FLASH NOTE

Company Update

USA | Healthcare | Biotechnology

October 14, 2014

Jefferies

Dicerna Pharmaceuticals (DRNA)
ALNY PH1 Program Emerges As A Competitor

BUY
Price target $48.00
Price $10.31

EQUITY RESEARCH AMERICAS

Key Takeaway

ALNY unveiled its primary hyperoxaluria 1 (PH1) program, ALN-GO1, through a poster of preclinical data at the Oligonucleotide Therapeutics Society meeting. The preclinical efficacy data suggest that ALNY may have less potency than DRNA's candidate and trails by roughly a year in development, but ALNY could offer the advantage of subcutaneous dosing.

ALNY's Program Is Not A Complete Surprise – Still Too Early To Draw Definitive Comparisons. We first noted that ALNY had provided siRNAs against HAO1 (also known as GO1) to investigators in June when we attended a PH meeting. However, at the time, these investigators were only able to see reductions in urinary oxalate of 27%. ALNY's own scientists have been able to use its GalNAc-siRNA conjugate to lower urinary oxalate to a more competitive level. The ALNY poster showed >50% urinary oxalate reductions for the 1mg/kg dose through IV at Day 15 in a mouse knockout model of PH1. However, the poster was unclear if this was with multiple doses or a single dose. By comparison, multiple doses of DRNA's DCR-PH1 at 0.3mg/kg in a similar model reduced urinary oxalate by >70%. Additionally, knockdown of the HAO1 gene using a single 10mg/kg dose of the ALNY's subcutaneous formulation was ~80%, whereas a 1mg/kg dose of DCR-PH1 was 97%. We note that ALNY will continue to develop and fine-tune their lead candidate, and as such, efficacy may improve.

DRNA Has First-Mover Advantage For Roughly A Year. ALNY announced that it would select its PH1 candidate by mid-2015 and file an IND in 2016. DRNA expects to be in the clinic in 2015 and have proof-of-concept data in 2H15.

ALNY's Subcutaneous Formulation Could Be A Differentiator. The current formulation of DCR-PH1 is intravenous due to the lipid delivery system. Thus, the subcutaneous formulation from ALNY would be a potential differentiating feature. This could potentially be partially mitigated with a longer duration of response for DCR-PH1 and less frequent dosing. We believe DRNA is targeting once-monthly IV dosing. It is difficult to extrapolate the data from the ALNY poster in mice and rats to a human dosing schedule. Markets such as rheumatoid arthritis show that there can still be a substantial market for a long-duration IV drug even with subcutaneous competitors. On its poster at OTS, DRNA showed that its 0.3mg/kg dose had HAO1 knockdown of >50% after 30 days from a single dose. Additionally, DRNA believes that it could eventually develop a subcutaneous formulation of DCR-PH1 using the same GalNAc technology as ALNY.

Thomas Wei *
Equity Analyst
(212) 284-2326 twei@jefferies.com
Timothy Chou *
Equity Associate
(212) 284-2571 tchou@jefferies.com
Rebecca Forest *
Equity Associate
(212) 284-2170 rforest@jefferies.com
Shaunak Deepak *
Equity Analyst
(212) 284-2020 sdeepak@jefferies.com
* Jefferies LLC

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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Thomas Wei, Equity Analyst, (212) 284-2326, twei@jefferies.com

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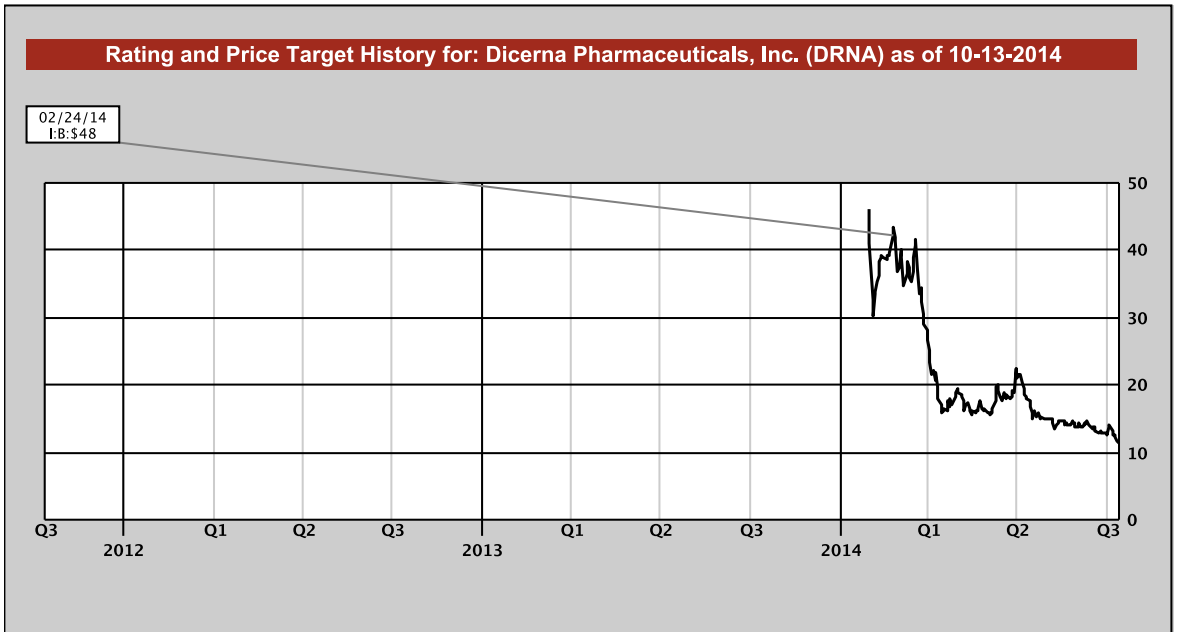

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Thomas Wei, Equity Analyst, (212) 284-2326, twei@jefferies.com

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Thomas Wei, Equity Analyst, (212) 284-2326, twei@jefferies.com

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Thomas Wei, Equity Analyst, (212) 284-2326, twei@jefferies.com