Ultragenyx

Quick Update from Inside The J.P. Morgan Healthcare Conference - ALERT

Overweight

RARE, RARE US
Price: $55.81

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RARE's breakout session is wrapping up at the 2015 J.P. Morgan Healthcare Conference, and we wanted to pass along a few brief takeaways. CEO Emil Kakkis presented (to a well-attended room and breakout) with an upbeat tone on RARE's efficient business model focused on in-licensing assets for rare genetic diseases with clear biology. The company's five clinical programs are rapidly advancing with momentum established in 2014 setting the stage for continued progress in 2015. Importantly, RARE is transitioning to a commercial company planning for 1st named-pt sales this year for rhGUS and SA-ER (pursuing conditional approval in the EU with an earlier-than-expected filing in 2H15). Below we provide further details.

- **KRN23 in XLH.** RARE plans to release 16-week data (phosphate control and safety) from the pediatric study in 1H15 with 40-week data on bone/growth expected in late 2015/early 2016. With the biology the same for adults and peds (change phosphate, change the disease), RARE exuded confidence in a potential benefit seen in XLH pediatric pts. The company is in discussions with the FDA on the Ph2b trial in adult XLH pts.

- **rhGUS in MPS7.** RARE identified 100 MPS7 pts to date (out of an estimated prevalence of ~200 WW). Of note, for the Ph3 study, the FDA will look at data on a case-by-case basis (vs. a declared endpoint) and the EMA will look at urinary GAG as the primary efficacy endpoint.

- **Trihep in LC-FAOD & Glut1 DS**. The Ph2 trial in LC-FAOD is fully enrolled with data expected in 2H15; the Ph2 Glut1 DS trial is still enrolling with data in 2H15. On the recently announced license agreement to support an IST in Huntington's disease, RARE noted that after one month of treatment in 10 pts, trihep showed near normal energy activation with a 30% reduction in metabolism scores.

- **SA-ER in HIBM**. The company has identified 1,400 pts which could suggest more than original estimated prevalence of ~2,000 WW. Most notably, the EMA has shown more flexibility on endpoints leading to potential conditional approval in the EU with an earlier-than-expected MAA filing in 2H15 and prep for commercialization. More specifically, the EMA was comfortable with the positive Ph2 data reported that showed upper extremity stabilization. RARE will pursue this faster path to market while conducting the Ph3 trial.

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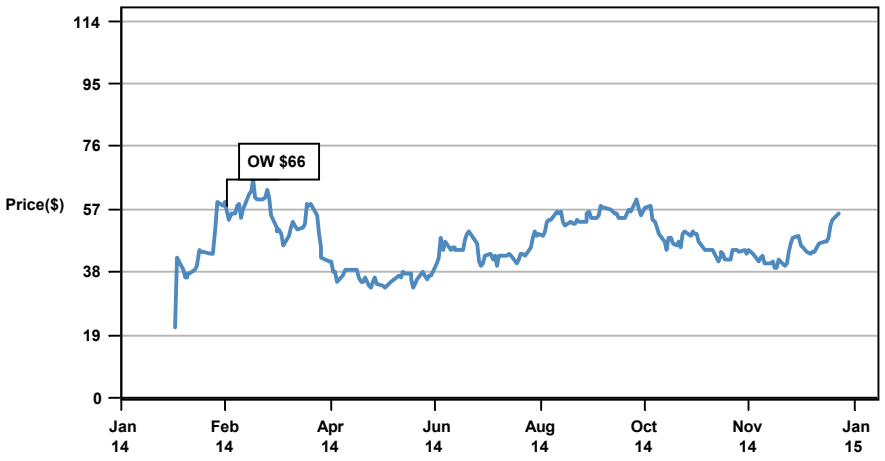
Ultragenyx (RARE, RARE US) Price Chart



Date	Rating	Share Price ($)	Price Target ($)
25-Feb-14	OW	58.01	66.00

Source: Bloomberg and J.P. Morgan; price data adjusted for stock splits and dividends.
Initiated coverage Feb 25, 2014.

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