

Joseph P. Schwartz
(617) 918-4575
Joseph.Schwartz@Leerink.com

Michael Schmidt, Ph.D.
(617) 918-4588
Michael.Schmidt@Leerink.com

Reason for report:

COMPANY UPDATE



LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

HYPERION THERAPEUTICS, INC.

3-Month Delay for Ravicti PDUFA Not That Surprising, May Actually Be Positive

• **Bottom Line:** Last night after the close HPTX announced that it has been informed by the FDA that the Ravicti PDUFA will be pushed out three months to January 23, 2013, in order to allow the FDA to conduct a full review of the NDA. We are not that surprised considering that such delays are common at the FDA, and HPTX submitted substantial (positive) pediatric safety data late in the review cycle. **Reiterate Outperform and \$18 fair value estimate in 12 months.**

• **We believe that most investors who heard the story on the recent IPO road show are cognizant that the additional data could be considered by the FDA to be a major amendment to the application.** Furthermore, this short delay should not significantly impact the company's commercialization plans since HPTX has not been planning to launch Ravicti in urea cycle disorder (UCD) before 1Q:13 anyway.

• **There may be positive read-through from FDA's willingness to consider additional data, since it could signify an expanded label for Ravicti being used in children as well as adults.** Recall that HPTX submitted an NDA for Ravicti for the chronic management of UCD patients aged 6 years and above on December 23, 2011. In the original NDA package, HPTX included data from Phase II and Phase III trials in patients aged 6 years and above as well as safety data from 69 UCD patients with 12 months of treatment on Ravicti and the results of two nonclinical carcinogenicity studies.

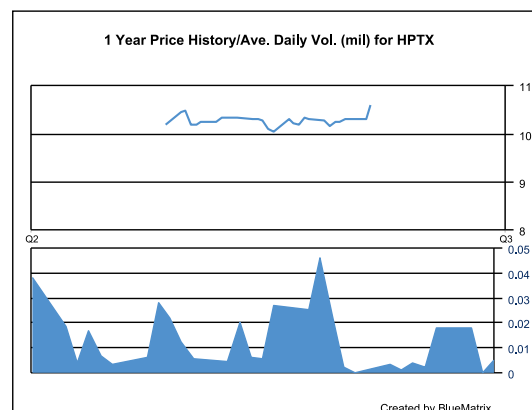
• **As part of an April 2012 update to the FDA, HPTX submitted a revised draft package insert requesting approval of Ravicti for UCD patients down to 29 days of age.** HPTX is currently in the process of conducting a clinical trial in UCD patients aged 29 days through 5 years. The efficacy portion of this trial is complete and was submitted to the FDA in April 2012. Data from the 12-month safety extension portion of the age 29 days – 5 years study will not be available until the second quarter of 2013.

• **Management noted that the recently announced acquisition of MRX (owner of Ucylyd subsidiary) by VRX does not impact HPTX's option to purchase rights to Buphenyl and Ammonul.** Recall that HPTX entered into an option agreement with Ucylyd/Medicis to purchase worldwide rights to Buphenyl and Ammonul for \$22MM, which may be funded by drawing on a loan commitment from Ucylyd. Option period runs from January 1, 2013, to June 30, 2013. Ucylyd has the right to retain Ammonul for \$32MM. If the Ravicti NDA for UCD is not approved by January 1, 2013, then Ucylyd is obligated to make monthly payments of \$0.5MM to HPTX until FDA approval of Ravicti or June 30, 2013.

Key Stats:

(NASDAQ:HPTX)

S&P 600 Health Care Index:	863.42
Price:	\$10.60
52 Week High:	\$11.99
52 Week Low:	\$9.95
Shares Outstanding (mil):	16.6
Market Capitalization (mil):	\$176.0
Book Value/Share:	\$0.00
Cash Per Share:	\$3.16
Dividend (ann):	\$0.00
Dividend Yield:	0.0%
Valuation:	\$18 on DCF analysis



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2011A	0.0	0.0	0.0	0.0	0.0	--	--	--	--	(\$62.68)	NM
2012E	0.0A	0.0	0.0	0.0	0.0	(\$25.33)A	(\$15.80)	(\$0.68)	(\$0.67)	(\$5.24)	NM
2013E	\$19.7	\$21.1	\$23.1	\$24.5	\$88.3	(\$1.13)	\$0.22	\$0.30	\$0.31	(\$0.30)	NM
2014E	--	--	--	--	\$126.1	--	--	--	--	\$2.90	3.7x

Source: Company Information and Leerink Swann LLC Research
Revenues in millions. HPTX completed an IPO on 7/31/12.



INVESTMENT THESIS

We believe that HPTX shares present an attractive opportunity to invest in the orphan drug business model and rate the stock Outperform. Lead agent Ravicti is in late-stage development for urea cycle disorders (UCD) and hepatic encephalopathy (HE), two rare diseases characterized by elevated levels of ammonia in the bloodstream, which can cause significant neurological complications. HPTX generated positive Phase III data for Ravicti in adult urea cycle disorder patients pursuant to a special protocol assessment (SPA) and has a PDUFA date of 1/23/13. HPTX has also completed Phase II trials for Ravicti in HE with an end-of-Phase II meeting planned in 4Q:12. Ravicti has a similar mechanism of action to MRX's FDA-approved Buphenyl, the use of which is constrained by a large dose burden, frequent (3-6 times/day) administration, unpleasant taste and smell, tolerability issues, and high sodium content. Easier patient compliance to Ravicti therapy may enable better disease management ultimately translating into fewer hyperammonemic (HA) crises relative to what is currently available with Buphenyl. The rate of HA crises with Ravicti was 40% lower than that seen for Buphenyl in the 12-month safety extension study following HPTX's pivotal Phase III trial. HPTX is led by seasoned orphan drug company executives who have stayed close to key physicians and patient support organizations who are expected to influence Ravicti uptake. HPTX expects to launch Ravicti in early 2013 with a field staff of 10 people and 10 individuals running back-office operations. We project that HPTX achieves breakeven by 2014 and generates peak sales around \$150MM in UCD in 2019. HPTX may influence the conversion and expansion of the UCD market since the company has the option to purchase worldwide rights to Buphenyl and Ammonul from MRX for \$22MM, which may be funded by drawing on a loan commitment from MRX. HE presents an upside market opportunity around \$500MM, in our estimation. In contrast to SLXP's Xifaxan, which blocks nitrogen absorption in the gut for HE patients, Ravicti lowers ammonia systemically by increasing its clearance. Ravicti could thus potentially be complementary to currently approved agents that limit the local production of ammonia. HPTX completed a Phase II clinical study of similar design to the pivotal trial used to evaluate Xifaxan, the only therapy approved by the FDA for episodic HE within the last 30 years. Phase II data indicates that Ravicti may have superior efficacy compared to Xifaxan and may improve outcomes when given in combination.

VALUATION

Our 12-month fair value estimate for HPTX is \$18/share based on discounted cash flow (DCF) analysis. We assume a 12% discount rate and 0% terminal growth rate. We discount free cash flow until early 2020, when orphan drug exclusivity for Ravicti for treating UCD expires.

RISKS TO VALUATION

The key risks to HPTX's valuation include the potential for disappointing clinical data, regulatory setbacks, and commercial and financial shortfalls. Since HPTX presently has only one late-stage product candidate, any of those possible setbacks may impact the stock significantly.

HPTX P&L (\$MM)	2010	2011	1Q12	2Q12E	3Q12E	4Q12E	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E
Revenue	-	-	-	-	-	-	-	19.7	21.1	23.1	24.5	88.3	126.1	147.0
COGS	-	-	-	-	-	-	-	3.0	3.2	3.5	3.7	13.2	18.9	22.1
R&D	23.1	17.2	8.9	3.0	3.5	4.0	19.4	5.0	5.0	5.0	5.0	20.0	25.2	29.4
SG&A	3.5	8.9	2.3	3.0	4.0	7.0	16.3	8.0	8.5	9.0	10.0	35.5	31.5	36.8
Operating expenses	26.6	26.2	11.2	6.0	7.5	11.0	35.7	16.0	16.7	17.5	18.7	68.7	75.6	88.2
Operating income	(26.6)	(26.2)	(11.2)	(6.0)	(7.5)	(11.0)	(35.7)	3.7	4.4	5.6	5.8	19.6	50.4	58.8
Interest income	0.0	0.0	0.0	0.0	0.1	0.1	0.2	0.0	0.0	0.0	0.0	0.1	0.9	1.4
Interest expense	(0.0)	(2.6)	(1.0)	(1.4)	(0.6)	(0.2)	(3.3)	(0.5)	(0.7)	(0.7)	(0.7)	(2.6)	(2.9)	(1.4)
Other income (expense)	1.1	(0.7)	0.4	-	-	-	0.4	(22.0)	-	-	-	(22.0)	-	-
EBT	(25.5)	(29.4)	(11.9)	(7.4)	(8.0)	(11.1)	(38.4)	(18.7)	3.7	4.9	5.1	(5.0)	48.5	58.8
Tax expense	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net income (loss)	(25.5)	(29.4)	(11.9)	(7.4)	(8.0)	(11.1)	(38.4)	(18.7)	3.7	4.9	5.1	(5.0)	48.5	58.8
Diluted EPS	(61.70)	(62.68)	(25.33)	(15.80)	(0.68)	(0.67)	(5.24)	(1.13)	0.22	0.30	0.31	(0.30)	2.90	3.50
Basic shares outstanding	0.4	0.5	0.5	0.5	11.8	16.6	7.3	16.6	16.6	16.6	16.6	16.6	16.7	16.8

Source: SEC filings and Leerink Swann Estimates



Disclosures Appendix

Analyst Certification

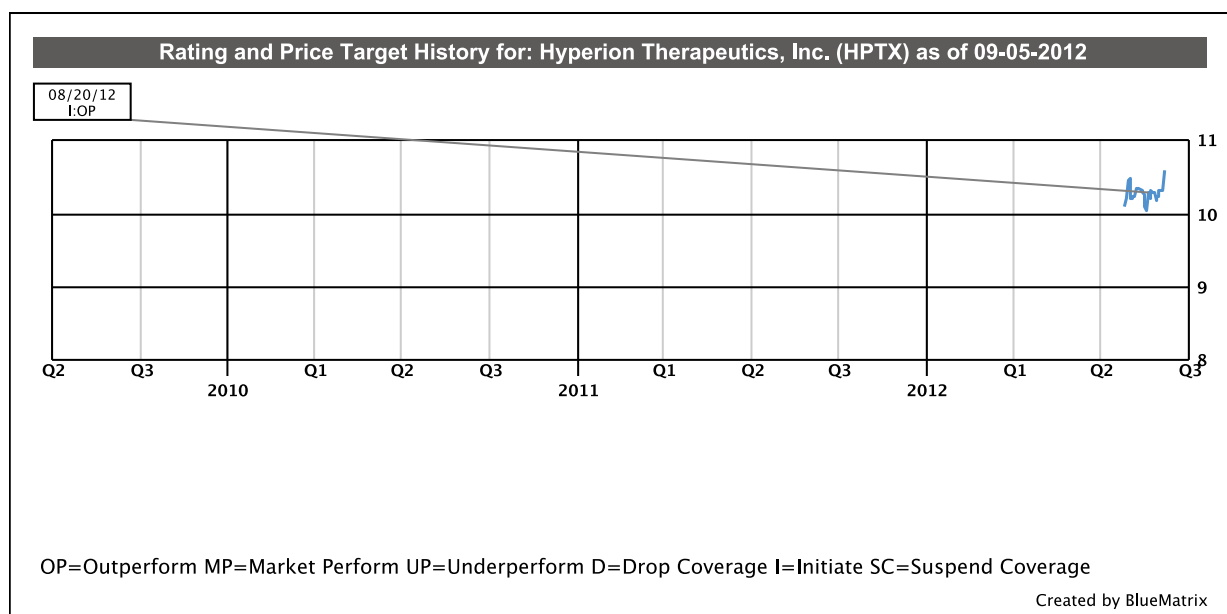
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

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Risks to Valuation

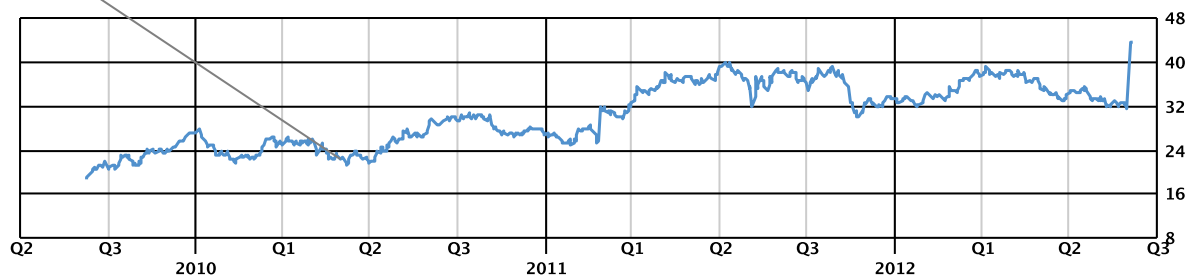
The key risks to HPTX's valuation include the potential for disappointing clinical data, regulatory setbacks, and commercial and financial shortfalls. Since HPTX presently has only one late-stage product candidate, any of those possible setbacks may impact the stock significantly.





Rating and Price Target History for: Medicis Pharmaceutical Corp. (MRX) as of 09-05-2012

06/01/10
SC



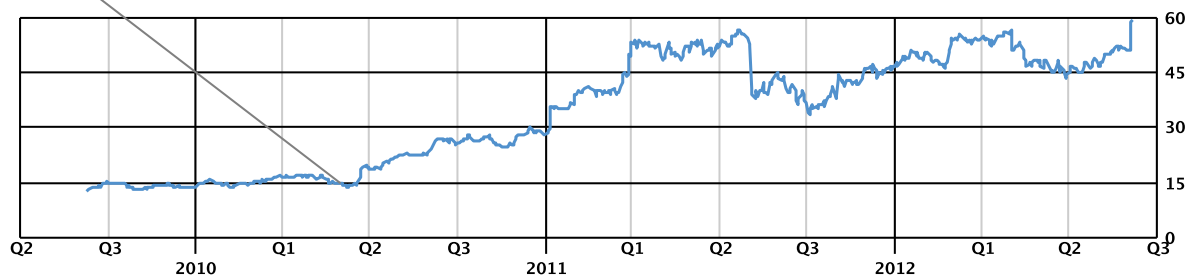
Leerink Swann placed a Market Perform rating on MRX on November 8, 2006.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

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Rating and Price Target History for: Valeant Pharmaceuticals (VRX) as of 09-05-2012

06/01/10
SC



OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix



Distribution of Ratings/Investment Banking Services (IB) as of 06/30/12				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	92	57.1	23	25.0
HOLD [MP]	69	42.9	4	5.8
SELL [UP]	0	0.0	0	0.0

Explanation of Ratings

Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark over the next 12 months. The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over \$2 billion.

From October 1, 2006 through January 8, 2009, the relevant benchmarks for the above definitions were the Russell 2000® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over \$2 billion.

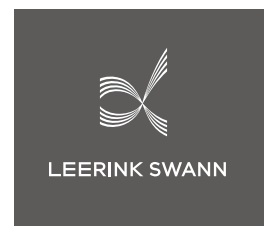
Definitions of Leerink Swann Ratings prior to October 1, 2006 are shown below:

Outperform (Buy): We expect this stock to outperform its benchmark by more than 10 percentage points over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform within a range of plus or minus 10 percentage points of its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark by more than 10 percentage points over the next 12 months.

For the purposes of these definitions, the relevant benchmark were the Russell 2000® Health Care Index for issuers with a market capitalization of less than \$2 billion and the S&P 500® Index for issuers with a market capitalization over \$2 billion.



Important Disclosures

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Leerink Swann Consulting LLC, an affiliate of Leerink Swann LLC, is a provider of evidence-based strategy and consulting to the healthcare industry.

In the past 12 months, the Firm has received compensation for providing investment banking services to Hyperion Therapeutics, Inc.

Leerink Swann LLC makes a market in Hyperion Therapeutics, Inc.

Leerink Swann LLC is willing to sell to, or buy from, clients the common stock of Medicis Pharmaceutical Corp. on a principal basis.

Leerink Swann LLC is willing to sell to, or buy from, clients the common stock of Valeant Pharmaceuticals on a principal basis.

Leerink Swann LLC has acted as the manager for a public offering of Hyperion Therapeutics, Inc. in the past 12 months.

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Leerink Swann LLC Equity Research

Director of Equity Research	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink.com
Associate Director of Research	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
Healthcare Strategy	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink.com
	Alice C. Avanian, CFA	(617) 918-4544	alice.avanian@leerink.com
Biotechnology	Howard Liang, Ph.D.	(617) 918-4857	howard.liang@leerink.com
	Joseph P. Schwartz	(617) 918-4575	joseph.schwartz@leerink.com
	Marko Kozul, M.D.	(415) 905-7221	marko.kozul@leerink.com
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink.com
	Irene Lau	(415) 905-7256	irene.lau@leerink.com
	Gena Wang, Ph.D.	(212) 277-6073	gena.wang@leerink.com
Life Science Tools & Diagnostics	Dan Leonard	(212) 277-6116	dan.leonard@leerink.com
	John L. Sullivan, CFA	(617) 918-4875	john.sullivan@leerink.com
Pharmaceuticals/Major	Seamus Fernandez	(617) 918-4011	seamus.fernandez@leerink.com
	Kathryn Alexander	(617) 918-4568	kathryn.alexander@leerink.com
	Swati Kumar	(617) 918-4576	swati.kumar@leerink.com
Specialty Pharmaceuticals, Generics	Jason M. Gerberry, JD	(617) 918-4549	jason.gerberry@leerink.com
Medical Supplies & Devices	Danielle Antalffy	(212) 277-6044	danielle.antalffy@leerink.com
	Richard Newitter	(212) 277-6088	richard.newitter@leerink.com
	Kathleen McGrath	(212) 277-6020	kathleen.mcgrath@leerink.com
Healthcare Services	Jason Gurda, CFA	(212) 277-6023	jason.gurda@leerink.com
	Michael Newshel, CFA	(212) 277-6049	michael.newshel@leerink.com
	George Villarina	(212) 277-6012	george.villarina@leerink.com
Healthcare Technology & Distribution	David Larsen, CFA	(617) 918-4502	david.larsen@leerink.com
	Christopher Abbott	(617) 918-4010	chris.abbott@leerink.com
Sr. Editor/Supervisory Analyst	Mary Ellen Eagan, CFA	(617) 918-4837	maryellen.eagan@leerink.com
Supervisory Analysts	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com

New York
1251 Avenue of Americas, 22nd Floor
New York, NY 10020
(888) 347-2342

Boston
One Federal Street, 37th Floor
Boston, MA 02110
(800) 808-7525

San Francisco
201 Spear Street, 16th Floor
San Francisco, CA 94105
(800) 778-1164