

Reason for report:

EARNINGS

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

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

Paul Matteis
(617) 918-4585
Paul.Matteis@Leerink.com

LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

HYPERION THERAPEUTICS, INC.

1Q13 EPS - Ravicti Launch Underway, Bullish on Quick Patient Uptake

• **Bottom Line:** Today after the close HPTX reported 1Q13 EPS of (\$0.52) versus our estimate of (\$0.80) and was able to recognize the first Ravicti (urea cycle disorders [UCD]) revenues after launching Ravicti one month earlier than we and the Street anticipated. We believe HPTX's ahead-of-schedule Ravicti launch reflects the company's extensive commercial preparedness and its ability to execute with a lean and effective organization. We have updated our model to reflect 1Q13 results, HPTX's follow-on equity offering, and have increased our Ravicti sales projections in 2013 and 2014. With roughly half of the clinical trial patients transitioned to commercial Ravicti, our 2013 HPTX revenue estimate of \$29MM may even be conservative. **Reiterate Outperform on HPTX, increasing our 12 month FVE to \$33 from \$30 previously.**

• **Management is encouraged by the physician demand they've seen for Ravicti, with more than half of the prescribers coming from outside the Ravicti clinical trial.** While HPTX did not provide specific patient or TRx guidance, the company is very impressed with physician interest in Ravicti with ~25% of targeted prescribers writing at least one TRx thus far. We believe the recent emergence of Sigma-Tau's generic Buphenyl will not have a significant influence on Ravicti. With only a 13% spread between branded and generic Buphenyl, which already costs just \$76k/patient/year vs. \$277k/patient/year for Ravicti, we do not believe that Buphenyl's lower price will be able to prevent patients from switching to Ravicti and should not inhibit HPTX from recognizing branded Buphenyl sales as planned.

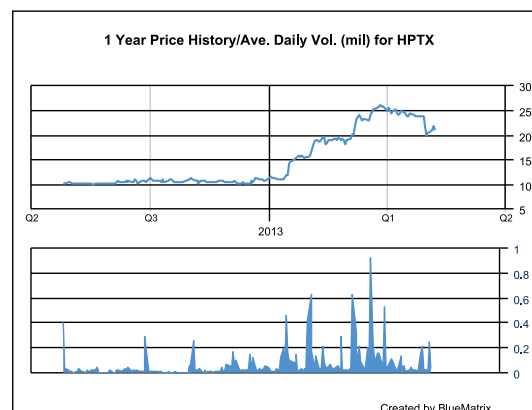
• **We continue to believe that Ravicti is best-in-class for UCD, and Buphenyl presents a variety of issues to UCD patients.** These include but are not limited to a large dose burden, frequent (3-6 times/day) administration, unpleasant taste and smell, tolerability issues, and high sodium content. From a physician point-of-view, 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. This should be attractive to payors, who in our checks do not indicate a willingness to manage this ultra-orphan category.

• **We anticipate that VRX will exercise its option to retain Ammunol, essentially providing Buphenyl to HPTX for free along with a ~\$13MM gain.** Recall, HPTX recently exercised its option to purchase all of Ucyd's (now VRX) WW rights in Buphenyl and Ammunol for an upfront payment of \$22MM, but VRX now has until May 22 to retain Ammunol at a purchase price of \$32MM.

Key Stats:

(NASDAQ:HPTX)

| | |
|---------------------------------------|-----------------------|
| S&P 600 Health Care Index: | 946.00 |
| Price: | \$21.09 |
| 52 Week High: | \$26.50 |
| 52 Week Low: | \$9.95 |
| Shares Outstanding (mil): | 16.6 |
| Market Capitalization (mil): | \$350.1 |
| Book Value/Share: | \$0.00 |
| Cash Per Share: | \$2.00 |
| Dividend (ann): | \$0.00 |
| Dividend Yield: | 0.0% |
| Valuation: | \$33 from \$30 on DCF |



| Dec Yr | 1Q | 2Q | 3Q | 4Q | FY Rev | 1Q | 2Q | 3Q | 4Q | FY EPS | P/E |
|-------------|---------------|--------------|--------------|---------------|----------------|-----------------|---------------|-----------------|-----------------|-----------------|--------------|
| 2012A | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 | (\$25.33) | (\$15.26) | (\$0.44) | (\$0.50) | (\$4.45) | NM |
| 2013E - New | \$0.8A | \$1.5 | \$9.6 | \$16.6 | \$28.5 | (\$0.52) | \$0.03 | (\$0.52) | (\$0.24) | (\$1.24) | NM |
| 2013E - Old | 0.0 | 0.0 | \$6.4 | \$9.3 | \$15.7 | (\$0.80) | (\$0.04) | (\$0.53) | (\$0.44) | (\$1.81) | NM |
| 2014E - New | -- | -- | -- | -- | \$100.4 | -- | -- | -- | -- | \$0.79 | 26.7x |
| 2014E - Old | -- | -- | -- | -- | \$99.1 | -- | -- | -- | -- | \$1.17 | 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 and obtained FDA approval pursuant to a special protocol assessment (SPA). HPTX has also completed Phase II trials for Ravicti in HE and had an end-of-Phase II meeting in 4Q:12. Ravicti has a similar mechanism of action to already 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 launched Ravicti in March 2013, a month ahead of expectations, with a lean team of experienced personnel including seven field sales representatives, one senior director who manages the group, and also a payor specialist team of four. On the clinical side, HPTX has a medical science liaison team of four specialists, consisting of one director and three field-based specialists running back-office operations. We project that HPTX achieves breakeven in 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 (MP) 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.

VALUATION

Our 12-month fair value estimate for HPTX is \$33/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 | 4Q12 | 2012 | 1Q13 | 2Q13E | 3Q13E | 4Q13E | 2013E | 2014E | 2015E |
|------------------------------|----------------|----------------|----------------|----------------|---------------|---------------|---------------|---------------|-------------|---------------|---------------|---------------|-------------|-------------|
| Revenue | - | - | - | - | - | - | - | 0.8 | 1.5 | 9.6 | 16.6 | 28.5 | 100.4 | 115.7 |
| COGS | - | - | - | - | - | - | - | 0.1 | 0.2 | 1.4 | 2.5 | 4.2 | 15.1 | 17.4 |
| R&D | 23.1 | 17.2 | 8.9 | 2.7 | 2.4 | 3.0 | 17.0 | 1.8 | 5.0 | 8.0 | 8.0 | 22.8 | 30.1 | 34.7 |
| SG&A | 3.5 | 8.9 | 2.3 | 2.0 | 2.4 | 4.8 | 11.5 | 7.9 | 8.5 | 9.0 | 10.0 | 35.4 | 39.0 | 40.9 |
| Operating expenses | 26.6 | 26.2 | 11.2 | 4.8 | 4.8 | 7.8 | 28.5 | 9.9 | 13.7 | 18.4 | 20.5 | 62.5 | 84.2 | 93.0 |
| Operating income | (26.6) | (26.2) | (11.2) | (4.8) | (4.8) | (7.8) | (28.5) | (9.1) | (12.2) | (8.8) | (3.9) | (34.0) | 16.2 | 22.7 |
| Total other income (expense) | 1.1 | (3.3) | (0.7) | (2.4) | (0.2) | (0.5) | (3.7) | 0.1 | 12.8 | (0.2) | (0.2) | 12.4 | (0.8) | (0.1) |
| EBT | (25.5) | (29.4) | (11.9) | (7.2) | (4.9) | (8.3) | (32.3) | (9.0) | 0.5 | (9.0) | (4.1) | (21.6) | 15.5 | 22.6 |
| Tax expense | - | - | - | - | - | - | - | - | - | - | - | - | - | - |
| Net income (loss) | (25.5) | (29.4) | (11.9) | (7.2) | (4.9) | (8.3) | (32.3) | (9.0) | 0.5 | (9.0) | (4.1) | (21.6) | 15.5 | 22.6 |
| Diluted EPS | (61.70) | (62.68) | (25.33) | (15.26) | (0.44) | (0.50) | (4.45) | (0.52) | 0.03 | (0.52) | (0.24) | (1.24) | 0.79 | 1.15 |
| Basic shares outstanding | 0.4 | 0.5 | 0.5 | 0.5 | 11.3 | 16.6 | 7.3 | 17.4 | 17.4 | 17.4 | 17.4 | 17.4 | 17.5 | 17.6 |

Source: SEC filings and Leerink Swann Estimates

| HPTX BS | 2010 | 2011 | 1Q12 | 2Q12 | 3Q12 | 4Q12 | 2012 | 1Q13 | 2Q13E | 3Q13E | 4Q13E | 2013E | 2014E | 2015E |
|-------------------|------|------|------|------|------|------|------|-------|-------|-------|-------|-------|-------|-------|
| Cash | 6.6 | 7.0 | 3.7 | 7.3 | 56.5 | 49.9 | 49.9 | 102.7 | 104.3 | 96.6 | 94.0 | 94.0 | 116.3 | 134.5 |
| Debt | - | 23.4 | 30.7 | 40.5 | 12.0 | 12.1 | 12.1 | 12.0 | 12.0 | 12.0 | 12.0 | 12.0 | 12.0 | - |
| Convertible notes | - | 23.4 | 30.7 | 31.2 | - | - | - | - | - | - | - | - | - | - |
| Venture debt | - | - | - | 9.4 | 12.0 | 12.1 | 12.1 | 12.0 | 12.0 | 12.0 | 12.0 | 12.0 | 12.0 | - |
| Ucyclyd loan | - | - | - | - | - | - | - | - | - | - | - | - | - | - |

| HPTX CFS | 2010 | 2011 | 1Q12 | 2Q12 | 3Q12 | 4Q12 | 2012 | 1Q13 | 2Q13E | 3Q13E | 4Q13E | 2013E | 2014E | 2015E |
|-----------------------------|--------|--------|--------|-------|-------|-------|--------|--------|-------|-------|-------|--------|-------|--------|
| Change in cash | (3.5) | 0.4 | (3.3) | 3.6 | 49.2 | (6.7) | 42.8 | 52.8 | 1.6 | (7.7) | (2.6) | 44.1 | 22.4 | 18.1 |
| Cash from operations | (25.9) | (24.5) | (10.7) | (5.8) | (5.7) | (6.3) | (28.5) | (10.8) | 1.6 | (7.7) | (2.6) | (19.5) | 22.4 | 30.1 |
| Net Income | (25.5) | (29.4) | (11.9) | (7.2) | (4.9) | (8.3) | (32.3) | (9.0) | 0.5 | (9.0) | (4.1) | (21.6) | 15.5 | 22.6 |
| SOE | 0.2 | 0.3 | 0.1 | 0.2 | 0.3 | 0.4 | 1.0 | 0.5 | 1.1 | 1.4 | 1.4 | 4.4 | 6.9 | 7.6 |
| Other | (0.6) | 4.5 | 1.1 | 1.1 | (1.1) | 1.6 | 2.7 | (2.3) | - | - | - | (2.3) | - | - |
| Cash from investing | (0.0) | (0.0) | (0.1) | 0.2 | (0.0) | (0.0) | 0.0 | (0.1) | - | - | - | (0.1) | - | - |
| Option to purchase Buphenyl | - | - | (0.3) | - | - | - | (0.3) | - | - | - | - | - | - | - |
| Other | (0.0) | (0.0) | 0.2 | 0.2 | (0.0) | (0.0) | 0.3 | (0.1) | - | - | - | (0.1) | - | - |
| Cash from financing | 22.4 | 25.0 | 7.6 | 9.2 | 55.0 | (0.4) | 71.4 | 63.7 | - | - | - | 63.7 | - | (12.0) |
| Issuance (buyback) shares | 22.5 | - | (0.0) | 0.1 | 53.4 | - | 53.5 | 63.7 | - | - | - | 63.7 | - | - |
| Issuance (repay) debt | - | 25.0 | 7.5 | 10.0 | 2.5 | - | 20.0 | - | - | - | - | - | - | (12.0) |
| Other | (0.0) | - | 0.1 | (0.9) | (0.9) | (0.4) | (2.1) | - | - | - | - | - | - | - |

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 | - | - | - | 6 | 46 | 13 | 64 | 88 | 119 | 120 | 121 | 122 | 123 |
| penetration, patients < age 6 | 0% | 0% | 0% | 1% | 8% | 2% | 11% | 15% | 20% | 20% | 20% | 20% | 20% |
| penetration, patients age 6-17 | 0% | 0% | 0% | 1% | 8% | 2% | 11% | 15% | 20% | 20% | 20% | 20% | 20% |
| penetration, adults | 0% | 0% | 0% | 1% | 8% | 2% | 11% | 15% | 20% | 20% | 20% | 20% | 20% |
| Prior Buphenyl treated pts on Ravicti | - | 14 | 26 | 86 | 171 | 74 | 302 | 391 | 438 | 442 | 446 | 450 | 453 |
| penetration, patients < age 6 | 0% | 3% | 6% | 20% | 40% | 17% | 70% | 90% | 100% | 100% | 100% | 100% | 100% |
| penetration, patients age 6-17 | 0% | 3% | 6% | 20% | 40% | 17% | 70% | 90% | 100% | 100% | 100% | 100% | 100% |
| penetration, adults | 0% | 3% | 6% | 20% | 40% | 17% | 70% | 90% | 100% | 100% | 100% | 100% | 100% |
| Total Ravicti pts | - | 14 | 26 | 91 | 218 | 87 | 366 | 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) | - | 0.8 | 1.5 | 5 | 13 | 20 | 84 | 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 | 412 | 401 | 342 | 257 | 353 | 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 | 0.019 | 0.019 | 0.019 | 0.019 | 0.075 | 0.230 | 0.230 | 0.230 | 0.230 | 0.230 | 0.230 | 0.230 |
| Buphenyl US sales in UCD (\$MM) | 10 | 3 | 3 | 3 | 2 | 11 | 12 | 4 | - | - | - | - | - |

| Buphenyl Model Medicare/Medicaid | 2012E | 1Q13E | 2Q13E | 3Q13E | 4Q13E | 2013E | 2014E | 2015E | 2016E | 2017E | 2018E | 2019E | 2020E |
|--|-----------|----------|----------|----------|----------|-----------|----------|----------|----------|----------|----------|----------|----------|
| Patients on Buphenyl | 425 | 412 | 401 | 342 | 257 | 353 | 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 | 3 | 3 | 3 | 2 | 12 | 4 | 1 | - | - | - | - | - |

| | | | | | | | | | | | | | |
|---------------------------------------|-----------|----------|----------|-----------|-----------|----------|----------|----------|----------|----------|----------|----------|----------|
| Total Buphenyl US sales (\$MM) | 24 | 5 | 4 | 23 | 16 | 5 | - | - | - | - | - | - | - |
|---------------------------------------|-----------|----------|----------|-----------|-----------|----------|----------|----------|----------|----------|----------|----------|----------|

| | | | | | | | | | | | | | |
|---------------------------------------|-----------|----------|----------|-----------|-----------|-----------|------------|------------|------------|------------|------------|------------|------------|
| Total sales Ravicti + Buphenyl | 33 | 1 | 1 | 10 | 17 | 29 | 100 | 116 | 128 | 129 | 130 | 131 | 132 |
|---------------------------------------|-----------|----------|----------|-----------|-----------|-----------|------------|------------|------------|------------|------------|------------|------------|

Source: SEC filings and Leerink Swann Estimates

| 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 | - | - | - | 6 | 46 | 13 | 64 | 88 | 119 | 120 | 121 | 122 | 123 |
| penetration, patients < age 6 | 0% | 0% | 0% | 1% | 8% | 2% | 11% | 15% | 20% | 20% | 20% | 20% | 20% |
| penetration, patients age 6-17 | 0% | 0% | 0% | 1% | 8% | 2% | 11% | 15% | 20% | 20% | 20% | 20% | 20% |
| penetration, adults | 0% | 0% | 0% | 1% | 8% | 2% | 11% | 15% | 20% | 20% | 20% | 20% | 20% |
| Prior Buphenyl treated pts on Ravicti | - | 14 | 26 | 86 | 171 | 74 | 302 | 391 | 438 | 442 | 446 | 450 | 453 |
| penetration, patients < age 6 | 0% | 3% | 6% | 20% | 40% | 17% | 70% | 90% | 100% | 100% | 100% | 100% | 100% |
| penetration, patients age 6-17 | 0% | 3% | 6% | 20% | 40% | 17% | 70% | 90% | 100% | 100% | 100% | 100% | 100% |
| penetration, adults | 0% | 3% | 6% | 20% | 40% | 17% | 70% | 90% | 100% | 100% | 100% | 100% | 100% |
| Total Ravicti pts | - | 14 | 26 | 91 | 218 | 87 | 366 | 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 | 1 | 5 | 13 | 20 | 84 | 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 | 412 | 401 | 342 | 257 | 353 | 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 | 0.019 | 0.019 | 0.019 | 0.019 | 0.075 | 0.230 | 0.230 | 0.230 | 0.070 | 0.070 | 0.070 | 0.070 |
| Buphenyl US sales in UCD (\$MM) | 10 | 3 | 3 | 3 | 2 | 11 | 12 | 4 | - | - | - | - | - |

| Buphenyl Model Medicare/Medicaid | 2012E | 1Q13E | 2Q13E | 3Q13E | 4Q13E | 2013E | 2014E | 2015E | 2016E | 2017E | 2018E | 2019E | 2020E |
|--------------------------------------|-------|-------|-------|-------|-------|-------|-------|-------|-------|-------|-------|-------|-------|
| Patients on Buphenyl | 425 | 412 | 401 | 342 | 257 | 353 | 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 | 3 | 3 | 3 | 2 | 12 | 4 | 1 | - | - | - | - | - |

| | | | | | | | | | | | | | |
|--------------------------------|----|---|---|---|---|----|----|---|---|---|---|---|---|
| Total Buphenyl US sales (\$MM) | 24 | 7 | 6 | 5 | 4 | 23 | 16 | 5 | - | - | - | - | - |
|--------------------------------|----|---|---|---|---|----|----|---|---|---|---|---|---|

| Ravicti for HE | 2012E | 1Q13E | 2Q13E | 3Q13E | 4Q13E | 2013E | 2014E | 2015E | 2016E | 2017E | 2018E | 2019E | 2020E |
|-------------------------------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|
| Total US HE pts diagnosed | 140,000 | 140,297 | 140,594 | 140,892 | 141,190 | 140,743 | 141,939 | 143,146 | 144,363 | 145,590 | 146,827 | 148,075 | 149,334 |
| Total severe HE patients | 28,000 | 28,059 | 28,119 | 28,178 | 28,238 | 28,149 | 28,388 | 28,629 | 28,873 | 29,118 | 29,365 | 29,615 | 29,867 |
| % severe HE patients | 20% | 20% | 20% | 20% | 20% | 20% | 20% | 20% | 20% | 20% | 20% | 20% | 20% |
| % Ravicti patients | 0% | 0% | 0% | 0% | 0% | 0% | 0% | 0% | 0% | 5% | 10% | 15% | 25% |
| Ravicti pts in HE | - | - | - | - | - | - | - | - | - | 1,456 | 2,937 | 4,442 | 7,467 |
| Avg cost/pt(\$mm) | - | - | - | - | - | - | - | - | - | 0.070 | 0.070 | 0.070 | 0.070 |
| Ravicti US sales in HE (\$MM) | - | - | - | - | - | - | - | - | - | 102 | 206 | 311 | 523 |

| | | | | | | | | | | | | | |
|--------------------------------|----|---|---|----|----|----|-----|-----|-----|-----|-----|-----|-----|
| Total sales Ravicti + Buphenyl | 33 | 1 | 1 | 10 | 17 | 29 | 100 | 116 | 128 | 141 | 245 | 351 | 563 |
|--------------------------------|----|---|---|----|----|----|-----|-----|-----|-----|-----|-----|-----|

Source: SEC filings and Leerink Swann Estimates

| 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 | (19) | 22 | 30 | 29 | 57 | 89 | 136 | 218 | 211 | 213 | 215 | 216 | 73 | 57 | 37 | 25 | 25 | 21 | 4 | |
| Discount periods | - | 0.8 | 1.8 | 2.8 | 3.8 | 4.8 | 5.8 | 6.8 | 7.8 | 8.8 | 9.8 | 10.8 | 11.8 | 12.8 | 13.8 | 14.8 | 15.8 | 16.8 | 17.8 | |
| NPV | (19) | 21 | 25 | 21 | 37 | 52 | 71 | 102 | 88 | 79 | 71 | 64 | 19 | 13 | 8 | 5 | 4 | 3 | 1 | 8 |
| Valuation | 671 | | | | | | | | | | | | | | | | | | | |

| | |
|-----------------|-----|
| Discount Rate | 12% |
| Terminal Growth | 5% |

| Valuation | Valuation | per shr | Probability | P/W |
|---|-----------|---------|-------------|-------|
| Scenario 1, Ravicti approved for UCD only | 327 | \$ 17 | 35% | 114 |
| Scenario 2, Ravicti approved for UCD and HE | 671 | \$ 35 | 65% | 436 |
| Blended Valuation | | | | 551 |
| Net cash | | | | 82.0 |
| Diluted Shares Outstanding | | | | 19.4 |
| Per share valuation | | | | \$ 33 |

Source: SEC filings and Leerink Swann Estimates

| Hyperion Therapeutics, Inc. (HPTX) Expected Milestones | | | Timing |
|--|----|--|-----------|
| Ammunol | | Expiration MRX/VRX right to retain Ammunol | 5/22/2013 |
| 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

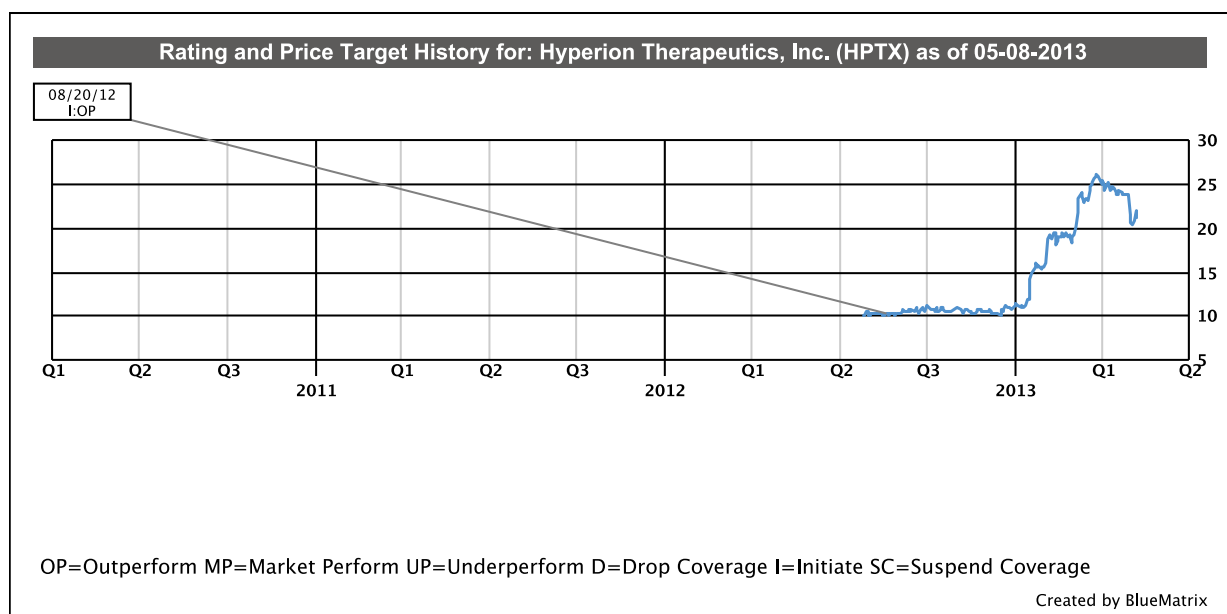
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Valuation

Our 12-month fair value estimate for HPTX is \$33/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 Banking Services (IB) as of 03/31/13 | | | | |
|---|-------|---------|-----------------------|---------|
| Rating | Count | Percent | IB Serv./Past 12 Mos. | |
| | | | Count | Percent |
| BUY [OP] | 107 | 61.14 | 32 | 29.91 |
| HOLD [MP] | 68 | 38.86 | 0 | 0.00 |
| SELL [UP] | 0 | 0.00 | 0 | 0.00 |

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

This information (including, but not limited to, prices, quotes and statistics) has been obtained from sources that we believe reliable, but we do not represent that it is accurate or complete and it should not be relied upon as such. All information is subject to change without notice. This is provided for information purposes only and should not be regarded as an offer to sell or as a solicitation of an offer to buy any product to which this information relates. The Firm, its officers, directors, employees, proprietary accounts and affiliates may have a position, long or short, in the securities referred to in this report, and/or other related securities, and from time to time may increase or decrease the position or express a view that is contrary to that contained in this report. The Firm's salespeople, traders and other professionals may provide oral or written market commentary or trading strategies that are contrary to opinions expressed in this report. The Firm's asset management group and proprietary accounts may make investment decisions that are inconsistent with the opinions expressed in this report. The past performance of securities does not guarantee or predict future performance. Transaction strategies described herein may not be suitable for all investors. Additional information is available upon request by contacting the Publishing Department at One Federal Street, 37th Floor, Boston, MA 02110.

Like all Firm employees, analysts receive compensation that is impacted by, among other factors, overall firm profitability, which includes revenues from, among other business units, the Private Client Division, Institutional Equities, and Investment Banking. Analysts, however, are not compensated for a specific investment banking services transaction.

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.

©2013 Leerink Swann LLC. All rights reserved. This document may not be reproduced or circulated without our written authority.

| 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 |
| | Rene Shen | (212) 277-6074 | rene.shen@leerink.com |
| | Gena Wang, Ph.D. | (212) 277-6073 | gena.wang@leerink.com |
| | Paul Matteis | (617) 918-4585 | paul.matteis@leerink.com |
| Life Science Tools and Diagnostics | Dan Leonard | (212) 277-6116 | dan.leonard@leerink.com |
| | John L. Sullivan, CFA | (617) 918-4875 | john.sullivan@leerink.com |
| | Justin Bowers, CFA | (212) 277-6066 | justin.bowers@leerink.com |
| Pharmaceuticals/Major | Seamus Fernandez | (617) 918-4011 | seamus.fernandez@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 |
| | Christopher W. Kuehnle, JD | (617) 918-4851 | chris.kuehnle@leerink.com |
| Medical Devices, Cardiology & Orthopedics | Danielle Antalffy | (212) 277-6044 | danielle.antalffy@leerink.com |
| | Richard Newitter | (212) 277-6088 | richard.newitter@leerink.com |
| | Robert Marcus, CFA | (212) 277-6084 | robert.marcus@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 |
| Research Assistant | Paul Matteis | (617) 918-4585 | paul.matteis@leerink.com |
| | George Villarina | (212) 277-6012 | george.villarina@leerink.com |
| | | | |
| New York 1251 Avenue of Americas, 22 nd 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, 16 th Floor San Francisco, CA 94105 (800) 778-1164 | |