**OUTPERFORM** 

Reason for report: ESTIMATE CHANGE

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# HYPERION THERAPEUTICS, INC.

Ravicti Approved With favorable Label; New Patent Extends Life Cycle

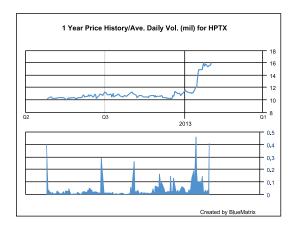
- Bottom Line: HPTX announced on Friday, 2/1, that the FDA approved Ravicti for the treatment of Urea Cycle Disorders (UCD) in patients two years of age and older. The also announced it received a notice of allowance from the US Patent & Trademark Office of a patent application, which HPTX expects to list in the Orange Book when issued. Issuance of this new patent, which expires in March 2032 and potential nearterm issuance of two additional patents covering methods of using Ravicti is a major upside surprise not reflected in our prior valuation. We are adjusting our model to reflect mgmt's comments on the Ravicti launch and are raising our fair value estimate to \$30 from \$18 previously. Reiterate OP.
- Ravicti approved for chronic management of UCD in children age > 2 years without REMS. We view the approved label as a positive, given FDA's prior concerns around potentially higher PAA exposure (the major metabolite of Ravicti) in pediatric patients. Recall, HPTX submitted a proposed REMS program with the NDA package which was intended to limit access to Ravicti only to patients aged 6 years and over until future label expansion. The approved label denotes only a few limitations of use including the lack of indication for the treatment of acute hyperammonemia, a statement that the safety and efficacy for the treatment of NAGS (N-Acetylglutamate Synthetase) deficiency has not been established and a contraindication for use in children less than two years of age.
- Ravicti commercial launch by late April 2013. HPTX has implemented a dedicated call center, which will serve as an integrated resource for Ravicti prescription, intake and distribution, reimbursement adjudication, patient financial support and ongoing compliance support. HPTX extended offers to the field sales team and account executives joining the company in mid-February and will target 80% of current Buphenyl prescribers. Ravicti's annual gross cost of therapy of \$250k-\$290k translates into an annual net revenue per patient of around \$205k-\$235k based on our estimates when factoring in discounts and Medicare rebates. Mgmt expects initially a 120-180 day coverage verification lag.
- Future label extension to treat HE remains a source of significant upside. HPTX is currently working on the final protocol including a validated endpoint measure and plans on obtaining special protocol assessment (SPA) for Phase III likely to be launched in 2014. Hepatic encephalopathy (HE) presents an upside market opportunity around \$500MM, in our estimation and we only account for a 35% sNDA approval probability in our current valuation which is conservative.

(NASDAQ:HPTX)

**Kev Stats:** 

HEALTHCARE EQUITY RESEARCH

S&P 600 Health Care Index: Price:	881.29 \$16.01
52 Week High:	\$16.48
52 Week Low:	\$9.95
Shares Outstanding (mil):	16.6
Market Capitalization (mil):	\$265.8
Book Value/Share:	\$0.00
Cash Per Share:	\$2.28
Dividend (ann):	\$0.00
Dividend Yield:	0.0%
Valuation:	\$30 from \$18 on DCF



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	0.0A	0.0A	0.0	0.0	(\$25.33)A	(\$15.26)A	(\$0.44)A	(\$0.32)	(\$4.05)	NM
2013E - New	0.0	0.0	\$11.5	\$15.3	\$26.7	(\$0.79)	(\$0.06)	(\$0.30)	(\$0.16)	(\$1.31)	NM
2013E - Old	\$19.7	\$21.1	\$23.1	\$24.5	\$88.3	(\$1.13)	\$0.23	\$0.30	\$0.31	(\$0.29)	NM
2014E - New					\$99.1	j				\$1.36	11.8x
2014E - Old					\$126.1					\$2.87	NM

Source: Company Information and Leerink Swann LLC Research Revenues in millions. Diluted EPS. 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 FDAapproved 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 2030. 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 of 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 indicate that Ravicti may have superior efficacy compared to Xifaxan and may improve outcomes when given in combination.

• Changes to model: We are adjusting our estimates to account for the launch timing in late April and also assuming a 6-month reimbursement window until the first Ravicti sales will be recorded. We are now assuming generic Ravicti launch in 2031 vs. our prior assumption of 2020. We are also changing our assumptions for execution of the Buphenyl license option and now assume VRX will retain rights to Ammunol.

### **VALUATION**

Our 12-month fair value estimate for HPTX is \$30/share based on discounted cash flow (DCF) analysis. We assume a 12% discount rate and 5% terminal growth rate. We discount free cash flow until 2030E, 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	2Q12	3Q12	4Q12E	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E
Revenue	- 1	-	-	-	-	-	-	-	-	11.5	15.3	26.7	99.1	115.7
COGS	- 1	-	-	-	-	-	-	-	-	1.7	2.3	4.0	14.9	17.4
R&D	23.1	17.2	8.9	2.7	2.4	2.5	16.5	5.0	5.0	5.0	5.0	20.0	19.8	23.1
SG&A	3.5	8.9	2.3	2.0	2.4	2.5	9.2	8.0	8.5	9.0	10.0	35.5	39.1	41.0
Operating expenses	26.6	26.2	11.2	4.8	4.8	5.0	25.7	13.0	13.5	15.7	17.3	59.5	73.7	81.5
Operating income	(26.6)	(26.2)	(11.2)	(4.8)	(4.8)	(5.0)	(25.7)	(13.0)	(13.5)	(4.3)	(2.0)	(32.8)	25.3	34.2
Total other income (expense)	1.1	(3.3)	(0.7)	(2.4)	(0.2)	(0.2)	(3.5)	(0.2)	12.6	(0.7)	(0.7)	11.1	(2.6)	(1.0)
ЕВТ	(25.5)	(29.4)	(11.9)	(7.2)	(4.9)	(5.2)	(29.2)	(13.2)	(0.9)	(4.9)	(2.7)	(21.7)	22.8	33.2
Tax expense	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net income (loss)	(25.5)	(29.4)	(11.9)	(7.2)	(4.9)	(5.2)	(29.2)	(13.2)	(0.9)	(4.9)	(2.7)	(21.7)	22.8	33.2
Diluted EPS	(61.70)	(62.68)	(25.33)	(15.26)	(0.44)	(0.32)	(4.05)	(0.79)	(0.06)	(0.30)	(0.16)	(1.31)	1.36	1.98
Basic shares outstanding	0.4	0.5	0.5	0.5	11.3	16.6	7.2	16.6	16.6	16.6	16.6	16.6	16.7	16.8
HPTX BS	2010	2011	1Q12	2Q12	3Q12	4Q12E	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E
Cash	6.6	7.0	3.7	7.3	56.5	51.5	51.5	39.4	39.6	35.8	34.3	34.3	62.9	102.5
Debt	- 1	23.4	30.7	40.5	12.0	10.0	10.0	10.0	32.0	32.0	32.0	32.0	32.0	-
Convertible notes	-	23.4	30.7	31.2	-	-	-	-	-	-	-	-	-	-
Venture debt	- 1													
Heyelyd Joan		-	-	9.4	12.0	10.0	10.0	10.0	10.0	10.0	10.0	10.0	10.0	-
Ucyclyd Ioan	-	-	-	9.4 -	12.0 -	10.0	10.0	10.0	10.0 22.0	10.0 22.0	10.0 22.0	10.0 22.0	10.0 22.0	-
Осустуа тоатт	-	-	-	9.4 -	12.0	10.0	10.0	10.0						<u>-</u>
HPTX CFS	2010	2011	1Q12	9.4 - <b>2Q12</b>	12.0 - 3Q12	- 4Q12E	10.0 - <b>2012E</b>	1Q13E	22.0 2Q13E	22.0 <b>3Q13E</b>	22.0 4Q13E	22.0 2013E		2015E
	2010	2011	-	-		-	-	-	22.0	22.0	22.0	22.0	22.0	2015E 39.6
HPTX CFS			1Q12	2Q12	3Q12	- 4Q12E	- 2012E	1Q13E	22.0 2Q13E	22.0 <b>3Q13E</b>	22.0 4Q13E	22.0 2013E	22.0 2014E	
HPTX CFS Change in cash	(3.5)	0.4	1Q12 (3.3)	2Q12 3.6	3Q12 49.2	4Q12E (5.0)	2012E 44.5	1Q13E (12.1)	22.0 2Q13E 0.2	22.0 3Q13E (3.8)	22.0 4Q13E (1.5)	22.0 2013E (17.3)	22.0 2014E 28.6	39.6
HPTX CFS Change in cash Cash from operations	(3.5) (25.9)	0.4 (24.5)	1Q12 (3.3) (10.7)	2Q12 3.6 (5.8)	3Q12 49.2 (5.7)	4Q12E (5.0) (5.0)	2012E 44.5 (27.2)	1Q13E (12.1) (12.1)	22.0 2Q13E 0.2 0.2	3Q13E (3.8) (3.8)	22.0 4Q13E (1.5) (1.5)	22.0 2013E (17.3) (17.3)	22.0 2014E 28.6 28.6	39.6 39.6
HPTX CFS Change in cash Cash from operations Net Income	(3.5) (25.9) (25.5)	<b>0.4</b> (24.5) (29.4)	1Q12 (3.3) (10.7) (11.9)	2Q12 3.6 (5.8) (7.2)	3Q12 49.2 (5.7) (4.9)	4Q12E (5.0) (5.0) (5.2)	2012E 44.5 (27.2) (29.2)	1Q13E (12.1) (12.1) (13.2)	22.0  2Q13E  0.2  0.2  (0.9)	3Q13E (3.8) (3.8) (4.9)	4Q13E (1.5) (1.5) (2.7)	22.0 2013E (17.3) (17.3) (21.7)	22.0  2014E  28.6  28.6  22.8	<b>39.6</b> <b>39.6</b> 33.2
HPTX CFS Change in cash Cash from operations Net Income SOE	(3.5) (25.9) (25.5) 0.2	0.4 (24.5) (29.4) 0.3	1Q12 (3.3) (10.7) (11.9) 0.1	2Q12 3.6 (5.8) (7.2) 0.2	3Q12 49.2 (5.7) (4.9) 0.3	4Q12E (5.0) (5.0) (5.2)	2012E 44.5 (27.2) (29.2) 0.9	1Q13E (12.1) (12.1) (13.2)	22.0  2Q13E  0.2  0.2  (0.9)	3Q13E (3.8) (3.8) (4.9)	4Q13E (1.5) (1.5) (2.7)	22.0 2013E (17.3) (17.3) (21.7)	22.0  2014E  28.6  28.6  22.8	<b>39.6</b> <b>39.6</b> 33.2
HPTX CFS Change in cash Cash from operations Net Income SOE Other	(3.5) (25.9) (25.5) 0.2 (0.6)	0.4 (24.5) (29.4) 0.3 4.5	1Q12 (3.3) (10.7) (11.9) 0.1 1.1	2Q12 3.6 (5.8) (7.2) 0.2 1.1	3Q12 49.2 (5.7) (4.9) 0.3 (1.1)	4Q12E (5.0) (5.0) (5.2) 0.3	- 2012E 44.5 (27.2) (29.2) 0.9 1.1	1Q13E (12.1) (12.1) (13.2)	22.0  2Q13E  0.2  0.2  (0.9)	3Q13E (3.8) (3.8) (4.9)	4Q13E (1.5) (1.5) (2.7)	22.0 2013E (17.3) (17.3) (21.7)	22.0  2014E  28.6  28.6  22.8	<b>39.6</b> <b>39.6</b> 33.2
HPTX CFS Change in cash Cash from operations Net Income SOE Other Cash from investing	(3.5) (25.9) (25.5) 0.2 (0.6)	0.4 (24.5) (29.4) 0.3 4.5	1Q12 (3.3) (10.7) (11.9) 0.1 1.1 (0.1)	2Q12 3.6 (5.8) (7.2) 0.2 1.1 0.2	3Q12 49.2 (5.7) (4.9) 0.3 (1.1)	4Q12E (5.0) (5.0) (5.2) 0.3	2012E 44.5 (27.2) (29.2) 0.9 1.1 0.0	1Q13E (12.1) (12.1) (13.2)	22.0  2Q13E  0.2  0.2  (0.9)	3Q13E (3.8) (3.8) (4.9)	4Q13E (1.5) (1.5) (2.7)	22.0 2013E (17.3) (17.3) (21.7)	22.0  2014E  28.6  28.6  22.8	<b>39.6</b> <b>39.6</b> 33.2
HPTX CFS Change in cash Cash from operations Net Income SOE Other Cash from investing Option to purchase Buphenyl	(3.5) (25.9) (25.5) 0.2 (0.6) (0.0)	0.4 (24.5) (29.4) 0.3 4.5 (0.0)	1Q12 (3.3) (10.7) (11.9) 0.1 1.1 (0.1) (0.3)	2Q12 3.6 (5.8) (7.2) 0.2 1.1 0.2	3Q12 49.2 (5.7) (4.9) 0.3 (1.1) (0.0)	- 4Q12E (5.0) (5.0) (5.2) 0.3	2012E 44.5 (27.2) (29.2) 0.9 1.1 0.0 (0.3)	1Q13E (12.1) (12.1) (13.2)	22.0  2Q13E  0.2  0.2  (0.9)	3Q13E (3.8) (3.8) (4.9)	22.0 4Q13E (1.5) (1.5) (2.7) 1.2 - -	22.0 2013E (17.3) (17.3) (21.7)	22.0  2014E  28.6  28.6  22.8	<b>39.6</b> <b>39.6</b> 33.2
HPTX CFS Change in cash Cash from operations Net Income SOE Other Cash from investing Option to purchase Buphenyl Other	(3.5) (25.9) (25.5) 0.2 (0.6) (0.0)	0.4 (24.5) (29.4) 0.3 4.5 (0.0)	1Q12 (3.3) (10.7) (11.9) 0.1 1.1 (0.1) (0.3) 0.2	2Q12 3.6 (5.8) (7.2) 0.2 1.1 0.2	3Q12 49.2 (5.7) (4.9) 0.3 (1.1) (0.0)	- (5.0) (5.0) (5.2) 0.3 - - -	- 2012E 44.5 (27.2) (29.2) 0.9 1.1 0.0 (0.3) 0.3	1Q13E (12.1) (12.1) (13.2)	22.0  2Q13E  0.2  0.2  (0.9)	3Q13E (3.8) (3.8) (4.9)	22.0 4Q13E (1.5) (1.5) (2.7) 1.2 - -	22.0 2013E (17.3) (17.3) (21.7)	22.0  2014E  28.6  28.6  22.8	<b>39.6</b> <b>39.6</b> 33.2
HPTX CFS Change in cash Cash from operations Net Income SOE Other Cash from investing Option to purchase Buphenyl Other Cash from financing	(3.5) (25.9) (25.5) 0.2 (0.6) (0.0) - (0.0) 22.4	0.4 (24.5) (29.4) 0.3 4.5 (0.0) - (0.0) 25.0	1Q12 (3.3) (10.7) (11.9) 0.1 1.1 (0.1) (0.3) 0.2 7.6	2Q12 3.6 (5.8) (7.2) 0.2 1.1 0.2 - 0.2 9.2	3Q12 49.2 (5.7) (4.9) 0.3 (1.1) (0.0) - (0.0) 55.0	- (5.0) (5.0) (5.2) 0.3 - - - -	2012E 44.5 (27.2) (29.2) 0.9 1.1 0.0 (0.3) 0.3 71.7	1Q13E (12.1) (12.1) (13.2)	22.0  2Q13E  0.2  0.2  (0.9)	3Q13E (3.8) (3.8) (4.9)	22.0 4Q13E (1.5) (1.5) (2.7) 1.2 - -	22.0 2013E (17.3) (17.3) (21.7)	22.0  2014E  28.6  28.6  22.8	<b>39.6</b> <b>39.6</b> 33.2

Source: SEC filings and Leerink Swann Estimates

UCD Scenario 1: Ravicti approved in UCD only	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Total US pts diagnosed	1,000	1,002	1,004	1,006	1,009	1,005	1,014	1,022	1,031	1,040	1,049	1,058	1,067
Total untreated pts	575	576	577	579	580	578	583	588	593	598	603	608	613
patients < age 6	161	161	162	162	162	162	163	165	166	167	169	170	172
patients age 6-17	184	184	185	185	186	185	187	188	190	191	193	195	196
adults	230	230	231	231	232	231	233	235	237	239	241	243	245
Total Buphenyl treated pts	425	426	427	428	429	427	431	435	438	442	446	450	453
patients < age 6	119	119	120	120	120	120	121	122	123	124	125	126	127
patients age 6-17	136	136	137	137	137	137	138	139	140	141	143	144	145
adults	170	170	171	171	171	171	172	174	175	177	178	180	181
Untreated pts on Ravicti	-	-	-	-	12	3	58	88	119	120	121	122	123
penetration, patients < age 6	0%	0%	0%	0%	2%	1%	10%	15%	20%	20%		20%	20%
penetration, patients age 6-17	0%	0%	0%	0%	2%	1%	10%	15%	20%	20%		20%	
penetration, adults	0%	0%	0%	0%	2%	1%	10%	15%	20%	20%	20%	20%	20%
Prior Buphenyl treated pts on Ravicti	-	-	-	21	43	16	302	391	438	442	446	450	453
penetration, patients < age 6	0%	0%	0%	5%	10%	4%	70%	90%	100%	100%	100%	100%	100%
penetration, patients age 6-17	0%	0%	0%	5%	10%	4%	70%	90%	100%	100%	100%	100%	100%
penetration, adults	0%	0%	0%	5%	10%	4%	70%	90%	100%	100%	100%	100%	100%
Total Ravicti pts	-	-	-	21	54	19	360	479	557	562	566	571	576
Avg cost/pt(\$mm)	-	0.058	0.058	0.058	0.058	0.230	0.230	0.230	0.230	0.230	0.230	0.230	0.230
Ravicti US sales in UCD (\$MM)	-	-	-	1	3	4	83	110	128	129	130	131	132
Buphenyl Model Private Payors	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Patients on Buphenyl	425	426	427	406	386	411	129	43	-	-	-	-	-
Branded Buphenyl market private payor	40%	40%	40%	40%	40%	40%	40%	40%	40%	10%	0%	0%	0%
Avg cost/pt	0.056	0.058	0.058	0.058	0.058	0.230	0.230	0.230	0.230	0.230	0.230	0.230	0.230
Buphenyl US sales in UCD (\$MM)	10	10	10	9	9	38	12	4	-	-	-	-	-
Buphenyl Model Medicare/Medicaid	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Patients on Buphenyl	425	426	427	406	386	411	129	43	-	-	-	-	-
Branded Buphenyl market public payor	60%	60%	60%	60%	60%	60%	60%	60%	60%	10%	0%	0%	0%
Avg cost/pt	0.056	0.014	0.014	0.014	0.014	0.056	0.056	0.056	0.056	0.056	0.056	0.056	0.056
Buphenyl US sales in UCD (\$MM)	14	4	4	3	3	14	4	1					
Tarris Barris and U.S. and a CAMAN		4-	40	4-	4-		1 4-	_					
Total Buphenyl US sales (\$MM)	24	13	13	13	12	52	16	5	-	-	-	-	-

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Source: SEC filings and Leerink Swann Estimates

Total sales Ravicti + Buphenyl

UCD Scenario 2: Ravicti approved in HE	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Total US UCD pts diagnosed	1,000	1,002	1,004	1,006	1,009	1,005	1,014	1,022	1,031	1,040	1,049	1,058	1,067
Total untreated pts	575	576	577	579	580	578	583	588	593	598	603	608	613
patients < age 6	161	161	162	162	162	162	163	165	166	167	169	170	172
patients age 6-17	184	184	185	185	186	185	187	188	190	191	193	195	196
adults	230	230	231	231	232	231	233	235	237	239	241	243	245
Total Buphenyl treated pts	425	426	427	428	429	427	431	435	438	442	446	450	453
patients < age 6	119	119	120	120	120	120	121	122	123	124	125	126	127
patients age 6-17	136	136	137	137	137	137	138	139	140	141	143	144	145
adults	170	170	171	171	171	171	172	174	175	177	178	180	181
Untreated pts on Ravicti	-	-	-	-	12	3	58	88	119	120	121	122	123
penetration, patients < age 6	0%	0%	0%	0%	2%	1%	10%	15%	20%	20%	20%	20%	20%
penetration, patients age 6-17	0%	0%	0%	0%	2%	1%	10%	15%	20%	20%	20%	20%	20%
penetration, adults	0%	0%	0%	0%	2%	1%	10%	15%	20%	20%	20%	20%	20%
Prior Buphenyl treated pts on Ravicti	-	-	-	21	43	16	302	391	438	442	446	450	453
penetration, patients < age 6	0%	0%	0%	5%	10%	4%	70%	90%	100%	100%	100%	100%	100%
penetration, patients age 6-17	0%	0%	0%	5%	10%	4%	70%	90%	100%	100%	100%	100%	100%
penetration, adults	0%	0%	0%	5%	10%	4%	70%	90%	100%	100%	100%	100%	100%
Total Ravicti pts	-	-	-	21	54	19	360	479	557	562	566	571	576
Avg cost/pt(\$mm)	-	0.058	0.058	0.058	0.058	0.230	0.230	0.230	0.230	0.070	0.070	0.070	0.070
Ravicti US sales in UCD (\$MM)	-	-	-	1	3	4	83	110	128	39	40	40	40
Buphenyl Model Private Payors	2012E	1Q13E	2Q13E	3Q13E	4Q13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Patients on Buphenyl	425	426	427	406	386	2013E 411	129	2015E 43	2010E -	2017E	2010E -	2019E	2020E -
					40%				400/	400/	00/	0%	00/
Branded Buphenyl market private payor	40%	40%	40%	40%		40%	40%	40%	40%	10%	0%		0%
Avg cost/pt	0.056	0.058	0.058	0.058	0.058	0.230	0.230	0.230	0.230	0.070	0.070	0.070	0.070
Buphenyl US sales in UCD (\$MM)	10	10	10	9	9	38	12	4	-	-	-	-	-
Buphenyl Model Medicare/Medicaid Patients on Buphenyl	<b>2012E</b> 425	1Q13E 426	<b>2Q13E</b> 427	<b>3Q13E</b> 406	<b>4Q13E</b> 386	<b>2013E</b> 411	<b>2014E</b> 129	<b>2015E</b> 43	2016E	2017E	2018E	2019E	2020E
				400		411			-	-	-	-	-
Branded Buphenyl market public payor	60%	600/											
Avg cost/pt		60%	60%	60%	60%	60%	60%	60%	60%	10%	0%	0%	0%
ring occupi	0.056	0.014	60% 0.014	60% 0.014	60% 0.014	60% 0.056	60% 0.056	0.056	0.056	0.056	0.056	0.056	0.056
Buphenyl US sales in UCD (\$MM)	0.056 <b>14</b>												
Buphenyl US sales in UCD (\$MM)	14	0.014 <b>4</b>	0.014 <b>4</b>	0.014 <b>3</b>	0.014 <b>3</b>	0.056 <b>14</b>	0.056 <b>4</b>	0.056 <b>1</b>					
		0.014	0.014	0.014	0.014	0.056	0.056	0.056					
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)	14	0.014	0.014 4 13	0.014 3 13	0.014 3	0.056 14	0.056	0.056	0.056	0.056	0.056	0.056	0.056
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE	14 24 2012E	0.014 4 13	0.014 4 13 2Q13E	0.014 3 13 3Q13E	0.014 3 12 4Q13E	0.056 14 52 2013E	0.056 4 16 2014E	0.056 1 5 2015E	0.056 - - 2016E	0.056 - - 2017E	0.056 - - 2018E	0.056 - - 2019E	0.056 - - 2020E
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)	14	0.014	0.014 4 13	0.014 3 13	0.014 3	0.056 14	0.056	0.056	0.056	0.056	0.056	0.056	0.056
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE  Total US HE pts diagnosed	24 2012E 140,000	0.014 4 13 1Q13E 140,297	0.014 4 13 2Q13E 140,594	0.014 3 13 3Q13E 140,892	0.014 3 12 4Q13E 141,190	0.056 14 52 2013E 140,743	0.056 4 16 2014E 141,939	0.056 1 5 2015E 143,146	0.056 - - 2016E 144,363	0.056 - - 2017E 145,590	0.056 - - - 2018E 146,827	0.056 - - - 2019E 148,075	0.056 - - 2020E 149,334
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE	14 24 2012E	0.014 4 13	0.014 4 13 2Q13E	0.014 3 13 3Q13E	0.014 3 12 4Q13E	0.056 14 52 2013E	0.056 4 16 2014E	0.056 1 5 2015E	0.056 - - 2016E	0.056 - - 2017E	0.056 - - 2018E	0.056 - - 2019E	0.056 - - 2020E
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE  Total US HE pts diagnosed	24 2012E 140,000	0.014 4 13 1Q13E 140,297	0.014 4 13 2Q13E 140,594	0.014 3 13 3Q13E 140,892	0.014 3 12 4Q13E 141,190	0.056 14 52 2013E 140,743	0.056 4 16 2014E 141,939	0.056 1 5 2015E 143,146	0.056 - - 2016E 144,363	0.056 - - 2017E 145,590	0.056 - - - 2018E 146,827	0.056 - - - 2019E 148,075	0.056 - - 2020E 149,334
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE  Total US HE pts diagnosed  Total severe HE patients	24 2012E 140,000 28,000	0.014 4 13 1Q13E 140,297 28,059	0.014 4 13 2Q13E 140,594 28,119	0.014 3 13 3Q13E 140,892 28,178	0.014 3 12 4Q13E 141,190 28,238	0.056 14 52 2013E 140,743 28,149	0.056 4 16 2014E 141,939 28,388	0.056 1 5 2015E 143,146 28,629	0.056 - - 2016E 144,363 28,873	0.056 - - 2017E 145,590 29,118	0.056 - - 2018E 146,827 29,365	0.056 - - 2019E 148,075 29,615	0.056 - - - 2020E 149,334 29,867
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE  Total US HE pts diagnosed  Total severe HE patients % severe HE patients	24 2012E 140,000 28,000	0.014 4 13 1Q13E 140,297 28,059	0.014 4 13 2Q13E 140,594 28,119	0.014 3 13 3Q13E 140,892 28,178	0.014 3 12 4Q13E 141,190 28,238	0.056 14 52 2013E 140,743 28,149	0.056 4 16 2014E 141,939 28,388	0.056 1 5 2015E 143,146 28,629	0.056 - - 2016E 144,363 28,873	0.056 - - 2017E 145,590 29,118	0.056 - - 2018E 146,827 29,365	0.056 - - 2019E 148,075 29,615	0.056 - - - 2020E 149,334 29,867
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE  Total US HE pts diagnosed  Total severe HE patients % severe HE patients % Ravicti patients	24 2012E 140,000 28,000 20%	0.014 4 13 1Q13E 140,297 28,059 20%	0.014 4 13 2Q13E 140,594 28,119 20%	0.014 3 13 3Q13E 140,892 28,178 20%	0.014 3 12 4Q13E 141,190 28,238 20%	0.056 14 52 2013E 140,743 28,149 20%	0.056 4 16 2014E 141,939 28,388 20%	0.056 1 5 2015E 143,146 28,629 20%	0.056 - - 2016E 144,363 28,873 20%	0.056 - - 2017E 145,590 29,118 20%	0.056 - - 2018E 146,827 29,365 20%	0.056 - - 2019E 148,075 29,615 20%	0.056 - - 2020E 149,334 29,867 20%
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE  Total US HE pts diagnosed  Total severe HE patients % severe HE patients	24 2012E 140,000 28,000 20%	0.014 4 13 1Q13E 140,297 28,059 20%	0.014 4 13 2Q13E 140,594 28,119 20%	0.014 3 13 3Q13E 140,892 28,178 20%	0.014 3 12 4Q13E 141,190 28,238 20%	0.056 14 52 2013E 140,743 28,149 20%	0.056 4 16 2014E 141,939 28,388 20%	0.056 1 5 2015E 143,146 28,629 20%	0.056 - - 2016E 144,363 28,873 20%	0.056 - - 2017E 145,590 29,118 20%	0.056 - - 2018E 146,827 29,365 20%	0.056 - - 2019E 148,075 29,615 20%	0.056 - - 2020E 149,334 29,867 20%
Buphenyl US sales in UCD (\$MM)  Total Buphenyl US sales (\$MM)  Ravicti for HE  Total US HE pts diagnosed  Total severe HE patients % severe HE patients % Ravicti patients	24 2012E 140,000 28,000 20%	0.014 4 13 1Q13E 140,297 28,059 20%	0.014 4 13 2Q13E 140,594 28,119 20%	0.014 3 13 3Q13E 140,892 28,178 20%	0.014 3 12 4Q13E 141,190 28,238 20%	0.056 14 52 2013E 140,743 28,149 20%	0.056 4 16 2014E 141,939 28,388 20%	0.056 1 5 2015E 143,146 28,629 20%	0.056 - - 2016E 144,363 28,873 20%	0.056 - - 2017E 145,590 29,118 20%	0.056 - - 2018E 146,827 29,365 20%	0.056 - - 2019E 148,075 29,615 20%	0.056 - - 2020E 149,334 29,867 20%

Source: SEC filings and Leerink Swann Estimates

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Total sales Ravicti + Buphenyl

11 15 27 99 116 128 141 245 351 563

HPTX DCF (Scenario 1)	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	TV
FCF	(17)	29	40	52	38	40	40	41	50	50	51	51	52	52	52	53	53	54	11	
Discount periods	-	1.0	2.0	3.0	4.0	5.0	6.0	7.0	8.0	9.0	10.0	11.0	12.0	13.0	14.0	15.0	16.0	17.0	18.0	
NPV	(17)	26	32	37	24	23	20	18	20	18	16	15	13	12	11	10	9	8	1	21
Valuation	316																			

Discount Rate	12%
Terminal Growth	5%

Valuation	Valuation	per shr	Probability	P/W
Scenario 1, Ravicti approved for UCD only	316	\$ 16	65%	206
Scenario 2, Ravicti approved for UCD and HE	944	\$ 48	35%	330
Blended Valuation				536
Net cash				44.6
Diluted Shares Outstanding				19.5
Per share valuation		·		\$ 30

Source: SEC filings and Leerink Swann Estimates

Hyperion Thera	apeutics, li	nc. (HPTX) Expected Milestones	Timing
Ravicti	UCD	Data presentations at ACMC	Mar-13
Puphopul		Expiration Ammunol/Buphenyl	5/2/2013
Buphenyl		licensing option period	5/2/2015
Ammunol		Expiration MRX/VRX right to retain	5/22/2013
Ammunoi		Ammunol	5/22/2015
Ravicti		2 addl. patent allowances	2013
Ravicti	HE	Phase III initiation	2014
Ravicti	HE	Phase III data	2015
Ravicti	HE	sNDA filing	2016
Ravicti	HE	sNDA approval	2017

Source: Company reports, Leerink Swann LLC estimates



# **Disclosures Appendix Analyst Certification**

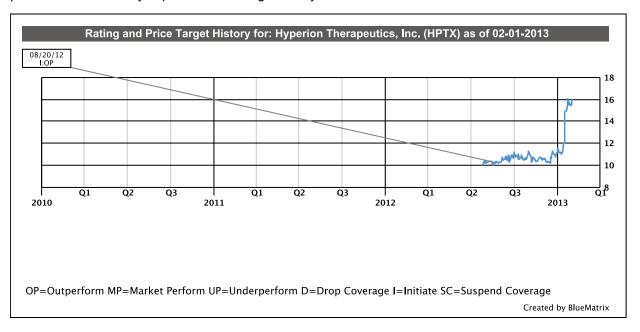
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

### **Valuation**

Our 12-month fair value estimate for HPTX is \$30/share based on discounted cash flow (DCF) analysis. We assume a 12% discount rate and 5% terminal growth rate. We discount free cash flow until 2030E, 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.





	Distribution of Ratings/Investment Bank	ing Services (IE	,	erv./Past 12 Mos.
Rating	Count	Percent	Count	Percent
BUY [OP]	105	61.76	32	30.48
HOLD [MP]	64	37.65	2	3.12
SELL [UP]	1	0.59	0	0.00

# **Explanation of Ratings**

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

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

<u>Underperform (Sell):</u> 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.

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

<u>Underperform (Sell):</u> 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 has acted as the manager for a public offering of Hyperion Therapeutics, Inc. in the past 12 months.

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