

# Hyperion Therapeutics

## Outperform (1)

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### Analysts

**Phil Nadeau, Ph.D.**

(646) 562-1336

phil.nadeau@cowen.com

**Nicholas Bishop, Ph.D.**

(646) 562-1378

nicholas.bishop@cowen.com

## Ravicti Approved, Allowed Patent Claims Should Extend IP To 2032

**Conclusion:** On Friday HPTX announced that the FDA approved Ravicti for the treatment of urea cycle disorders, and that the U.S. PTO allowed the claims of the 13/417,137 application. While Ravicti's label is broad and favorable, the approval was widely expected. However, the allowance of the claims was a surprise. We think the claims represent unexpected and novel scientific findings, and therefore our initial impression is that the patent has a good chance of withstanding challenge. Moreover, dosing language on Ravicti's label is closely aligned with the allowed claims, and therefore we believe that the patent should protect Ravicti from generic competition through its expiry in 2032. Today we are publishing a new DCF analysis which assumes Ravicti maintains exclusivity until the end of the patent, which adds nearly \$20/share to the DCF value of the UCD franchise. We believe that Hyperion is undervalued based on the potential of Ravicti in urea cycle disorders alone. We think investors should take advantage of the fact that the market is giving Hyperion no credit for additional years of exclusivity, and buy HPTX today.

■ **13/417,137 Claims Novel And Capable Of Protecting The Franchise.** While developing Ravicti, HPTX obtained novel data on the variability of blood ammonia (NH<sub>4</sub>) levels over 24 hrs. This led to the unexpected finding that a patient's fasting NH<sub>4</sub> level does not provide sufficient information to correctly dose an ammonia scavenger. Rather, the correct paradigm is one in which the dose is adjusted to produce a fasting plasma NH<sub>4</sub> levels less than half the upper limit of normal according to age. This '137 application claims this novel method, and Ravicti's label instructs physicians to use it to adjust Ravicti's dose. We suspect that HPTX will seek to modernize Buphenyl's label to include similar dosing language.

■ **We Expect HPTX To Announce Ravicti's Price On This Morning's Call.** We currently assume an average price of \$200K/patient/year. Our initial model adjustments are described on p. 2.

<b>HPTX (02/01)</b>	<b>\$16.01</b>	<b>Revenue \$MM</b>							
<b>Mkt cap</b>	<b>262.6MM</b>	<b>FY</b>	<b>2011</b>	<b>2012E</b>		<b>2013E</b>		<b>2014E</b>	<b>2015E</b>
Dil shares out	16.4MM	<b>Dec</b>	<b>Actual</b>	<b>Prior</b>	<b>Current</b>	<b>Prior</b>	<b>Current</b>	<b>Current</b>	<b>Current</b>
Avg daily vol	410.6K	Q1	—	—	0.0A	—	0.0	—	—
52-wk range	\$10.0-16.5	Q2	—	—	0.0A	15.1	1.8	—	—
Dividend	Nil	Q3	—	—	0.0A	15.8	14.5	—	—
Dividend yield	Nil	Q4	—	—	0.0	18.9	17.5	—	—
BV/sh	\$2.57	Year	<b>0.0</b>	—	<b>0.0</b>	<b>49.8</b>	<b>33.8</b>	<b>95.0</b>	<b>105.0</b>
Net cash/sh	\$2.90	EV/S	—	—	—	—	6.3x	2.3x	2.0x
Debt/cap	5.0%								
ROE (LTM)	NA								
5-yr fwd EPS growth (Norm)	NA								
		<b>EPS \$</b>	<b>2011</b>	<b>2012E</b>		<b>2013E</b>		<b>2014E</b>	<b>2015E</b>
		<b>Dec</b>	<b>Actual</b>	<b>Prior</b>	<b>Current</b>	<b>Prior</b>	<b>Current</b>	<b>Current</b>	<b>Current</b>
		Q1	—	—	(1.14)A	—	(0.55)	—	—
		Q2	—	—	(0.68)A	0.23	(0.50)	—	—
		Q3	—	—	(0.44)A	0.24	0.18	—	—
		Q4	—	—	(0.35)	0.36	0.30	—	—
<b>S&amp;P 500</b>	<b>1513.2</b>	Year	<b>3.34</b>	—	<b>(2.45)</b>	<b>0.30</b>	<b>(0.55)</b>	<b>1.80</b>	<b>1.85</b>
		P/E	—	—	—	—	—	8.9x	8.7x

## Investment Thesis

Hyperion is developing treatments in the areas of orphan disorders and hepatology. Hyperion's lead program includes a suite of products to treat urea cycle disorders (UCDs). UCDs are a constellation of diseases in which the body lacks the ability to clear ammonia, a byproduct of protein digestion. Approximately 2,000 people in the United States have a UCD, and just over 500 are receiving pharmacotherapy. Left untreated, UCDs can cause learning disabilities, mental retardation, and death. Hyperion owns an option to purchase the leading FDA-approved therapy for the chronic management of UCDs, Buphenyl, and will begin marketing it by mid-2013E. While Buphenyl can rescue patients from death, it has an excessive pill burden, a foul taste, and causes GI side effects in most patients. Hyperion has developed Ravicti to replace Buphenyl. Ravicti has succeeded in four Phase II and III trials, and received FDA approval on February 1, 2013. In a pooled analysis of all four studies, Ravicti has been shown to have significantly better ammonia control compared to Buphenyl. More important, Ravicti has far superior palatability in a dramatically reduced drug volume (1 tablespoon or less) with few GI side effects, making it much more tolerable for patients. Our checks suggest that Hyperion will successfully transfer the vast majority of treated UCD patients from Buphenyl to Ravicti over the next few years, and we project it will achieve \$100MM+ in sales by 2016. Outside of UCD, Ravicti has shown intriguing Phase II data in Hepatic Encephalopathy (HE), a potential \$1B market. Hyperion met with the FDA in Q4:12, and it expects to begin Phase III for HE in 2013. We believe that Hyperion is undervalued based on Ravicti's potential in UCD, without any contribution from other indications.

### Adjusting Model To Reflect Buphenyl Sales Starting In Q3, No Ammonul

We expect HPTX will take nearly the full contractually-allotted 90 days to exercise its option to purchase Buphenyl, and therefore have pushed out Buphenyl revenue from Q2 to Q3. This has pushed out our assumptions for Hyperion's profitability from Q2 to Q3, and FY profitability from 2013 to 2014. Additionally, Valeant has recently increased Ammonul's price nearly 3x, which suggests to us that VRX may retain it, so we have removed all Ammonul revenue assumptions from our model, but added a pro forma \$13MM payment from Valeant to Hyperion as is required if Valeant keeps Ammonul. While these changes have decreased near-term EPS, the inclusion of 13 years of exclusivity beyond the end of orphan increased the DCF value of the UCD franchise from \$16 to \$34.

#### Upcoming Hyperion Milestones

Event	Timing
Exercise option to purchase Buphenyl, Ammonul from Medicis	H1:13
Ravicti's U.S. commercial launch	H1:13
Potential initiation of Ravicti's pivotal trials in HE	2013

Source: Cowen and Company

## U.S. Urea Cycle Disorder Revenue Model

	2011A	2012E	2013E	2014E	2015E	2016E	2017E
<b>U.S. Urea Cycle Disorder Market</b>							
<b>Chronic Therapy</b>							
Prevalence Of Urea Cycle Disorders In U.S.	2090	2138	2186	2234	2282	2330	2378
Annual Incidence of UCD	160	160	160	160	160	160	160
Annual Mortality from UCD	112	112	112	112	112	112	112
Total Patients With Urea Cycle Disorders In The U.S.	2138	2186	2234	2282	2330	2378	2426
% Diagnosed	50%	50%	50%	50%	50%	50%	50%
Number Diagnosed	1045	1069	1093	1117	1141	1165	1189
<b>Buphenyl</b>							
% on Buphenyl	41%	40%	31%	24%	13%	3%	1%
Number of Patients On Buphenyl	425	425	342	192	154	38	15
Average Cost per Patient per Year (\$000)	55	55	95	130	130	130	130
<b>Buphenyl Revenue (\$MM)</b>	<b>23.4</b>	<b>23.4</b>	<b>32.5</b>	<b>25.0</b>	<b>20.0</b>	<b>5.0</b>	<b>2.0</b>
<b>Ravicti</b>							
% on Ravicti			6%	27%	37%	47%	53%
Number of Patients On Ravicti			67	350	425	550	625
Average Cost per Patient per Year (\$000)			200	200	200	200	200
<b>Ravicti Revenue</b>			<b>\$13.3</b>	<b>\$70.0</b>	<b>\$85.0</b>	<b>\$110.0</b>	<b>\$125.0</b>
Y/Y Growth				425%	21%	29%	14%

Source: Cowen and Company

### Hyperion Quarterly P&L (\$MM)

	Q1:12A	Q2:12A	Q3:12A	Q4:12E	2012E	Q1:13E	Q2:13E	Q3:13E	Q4:13E	2013E
Ravicti						-	1.8	3.5	8.0	13.3
Buphenyl						-	-	11.0	9.5	20.5
Ammonul						-	-	-	-	-
License and Other										
Total Revenue	-	-	-	-	-	-	1.8	14.5	17.5	33.8
COGS						-	0.2	0.9	1.3	2.4
Gross Margin							90%	94%	93%	93%
R&D	8.9	2.7	2.4	2.5	16.5	4.0	4.8	5.0	5.3	19.1
SG&A	2.3	2.0	2.4	3.1	9.8	5.2	5.3	5.4	5.5	21.4
Other										
Operating Expenses	11.2	4.8	4.8	5.6	26.3	9.2	10.3	11.3	12.1	42.9
Operating Income / (Loss)	(11.2)	(4.8)	(4.8)	(5.6)	(26.3)	(9.2)	(8.5)	3.2	5.4	(9.1)
Interest and other income, net	(0.7)	(2.4)	(0.2)	(0.2)	(3.4)	(0.1)	(0.1)	(0.1)	(0.1)	(0.5)
Pretax net income	(11.9)	(7.2)	(4.9)	(5.8)	(29.8)	(9.3)	(8.6)	3.1	5.3	(9.6)
Taxes						-	-	-	-	-
Tax Rate						0%	0%	0%	0%	0%
GAAP Net Income	(11.9)	(7.2)	(4.9)	(5.8)	(29.8)	(9.3)	(8.6)	3.1	5.3	(9.6)
GAAP EPS	\$(1.14)	\$(0.68)	\$(0.44)	\$(0.35)	\$(2.45)	\$(0.55)	\$(0.50)	\$ 0.18	\$ 0.30	\$(0.55)
Diluted Shares Outstanding (MM)	10.4	10.5	11.3	16.4	12.2	17.0	17.3	17.5	17.8	17.4

Source: Cowen and Company

### Hyperion Annual P&L (\$MM)

	2011A	2012E	2013E	2014E	2015E	2016E	2017E
Ravicti	-	-	13.3	70.0	85.0	110.0	125.0
Buphenyl	-	-	20.5	25.0	20.0	5.0	2.0
Ammonul	-	-	-	-	-	-	-
License and Other	-	-	-	-	-	-	-
Total Revenue	-	-	33.8	95.0	105.0	115.0	127.0
COGS	-	-	2.4	8.3	9.5	11.3	12.6
Gross Margin			93%	91%	91%	90%	90%
R&D	17.2	16.5	19.1	22.0	24.0	24.5	25.0
SG&A	8.9	9.8	21.4	24.2	26.8	28.2	29.2
Other	-	-	-	-	-	-	-
Operating Expenses	26.2	26.3	42.9	54.5	60.3	64.0	66.8
Operating Income / (Loss)	(26.2)	(26.3)	(9.1)	40.6	44.7	51.1	60.2
Interest and other income, net	(3.3)	(3.4)	(0.5)	(2.2)	-	4.0	4.0
Pretax net income	(29.4)	(29.8)	(9.6)	38.4	44.7	55.1	64.2
Taxes	-	-	-	2.1	6.7	16.5	19.3
Tax Rate	-	-	0%	6%	15%	30%	30%
GAAP Net Income	(29.4)	(29.8)	(9.6)	36.2	38.0	38.5	44.9
GAAP EPS	(3.34)	(2.45)	(0.55)	1.80	1.85	1.85	2.10
Diluted Shares Outstanding (MM)	8.8	12.2	17.4	20.1	20.6	20.8	21.4

Source: Cowen and Company

## Hyperion DCF - With Patent Protection

Financial Year End	12/31/2012
Valuation Date	2/2/2013
Discount Rate	10.0%
Terminal Growth Rate	-20.0%

### Hyperion: DCF Valuation Saturday, February 02, 2013

SMM	2012	2013	2014	2015	2016	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031	2032	2033
Ravicti Sales Growth (%)	0	13	70	85	110	125	138	144	152	159	167	175	184	193	203	213	224	235	247	259	272	218
Buphenyl Growth (%)	0	21	25	20	5	2	2	1	1	1	1	1	1	1	0	0	0	0	0	0	0	0
Ammonul Growth (%)	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
License and Other Growth (%)	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Total Revenues Growth (%)	0	34	95	105	115	127	139	146	153	160	168	176	185	194	204	214	224	235	247	259	272	218
COGS COGS as a % of sales	0	2	8	10	11	13	14	15	15	16	17	18	18	19	20	21	22	24	25	26	27	22
R&D R&D as a % of Revenues	17	19	22	24	25	25	18	16	15	16	17	18	18	19	20	21	22	24	25	26	27	22
SG&A SG&A as a % of Revenues	10	21	24	27	28	29	25	23	18	19	20	21	22	23	24	26	27	28	30	31	33	26
Operating Income	-26	-30	16	25	46	58	81	90	103	108	113	119	125	131	138	145	152	160	168	176	185	148
Tax Tax rate	0	0	1	4	14	17	24	27	31	32	34	36	38	39	41	43	46	48	50	53	56	44
NOL/ Tax Assets Utilized Tax rate	0%	0%	6%	15%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%
Taxes Paid	0	0	1	4	14	17	24	27	31	32	34	36	38	39	41	43	46	48	50	53	56	44
Approx Free Cash Flow	(26)	(30)	15	21	32	41	56	63	72	76	79	83	88	92	97	101	107	112	117	123	130	104
Years Discount Factor	-0.09 1.01	0.91 0.92	1.90 0.83	2.90 0.76	3.91 0.69	4.91 0.63	5.90 0.57	6.90 0.52	7.91 0.47	8.91 0.43	9.90 0.39	10.90 0.35	11.90 0.32	12.90 0.29	13.90 0.27	14.90 0.24	15.90 0.22	16.90 0.20	17.90 0.18	18.90 0.17	19.90 0.15	20.90 0.14
NPV of Cash flows	(27)	(27)	12	16	22	26	32	33	34	32	31	29	28	27	26	25	23	22	21	20	19	14

#### Terminal Value Calculation

Final year FCF	104
Perpetual Growth Rate	-20.0%
Terminal Value	276
Discount Factor	0.14
Present Value of Terminal Value	38
Present Value of Cash Flows	467
Enterprise Value	504
Add: Net cash	61
Market Value	565
Fully Diluted Shares Outstanding	16.4
Value per Fully Diluted Share	\$34.44

Source: Cowen and Company

**Hyperion DCF – No Patent**

Financial Year End	12/31/2012
Valuation Date	2/2/2013
Discount Rate	10.0%
Terminal Growth Rate	-10.0%

**Hyperion: DCF Valuation**  
**Saturday, February 02, 2013**

\$MM	2012	2013	2014	2015	2016	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026
<b>Ravicti Sales</b>	0	13	70	85	110	125	138	144	116	92	74	59	47	38	30
Growth (%)			426%	21%	29%	20%	10%	5%	-20%	-20%	-20%	-20%	-20%	-20%	-20%
<b>Buphenyl</b>	0	21	25	20	5	2	2	1	1	1	1	1	1	1	0
Growth (%)			22%	-20%	-75%	-15%	-15%	-15%	-15%	-15%	-15%	-15%	-15%	-15%	-15%
<b>Ammonul</b>	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Growth (%)			#DIV/0!	#DIV/0!	#DIV/0!	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
<b>License and Other</b>	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Growth (%)															
<b>Total Revenues</b>	0	34	95	105	115	127	139	146	117	93	75	60	48	38	31
Growth (%)			181%	11%	10%	10%	10%	5%	-20%	-20%	-20%	-20%	-20%	-20%	-20%
<b>COGS</b>	0	2	8	10	11	13	14	15	12	9	7	6	5	4	3
COGS as a % of sales		18%	12%	11%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%
<b>R&amp;D</b>	17	19	22	24	25	25	18	16	12	9	7	6	5	4	3
R&D as a % of Revenues	57%	23%	23%	21%	15%	13%	11%	10%	10%	10%	10%	10%	10%	10%	10%
<b>SG&amp;A</b>	10	21	24	27	28	29	25	23	14	11	9	7	6	5	4
SG&A as a % of Revenues	63%	25%	26%	25%	20%	18%	16%	12%	12%	12%	12%	12%	12%	12%	12%
<b>Operating Income</b>	-26	-30	16	25	46	58	81	90	78	63	50	40	32	26	20
<b>Tax</b>	0	0	1	4	14	17	24	27	23	19	15	12	10	8	6
Tax rate	0%	0%	6%	15%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%	30%
NOL/ Tax Assets Utilized															
Tax rate															
<b>Taxes Paid</b>	0	0	1	4	14	17	24	27	23	19	15	12	10	8	6
<b>Approx Free Cash Flow</b>	(26)	(30)	15	21	32	41	56	63	55	44	35	28	22	18	14
Years	-0.09	0.91	1.90	2.90	3.91	4.91	5.90	6.90	7.91	8.91	9.90	10.90	11.90	12.90	13.90
Discount Factor	1.01	0.92	0.83	0.76	0.69	0.63	0.57	0.52	0.47	0.43	0.39	0.35	0.32	0.29	0.27
<b>NPV of Cash flows</b>	(27)	(27)	12	16	22	26	32	33	26	19	14	10	7	5	4

**Terminal Value Calculation**

Final year FCF	14
Perpetual Growth Rate	-10.0%
<b>Terminal Value</b>	64
Discount Factor	0.27
<b>Present Value of Terminal Value</b>	17
<b>Present Value of Cash Flows</b>	198
<b>Enterprise Value</b>	215
Add: Net cash	61
<b>Market Value</b>	276
Fully Diluted Shares Outstanding	16.4
<b>Value per Fully Diluted Share</b>	\$16.81

Source: Cowen and Company

## Addendum

### STOCKS MENTIONED IN IMPORTANT DISCLOSURES

Ticker	Company Name
HPTX	Hyperion Therapeutics

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(a) Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period.

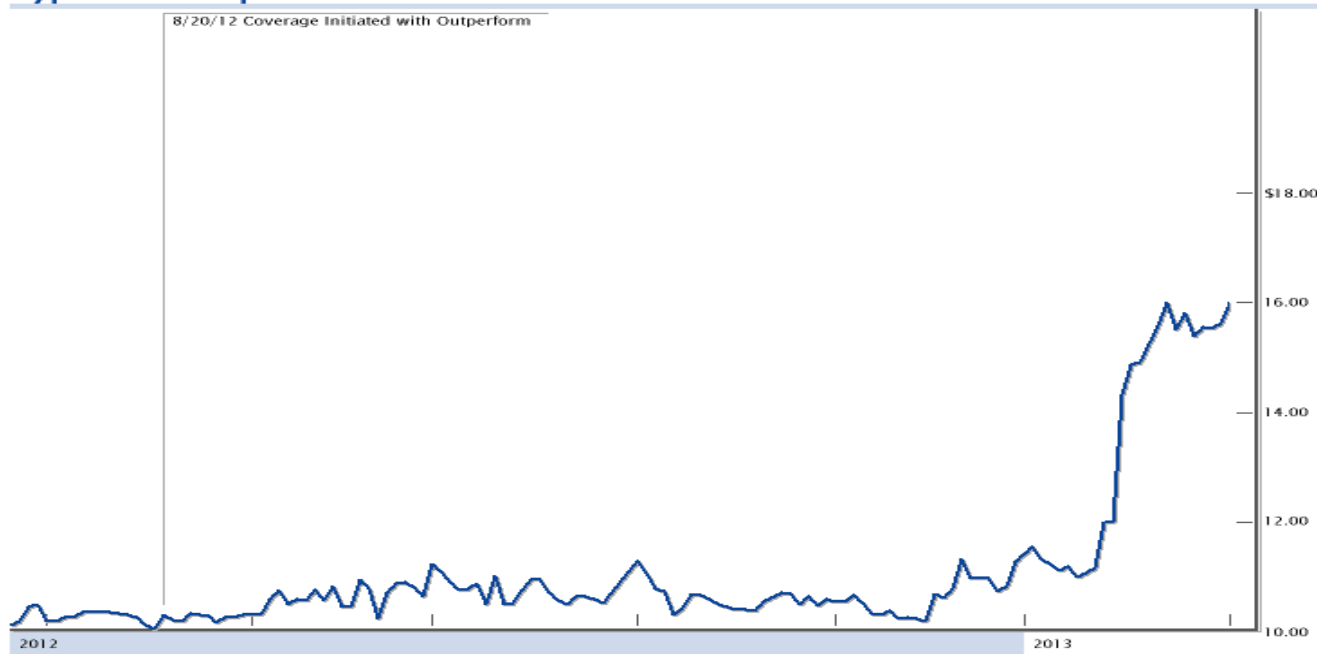
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Cowen and Company Price and Ratings History

### Hyperion Therapeutics - HPTX



Pricing data provided by Reuters America. Chart as of 2/1/13 in USD.



