TKMR \$2.5M upfront and \$22M in development milestones, which we view as great terms for DRNA who also agreed to pay mid-single-digit percent royalties to TKMR on future PH1 sales. TKMR will provide clinical supply and regulatory support in advancement of the candidate. This agreement is based on positive preclinical results in mice and non-human primates combining TKMR's LNP technology with DCR-PH1. DRNA remains on track to initiate a Phase I trial in PH1 in 2015, and expects to have initial Phase I data by the end of 2015. Preclinical data is expected to be presented at DRNA's upcoming R&D webcast on 12/15.

• **We view this collaboration as highly positive for DRNA and validating for TKMR.** First, we believe utilizing TKMR's 3rd generation LNP technology derisks DRNA's DCR-PH1 program, given we know TKMR's platform can be used to successfully delivery payload in humans. Second, we view the licensing terms as attractive for DRNA with only \$2.5M upfront expenses. Third, this strategic change streamlines DRNA's development process as it no longer has to invest in developing its own proprietary EnCore LNP technology for delivery to the liver. EnCore-delivered DCR-MYC (in Phase I for oncology indications) was optimized for tumor delivery, and DRNA mgmt highlighted important differences between liver and tumor-directed forms of EnCore, for example binding to ApoE, PEG coat as well as hydrophobicity of the core. For TKMR, we view this collaboration as validating for TKMR's LNP delivery technology.

• **DRNA's new improved RNAi payload potentially provides additional functionality and could bypass existing IP estates.** DRNA's DCR-PH1 asset will employ DRNA's new proprietary extended Dicer substrate

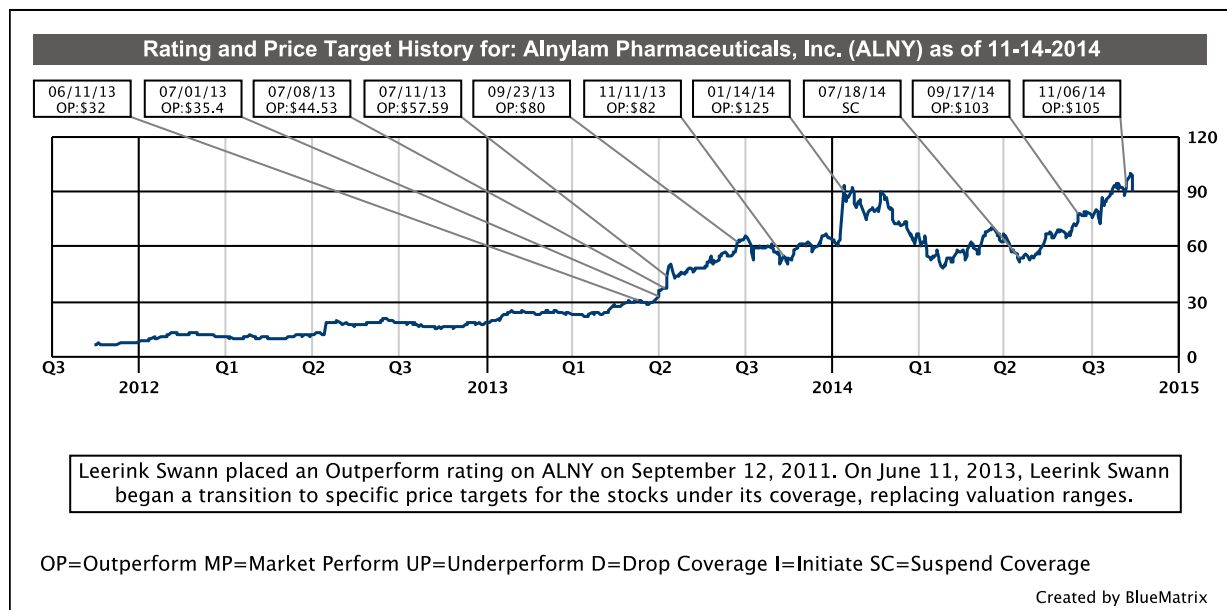
molecule (DsiRNA-EX) technology, in which one strand carries a 10-base extension. This extended structure improves the immunosilencing and stability properties of the DsiRNA-EX molecule. DsiRNA-EX technology is covered by DRNA patent 8,349,809 and other patent applications and is distinct from other existing RNAi patent estates. Recall, the Rossi patent estate forms the core of DRNA's IP claims and DRNA believes that it may not owe low single digit royalties to the City of Hope (Rossi IP holder). DRNA will host an R&D day on December 15th and will discuss these programs in greater detail.

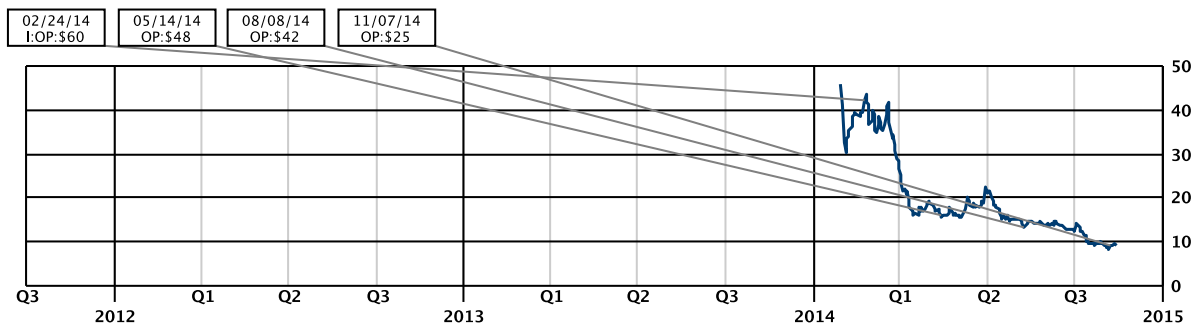
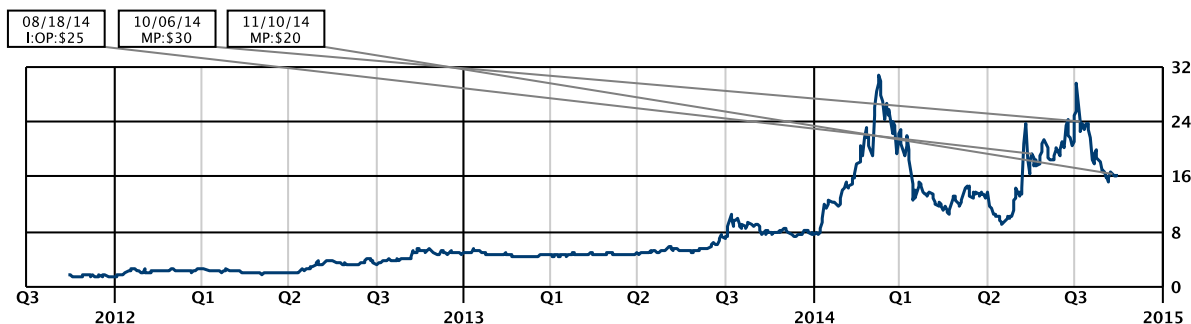
- **DRNA expects to declare its first DsiRNA-EX conjugate clinical candidate in 2015 for an undisclosed rare liver disease.** This conjugation will allow for subcutaneous delivery. DRNA is pursuing discovery research on several rare diseases involving the liver and intends to add additional programs over time.

## Disclosures Appendix

### Analyst Certification

I, Michael Schmidt, Ph.D., certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.



**Rating and Price Target History for: Dicerna Pharmaceuticals, Inc. (DRNA) as of 11-14-2014**

**Rating and Price Target History for: Tekmira Pharmaceuticals Corp. (TKMR) as of 11-14-2014**


Distribution of Ratings/Investment Banking Services (IB) as of 09/30/14				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	138	69.30	51	37.00
HOLD [MP]	61	30.70	2	3.30
SELL [UP]	0	0.00	0	0.00

## Explanation of Ratings

**Outperform (Buy):** We expect this stock to outperform its benchmark over the next 12 months.

**Market Perform (Hold/Neutral):** We expect this stock to perform in line with its benchmark over the next 12 months.

**Underperform (Sell):** We expect this stock to underperform its benchmark over the next 12 months. The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over \$2 billion.

## Important Disclosures

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Leerink Partners LLC has acted as the manager for a public offering of Dicerna Pharmaceuticals, Inc. in the past 12 months.

Leerink Partners LLC has acted as the manager for a public offering of Tekmira Pharmaceuticals Corp. in the past 12 months.

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