FLASH NOTE

Company Update

USA | Healthcare | Biotechnology

April 16, 2014

Jefferies

Dicerna Pharmaceuticals (DRNA)
Our Thoughts On Recent Developments In RNAi

BUY
Price target $48.00
Price $16.30

EQUITY RESEARCH AMERICAS

Key Takeaway

The recent Novartis announcement of withdrawal in the RNAi space does not affect our enthusiasm around RNAi. We remain positive on DRNA and expect topline DCR-MYC Phase 1 results in 2015, following today's announcement regarding initiation of that study. Phase 1 studies for DCR-PH1 for the treatment of primary hyperoxaluria will start in 1H15. We expect critical human validation of both the company's technology and lead programs in 2H15.

Novartis Downsizing Of RNAi Efforts Has No Implications For DRNA. Novartis (NOVN VX, CHF74.35, Hold) recently announced it is significantly reducing its internal RNAi drug discovery efforts, citing both delivery challenges and a limited number of clinically relevant targets where siRNA could be effective as a treatment. This news does not affect our positive stance on RNAi, as we note that Novartis had only 26 employees focused on RNAi, Novartis had already decided not to exercise its option to extend the collaboration back in 2010, and while Novartis (NVS) had access to Alnylam's (ALNY, $54.61, NR) delivery (both LNP and GalNAc), we believe that Novartis was primarily targeting oncogenes whereas ALNY has optimized delivery for liver-expressed targets. Insufficient target gene knockdown due to non-optimized delivery may have been the reason behind NVS' claim of a limited number of viable targets, as well as NVS' decision to pursue oncology, where often single target inhibition is insufficient to stop tumor growth.

Initiation Of DCR-MYC Phase 1 Trial; Data Expected In 2015. Today, DRNA announced the initiation of the first of two Phase 1 studies of DCR-MYC. Myc is an oncogene responsible for amplifying tumor growth signals. Dicerna plans to study DCR-MYC in a variety of tumor types with the initial focus on hepatocellular carcinoma (HCC). The newly initiated Phase 1 trial is a multi-center, dose-escalating study to assess the safety and tolerability of DCR-MYC in patients with solid tumors, multiple myeloma, or lymphoma who are refractory or unresponsive to standard therapies. DCR-MYC will be given by one hour intravenous infusion, once weekly for three weeks followed by a rest week. The trial is intended to identify the maximum tolerated dose (MTD) and pharmacokinetic profile, potential pharmacodynamic effects, and antitumor activity of DCR-MYC. The second Phase 1 study will be in patients with advanced HCC and is expected to begin in 2H14 and will have two parts as well, MDT and an expansion cohort. We expect topline Phase 1 data in 2015. We view the DCR-MYC program as high-risk, high reward, primarily related to the difficulty in translating tumor biology into clinical efficacy in patients.

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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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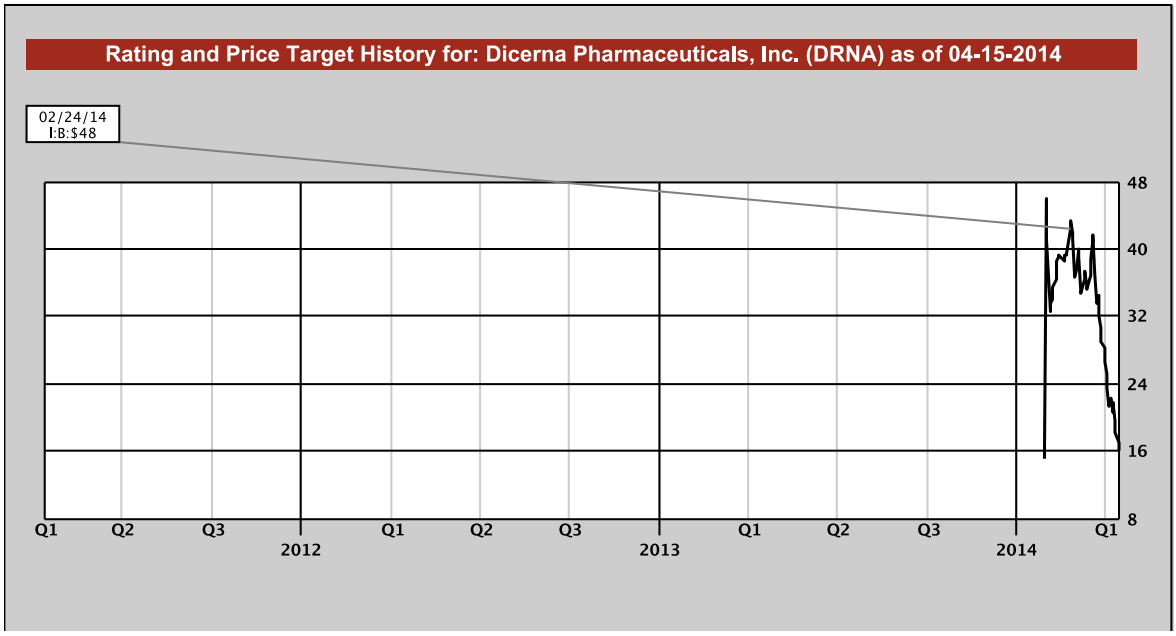
Other Companies Mentioned in This Report

- Novartis AG (NOVN VX: CHF74.35, HOLD)



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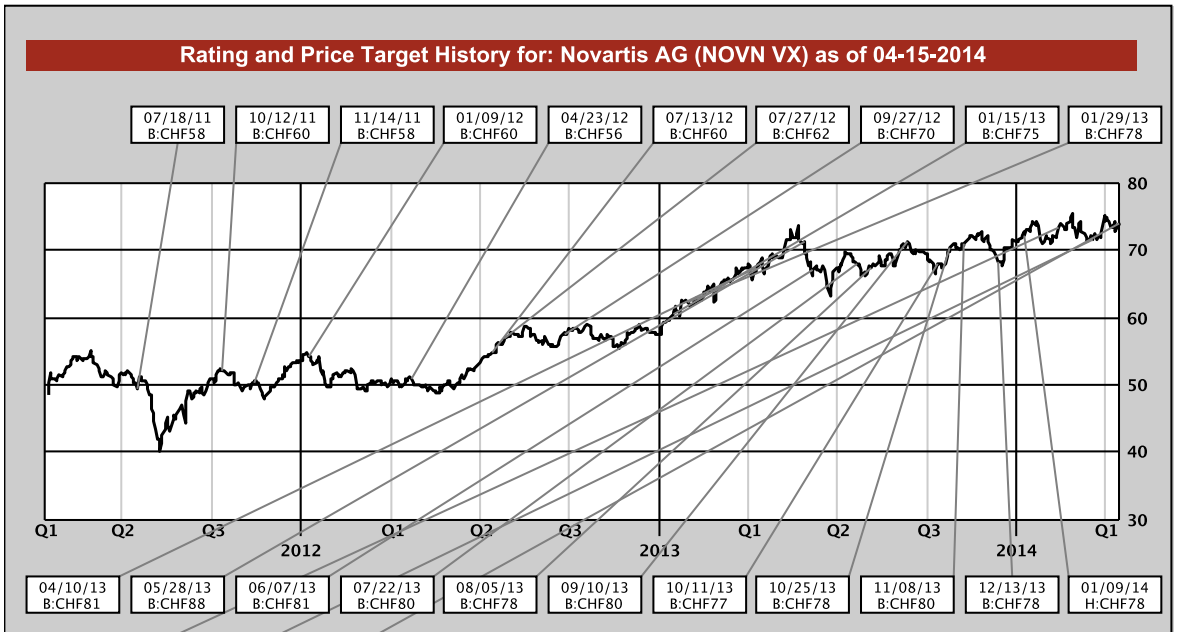

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