FLASH NOTE

Company Update

USA | Healthcare | Biotechnology

January 12, 2015

Jefferies

Ultragenyx (RARE)

Positive Update for SA-ER; Highlights Expansion Potential for THN and KRN23

BUY
Price target $74.00
Price $54.06

EQUITY RESEARCH AMERICAS

Key Takeaway

We view agreement with the EMA to conditionally file SA-ER for HIBM on existing phase 1/2 data as a vote of confidence in these data where expectations have been low. Highlighting the broad applicability of its two key value drivers, RARE recently announced it would be evaluating KRN23 for the treatment of TIO and THN for the treatment of HD. We believe positive KRN23 and THN data in 2015 from their ongoing trials could meaningfully impact share price

Filing SA-ER for conditional approval in Europe. While we believe SA-ER has a high probability of approval for Hereditary Inclusion Body myopathy (HIBM), the Street has been less positive on the drug's potential. We view today's announcement as validation of the program and data generated thus far. We believe the EMA should approve SA-ER based on the primary endpoint of upper extremity composite strength supported by secondary endpoints including those for lower extremity measures. We expect the planned phase 3 in 2H15 to confirm this and support FDA approval. RARE has already identified 1,400 patients globally suggesting that this patient population could be larger than previously estimated (~1,200-2,000). We currently project WW adjusted sales of ~$65M.

Early stage Triheptanoin (THN) data in Huntington's disease (HD) patients is promising, and could represent significant upside to our current estimates. RARE recently licensed intellectual property for HD treatment with THN. Similar to the Glut1DS and LC-FAOD indications currently being studied in phase 2, THN could improve energy deficiency in HD. Recent evidence suggests that mitochondrial dysfunction, which results in an energy deficiency within HD patients' brains, is an important feature of the disease and contributes to loss of neural function. Data from 10 HD patients suggested an improvement in brain energy metabolism and the UHDRS motor score. RARE is supporting an investigator study at ICM that will include a control arm and enroll ~100 patients. Preliminary phase 2 data for THN in Glut1DS/LC-FAOD (~$690M peak WW adjusted sales) is expected Mid/3Q15.

Expands KRN23 development into Tumor induced osteomalacia (TIO). We believe that KRN23 is ideally suited to treat TIO as FGF23 overproduction is a common feature of both XLH (where phase 2 data has been compelling) and TIO (where excess FGF23 is produced by benign tumors). RARE is planning to initiate a phase 2 study enrolling 6 adult TIO patients, with a similar design to the previous phase 1/2 trial in adult XLH patients (16 week dose titration followed by 28 week treatment period) to determine the appropriate dose and dosing regimen. Interim data is expected by YE15. We project peak WW KRN23 adjusted revenues to RARE of ~$295M in XLH, with the TIO opportunity representing 5-10% upside to this estimate. We estimate there are ~250-500 TIO patients in the US with non-resectable tumors.

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Company Description

Ultragenyx Pharmaceutical, Inc. is a clinical-stage biotechnology company. The company is focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. Ultragenyx Pharmaceutical was founded by Emil D. Kakkis on April 22, 2010 and is headquartered in Novato, CA.

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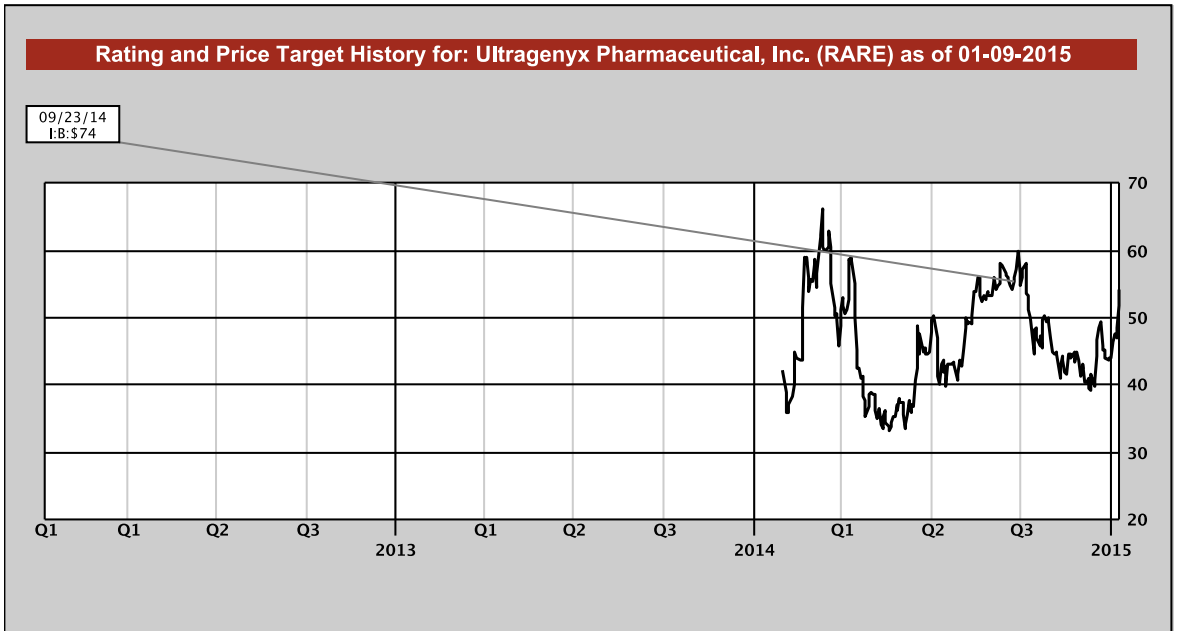

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