

Hyperion Therapeutics, Inc. (HPTX)

UCD Foundation Says Ravicti is a Life Changer

MARKET DATA

Price	\$24.30
52-Week Range:	\$9.95 - \$26.50
Shares Out. (M):	20.3
Market Cap (\$M):	\$493.3
Average Daily Vol. (000):	117.0
Cash (M):	\$50
LT Debt (M):	\$8

Source: Thomson Reuters and JMP Securities LLC

MARKET OUTPERFORM | Price: \$24.30 | Target Price: \$30.00

INVESTMENT HIGHLIGHTS

UCD Foundation says Ravicti is a life changer; reiterate Market Outperform rating and \$30 price target on Hyperion Therapeutics based on a risk-adjusted, discounted cash flow analysis. On Monday, in conjunction with our initiation of coverage of Hyperion Therapeutics, we hosted a call with Cynthia LeMons, the Executive Director of the UCD foundation - an organization that spans 41 countries and consists of families with UCD and specialist physicians. Our key takeaways are: 1) diagnoses should increase in the coming 1-3 years with expanded newborn screening, 2) switching to Ravicti from Buphenyl is life altering, and 3) Ravicti may bring more patients into treatment not only due to greatly improved tolerability, but also because of increased patient access, which was missing for Buphenyl. Our discussion with Ms. LeMons underscores the eagerness of the community and we model about 40% of the 500 patients on Buphenyl switching to Ravicti in 2013 and 70% in 2014.

Increasing diagnosis expected. Top-line data from a recent study show that over a 20 year period, 2,020 babies were born in the U.S. with UCD. Historically, it has been difficult to track the prevalence of the disease due to mis-diagnosis (such as epilepsy, other metabolic disorders, etc.) and newborn screening for only three of six enzymes that can be missing in UCD. The most prevalent, OTC, is not screened for; however, the technology is now in place and screening should be approved and implemented in the next 1-3 years, which we believe will aid in the appropriate diagnosis and treatment of more patients, increasing the patient pool, which would provide upside to our current model. Recall that up to 20% of SIDS (sudden infant death syndrome) cases are estimated to be undiagnosed UCD.

Ravicti a life changer. About 500-550 UCD patients in the U.S. use Buphenyl - a drug saddled with side effects, a high pill burden, and high salt content. Because of this, parents have negotiate with children to take the drug or hide it in food, leading to behavioral problems and children who refuse to eat. Children are taking Ravicti willingly, and anecdotally, children on the drug have started to perform better in school and act more like "normal kids". Awareness is high and patients want the drug. Ms. LeMons believes that at least 75% of all patients will switch to Ravicti.

Ravicti expensive, but accessible. Hyperion has met with many payors and Ms. LeMons believes no patients have been denied access so far, though we are only several weeks into the launch. Co-pay support and free drug programs run by Hyperion are new for UCD patients - Buphenyl did not have comprehensive patient support. Ms. LeMons also pointed out the connection between increased therapy compliance and the decrease in healthcare costs, such as fewer hospitalizations due to crisis. We believe the orphan nature of the disease and Hyperion's commitment to patient access should not make cost a gating factor in drug penetration.

FY DEC	2012A	2013E	2014E
Revenue (\$M) 1Q	\$0.0	\$0.3	--
2Q	\$0.0	\$1.9	--
3Q	\$0.0	\$8.1	--
4Q	\$0.0	\$11.8	--
FY	\$0.0	\$22.1	\$78.0
EPS 1Q	(\$25.16)	(\$0.44)	--
2Q	(\$15.26)	\$0.07	--
3Q	(\$0.44)	(\$0.05)	--
4Q	(\$0.50)	\$0.11	--
FY	(\$4.45)	(\$0.31)	\$0.96

Source: Company reports and JMP Securities LLC

STOCK PRICE PERFORMANCE



Company Description

Hyperion Therapeutics is a San Francisco-based biopharmaceutical company focused on the development of Ravicti, a delayed release formulation of buphenyl, an ammonia scavenger approved for use in UCD with the potential to be used in other diseases characterized by ammonia toxicity, notably HE.

Investment Risks

Clinical risk. Ravicti is currently being evaluated for use in children under age 2. Should a safety issue arise, ramifications could include approval extension into this age group and potentially for the broader age group. Ravicti could underperform in forthcoming clinical studies in hepatic encephalopathy (HE) or safety issues could arise.

Regulatory risk. The FDA is currently revising endpoints for HE. We expect the process to wrap up by year end, but it is conceivable that it could take longer, which would push back the start of pivotal studies for Ravicti in this indication. Hyperion has indicated that it will pursue a special protocol assessment (SPA) in advance of starting its pivotal program and it is possible that the FDA may disagree with development plans for Ravicti in HE.

Intellectual Property risk. The composition of matter patent for Ravicti expires in 2015, with market exclusivity extended to 2018 with Hatch Waxman. Hyperion may or may not receive orphan exclusivity, which, if granted, would extend the exclusivity period until 2020. Hyperion recently received notice that new patents will be allowed which cover instructions on how to monitor and adjust dosing, which management believes will extend protection until 2028. It is possible that a generic competitor could attempt a work around to these patents, which could put Ravicti revenue at risk, as early as 2018.

Commercial risk. As a small company, Hyperion may not be able to maximize the marketplace and bring non-treated UCD patients on board. Insurers may provide more push-back than anticipated in allowing patients to switch from Buphenyl to Ravicti. Patients may chose other alternatives such as generic sodium phenylbuterate powder or a tasteless formulation currently moving through the European regulatory process. Newer technological breakthroughs may occur rendering Hyperion's compound obsolete. Hyperion may have a harder time gaining traction in HE given the dominance of lactulose and Salix's Xifaxan in the marketplace.

Sector risk. Valuation of biopharmaceutical stocks is subject to both investors' assessments of the prospects of the underlying companies, as well as investor tolerance for risk and confidence in the prospects of pharmaceutical stocks as a group. Therefore, Hyperion's stock price may fall even while the company meets or exceeds investor expectations.

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JMP Securities currently makes a market in the security of Hyperion Therapeutics, Inc.

JMP Securities was manager or co-manager of a public offering for Hyperion Therapeutics, Inc. in the past 12 months.

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Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

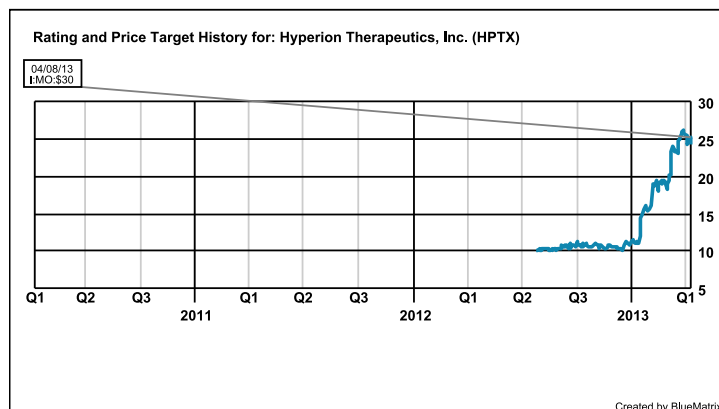
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

JMP Securities Research Ratings and Investment Banking Services: (as of April 9, 2013)

JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	213	59.33%	Buy	213	59.33%	65	30.52%
MARKET PERFORM	Hold	140	39.00%	Hold	140	39.00%	15	10.71%
MARKET UNDERPERFORM	Sell	6	1.67%	Sell	6	1.67%	0	0%
TOTAL:		359	100%		359	100%	80	22.28%

Stock Price Chart of Rating and Target Price Changes:

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with NASD Rule 2711, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.



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