# LEERINK MORNING ROUNDS



## **Today's Highlights**

**Featured Research:** ALKS, AMRN, ARNA, ATRC, BIO, CLVS, CTRX, HPTX, HTWR, MGLN, MYL, NXTM, OPHT, PODD, SLXP, SRPT, Biopharma, Healthcare, Specialty Pharmaceuticals

NXTM, PODD, SLXP, ALKS, OPHT, SRPT | MEDACorp: Specialty Pharmaceuticals, CLVS

**Reference Tools:** 

<u>Catalyst Tracker</u> | <u>Calendar of Events</u> | <u>HC Conferences</u> | <u>Earnings Rundown</u> | <u>Abbreviations & Acronyms</u> | <u>Leerink Events</u>

# **Today's Healthcare Events**

FDA AdCom: Vaccines and Related Biologics Products

**Investor Meeting: PRGO** 

Medical Meetings: Association of Academic Psychiatrists; American Society for Blood and Marrow Transplantation;

American Glaucoma Society; American Academy of Allergy, Asthma, and Immunology

EPS BMO: AUXL, DYAX, ENDP, ISIS

# **Today's Research**

Magellan Health Services, Inc. (MGLN) | Ana Gupte, Ph.D.

Initiating with MP; A Fairly Valued Show-Me Story, Upside from Take-Out

| Market Cap: \$1,681.1M | Price: \$60.69 | Price Target: \$65.00 | Methodology: P/E Of 16x On Our 2015 & Amp; 2016 EPS Estimate + Parent Cash

- Bottom Line: We initiate on MGLN with a Market Perform rating and a price target of \$65. The call on MGLN is about 1) CEO Barry Smith's ambitious, transformational agenda into a high growth player, 2) valuation and 3) likelihood of take-out. MGLN is targeting revenue growth of \$2.5 B each in its Medicaid/Duals and Specialty Pharmaceutical Business over the next 5 years. We expect 2014 to be a transition year and have an in line EPS estimate of \$2.55 as the company faces significant contract revenue losses and margin pressure from new business start-up. We are marginally above consensus in 2015 with a \$3.14 EPS estimate, compared to consensus at \$3.10. We estimate EPS CAGR to be as much as 20% commencing in 2016, as newly written businesses become increasingly accretive after initial pressure, though contract losses pose downside. We initiate with a Market Perform rating and a \$65 price target, though take-out offers upside given attractive synergies to a Managed Care player such as WLP (OP), CI (OP) or AET (OP).
- We view 2014 as a transition year and model an in line EPS of \$2.55, toward the high end of the guidance range of \$2.00 \$2.56.
- We see the ambitious agenda of \$2.5 B in revenue growth in each of the Medicaid/Duals & Specialty Rx businesses fueled by organic growth and acquisition to begin to bear fruit in 2015, with a marginally above consensus \$3.14 EPS and a 5 year EPS CAGR of as much as 20% excluding any potential future contract losses.
- Balance Sheet is solid with attractive cash flow generation and parent cash of \$9.40, though share repurchase activity has been dwindling in the last couple of years.
- · We initiate with a Market Perform rating though take-out presents attractive optional upside.
- 2013E EPS/REV: \$4.56 / \$3,517.0
- 2014E EPS/REV: \$2.55 / \$3,611.0

Revenues in \$ Million

Specialty Pharmaceuticals | Jason M. Gerberry, JD

**APS Call Improves Our Outlook for US Approvability of Defibrotide** 

Outperform: JAZZ, SLXP

- Bottom Line: Yesterday, we hosted a MEDACorp Pulse Call with a regulatory specialist to discuss the regulatory outlook for JAZZ/Sigma Tau's US application for defibrotide (Veno-Occlusive Disease [VOD]) and the upcoming FDA Advisory Committee to discuss theoretical safety risks associated with the mu opioid receptor antagonists for treatment of opioid induced constipation (OIC). On the call, the specialist was surprisingly optimistic about the regulatory prospects for defibrotide, based on some new data disclosed in the European review. On the mu's, the specialist was more pessimistic about the outlook for the class & SLXP's (OP) Relistor -- which is consistent with the consensus outlook. We are not making any changes to our JAZZ (OP) or SLXP estimates based on yesterday's APS call. See our preview note for additional background (LINK).
- EU review of defibrotide patient registry may shed light on JAZZ opportunity in US.
- Specialist surprisingly constructive on potential for defibrotide to get broad label; some regulatory hurdles remain.
- · Pathway forward for Relistor and class of mu's looks challenging.
- · Likely path forward will include another pre-registration study.

## Amarin Corporation PLC (AMRN) | Joseph P. Schwartz

### **4Q Recap: Vascepa Misses During Salesforce Transition**

Market Perform | Market Cap: \$313.5M | Price: \$1.78 | Price Target: \$2.00 | Methodology: DCF analysis, 11% WACC

- Bottom Line: Today after the close, AMRN reported 4Q13 revenues of \$10.1MM, which beat our estimate of \$9.5MM but missed consensus of \$10.7MM. We are updating our model to reflect 4Q13 results and AMRN's 2014 operating cashflow guidance of ~(\$80MM). Reiterate MP and \$2 PT in 12 months.
- AMRN continues to work through the Special Protocol Assessment (SPA) appeal process,
- · AMRN re-emphasized its commitment to attain profitability before it needs to return to the public markets,
- · New salesforce initiatives could bolster Vascepa uptake,
- · Vascepa's intellectual property (IP) is well suited to protect the product over the long term,
- 2014E EPS/REV: (\$0.70) / \$53.1 → (\$0.67) / \$55.5 Revenues in \$MM.

### Arena Pharmaceuticals, Inc. (ARNA) | Marko Kozul, M.D.

### Strong Belvig Indication Expansion/Potency & Pipeline; 4Q13 EPS; Reit OP, \$12PT

Outperform | Market Cap: \$1,472.5M | Price: \$6.77 | Price Target: \$12.00 | Methodology: DCF, 10% discount rate, 1% terminal growth rate

- Bottom Line: In 2014, we see ARNA emerging as a Belviq supplemental indication and pipeline story beyond its broadening obesity growth potential. We continue to believe OREX (OP) could emerge as the broader obesity market leader if approval in the US and EU is granted but comparatively, ARNA is increasingly attractive based on risk diversification away from Belviq's current approval profile. Mid-14, ARNA will have data from a Phase II Belviq-Phentermine combo trial at around the same time of Contrave's 6.10.14 PDUFA. Around YE14, ARNA will have Phase II data from a smoking cessation trial around the same time as a potential Contrave EU approval decision. We believe ARNA stands to benefit from both these intrinsic and external catalysts. ARNA could also be further fueled by data read out for pipeline candidates that currently attract no value from most investors. Finally, we continue to view the next few years as a broader obesity marketing effort and do not believe current market share gains or losses (i.e. VVUS) representing 2% of the obesity market are representative of longer term potential trends. We reiterate an Outperform (OP) rating and \$12 price target (PT).
- 4Q13 total revenue was \$6.5M and Belviq net product revenue to ARNA was \$2.4M (implying \$7.5M net Belviq sales booked by Eisai) beating our estimate of \$1.6M.
- · Mid-14 Belviq-Phentermine combo data could reposition Belviq's efficacy profile and serve as an important catalyst.
- YE14 Belviq smoking cessation data could begin expand the drug's potential beyond obesity.
- 2014E EPS/REV: (\$0.37) / \$47.8  $\rightarrow$  (\$0.42) / \$48.9

Revenues: \$MM.

AtriCure, Inc. (ATRC) | Danielle Antalffy

### **4Q13: No Major Surprises After a Positive Preannouncement**

Outperform | Market Cap: \$448.9M | Price: \$21.58 | Price Target: \$25.00 | Methodology: ~5x EV/2015E sales

- Bottom Line: Yesterday after the market close, ATRC announced 4Q13 sales of \$21.9M (+19%) -- which came in a healthy ~\$1.8M above us and the Street at ~\$20.2M prior to the mid-January preannouncement. And increasing our confidence that this strong momentum is sustainable, management reiterated 2014 sales guidance of \$100M-103M (+22%-26%), with organic growth ex. the recent Estech acquisition of 13%-15% vs. our 12% forecast prior to the preannouncement. Following a strong 2013, we believe ATRC is well on track to achieve if not exceed its 15%+ longer-term organic revenue growth target through its focus on four key areas: (1) Open heart surgical ablation; (2) Minimally invasive surgical (MIS) ablation; (3) Left atrial appendage; and (4) OUS expansion. We reiterate our OP rating \$25 price target, as we believe ATRC will deliver consistent "beat and raise" quarters under CEO Mike Carrel which -- in our view -- warrants a higher multiple.
- 2014E EPS/REV: (\$0.54) / \$89.9 → (\$0.89) / \$100.7 Revenues in \$M.

# Bio-Rad Laboratories, Inc. (BIO) | Dan Leonard

### 4Q Beat; Investments to Continue in 2014

Market Perform | Market Cap: \$3,823.7M | Price: \$131.85 | Price Target:  $$127.00 \rightarrow $131.00$  | Methodology:  $\sim 10x$  EBITDAO TTM Dec '15

- **Bottom Line:** We are reducing our estimates to reflect pressure in the company's Diagnostics segment increased opex. We maintain our Market Perform rating and increase our price target to \$131 (from \$127) to reflect group multiple expansion.
- · Revenue and EPS all beat the Street.
- · Life Sciences rebounded during the quarter.
- Diagnostics mixed; menu expansion likely.
- · Estimates reduced.
- 2014E EPS/REV: \$4.87 / \$2,192.3 → \$4.24 / \$2,192.6
- 2015E EPS/REV: \$5.25 / \$2,277.0 → \$4.74 / \$2,273.4

Revenues in US\$ millions.

Clovis Oncology, Inc. (CLVS) | Marko Kozul, M.D.

### Accelerated Potential for CO-1686; 4Q13 EPS; Reiterate OP; New \$110 PT

Outperform | Market Cap: \$2,657.1M | Price: \$78.38 | Price Target:  $$104.00 \rightarrow $110.00$  | Methodology: DCF analysis

- Bottom Line: CLVS remains a top pick in 2014 and we are increasing our price target (PT) to \$110 (from \$104). Management continues to dynamically advance CO-1686 ("1686") with further accelerated approval potential (i.e., 2H15) driven by an "expanded expansion" Phase II T790M+ strategy. We continue to model potential 1686 approval later in 2016 based on TIGER-2/3 trials, recognizing our valuation would increase if approval came earlier. We believe the 1686 ELCC 3.27.14 presentation will be a very important near-term catalyst that could also de-risk the front-line (FL) opportunity based on the potential for longer duration of response (DoR) trends in the data. Finally, "striking activity" in the first two pancreatic patients dosed with Rucaparib leads us to now include low probability of success (POS) pancreatic revenues in our model. We reiterate our Outperform (OP) rating.
- An "expanded expansion" strategy could lead to T790M+ approval faster than if it is based on TIGER-2/3 trials.
- The upcoming ELCC 3.27.14 1686 presentation could be a major near-term key catalyst (see our
- Rucaparib's early treatment benefit in a few pancreatic patients leads us to now include low POS revenues in our model.
- 2014E EPS/REV: (\$3.61) / 0.0 → (\$3.81) / 0.0

• **2015E EPS/REV:** (\$4.35) / 0.0 → (\$4.40) / \$7.0 *GAAP EPS presented.* 

### Catamaran Corp. (CTRX) | David Larsen, CFA

### **CTRX Making Appropriate Long-term Investments - Maintain OP**

Outperform | Market Cap: \$10,688.4M | Price: \$51.81 | Price Target:  $$68.00 \rightarrow $58.00$  | Methodology:  $\sim 22x$  2015E EPS

- Bottom Line: While we view CTRX's 97-98% customer renewal rate for 2014 favorably, the company is investing \$15-25M in 2014 to improve service levels, which will help drive client retention, up-sell and new wins going forward. Cigna is transitioning ahead of expectations, and we now expect ~\$4B of total Cigna (OP) revenue to hit in 2014. While 2014 EPS guidance is below expectations, it represents +18% y/y growth after normalizing the tax rate. We view 2014 as a transition year, but believe there could be upside from HC Reform, Medicaid expansion, HCIT to PBM conversion or new acquisitions, which aren't currently in guidance. Early commentary around the 2015 selling season suggests an active year and we believe CTRX is well positioned to compete in the health plan and large employer market. Maintain OP.
- · CTRX is investing in higher service levels.
- · Cigna transitioning ahead of plan.
- · Long-term drivers intact.
- · Model update.
- 2014E EPS/REV: \$2.50 / \$19,931.6  $\rightarrow$  \$2.20 / \$20,569.6
- 2015E EPS/REV:  $\$3.00 / \$24,192.4 \rightarrow \$2.65 / \$24,091.8$

Revenue are in millions of U.S. dollars. Adjusted EPS exclude acquisition amortization.

### Hyperion Therapeutics, Inc. (HPTX) | Joseph P. Schwartz

#### 4Q Recap – Strong Ravicti Launch Continues in UCD, as HE Plans Advance

Outperform | Market Cap: \$679.5M | Price: \$31.75 | Price Target:  $$32.00 \rightarrow $39.00$  | Methodology: DCF analysis

- Bottom Line: 4Q EPS of \$0.02 was below our estimate of \$0.07 and consensus of \$0.03, but represented the second straight quarter the base business was profitable, even as the company executes a plan to expand into a larger orphan market. Total revenue was \$18.6MM versus our estimate of \$16.5MM and consensus of \$15.8MM. We are updating our model to reflect 4Q results and 2014 expense guidance. Reiterate Outperform, raising PT to \$39 from \$32 previously.
- Ravicti sales were \$14.3MM versus our estimate of \$12MM and consensus of \$12MM.
- All metrics highlight solid execution in the urea cycle disorder (UCD) business.
- HPTX continues to make progress on the clinical development front for Ravicti in hepatic encephalopathy (HE).
- 2014E EPS/REV:  $\$0.38 / \$83.0 \rightarrow (\$0.20) / \$83.0$

Revenues in millions. Diluted EPS.

### HeartWare International, Inc. (HTWR) | Danielle Antalffy

### 2014 Outlook: A Catalyst Rich Year as Focus Shifts to the Pipeline

Outperform | Market Cap: \$1,586.1M | Price: \$95.55 | Price Target: \$120.00 | Methodology: ~7x EV/ '15E sales of \$272.2M

• Bottom Line: HTWR shares declined ~5% yesterday, likely due to: (1) Management's guidance that the next-gen MVAD isn't likely to begin U.S. pivotal trials until 2015, potentially ~6 months behind THOR (OP); and (2) slightly slower U.S. DT supplemental cohort enrollment, now expected to complete in 1Q15 vs. year-end 2014 previously. But we view the sell-off as overdone here as 2014 is setting up nicely from a catalyst perspective. Beyond MVAD FIM expected in March, at ISHLT in early April HTWR also expects data to be presented from the initial cohort of the U.S. BTT Post-Approval Study. And as we move through 2014, we remain optimistic that: (1) U.S. BTT market growth will

stabilize in the mid-single-digits with HTWR gaining modest incremental share; (2) robust OUS growth in the mid-to-high teens will continue with HTWR maintaining majority share; and (3) HTWR could potentially exceed expectations and complete enrollment of its DT supplemental cohort by year-end.

- · Reiterate OP; We're Buyers Here.
- · Making Progress Against Upcoming Clinical Milestones.
- Estimate Changes Incorporate CircuLite Acquisition.
- **2014E EPS/REV:** (\$1.42) / \$264.5  $\rightarrow$  (\$2.47) / \$258.5

Revenues in millions

Mylan, Inc. (MYL) | Jason M. Gerberry, JD

### 2014 Guidance Points to Solid Fundamentals; Raising Estimates & Remain OP

Outperform | Market Cap: \$22,237.9M | Price: \$56.27 | Price Target:  $$50.00 \rightarrow $65.00$  | Methodology:  $\sim 15.5 \times 2015 EPS$ 

- Bottom Line: We are raising our MYL estimates and price target following an in-line 4Q and better-than-expected mgmt guidance for 2014. Our top takeaways from today's call were: (1) mgmt's high level of confidence regarding g-Copaxone; (2) mgmt's '14 outlook for EpiPen was highly bullish suggesting stable market share and an ability to continue to take price increases; & (3) mgmt expects gross margin to expand by 200 bps, which we attribute to the mix benefit from g-Copaxone and EpiPen. We are raising our '14E non-GAAP EPS to \$3.49, which is at the high end of mgmt's guidance range of \$3.25-3.60. We are also raising our '15E/'16E EPS by ~10% based on our assumption that g-Copaxone and EpiPen will be durable contributors. We are raising our price target to \$65/shr (from \$50), based on ~15.5x our 2015E EPS of \$4.12.
- EpiPen continues to be an engine for growth; no updates on life cycle extension.
- · We are raising our g-Copaxone ests based on mgmt's conviction regarding its application.
- Mgmt bullish on M&A, but deals not baked into our numbers.
- **2014E EPS/REV:** \$3.36 / \$7,622.0 → \$3.49 / \$8,048.0

Revenues presented in \$M. Adjusted diluted EPS.

NxStage Medical, Inc. (NXTM) | Danielle Antalffy

2014 Outlook: Drivers in Place to Accelerate HHD Growth

Outperform | Market Cap: \$848.9M | Price: \$13.94 | Price Target:  $\$15.00 \rightarrow \$18.00$  | Methodology:  $\sim 3.25x$  EV/2015E sales

- Bottom Line: After a tough 2013, NXTM seems well-positioned to continue 4Q momentum and deliver accelerating Home Hemodialysis (HHD) sales in 2014 -- potentially toward management's 15% growth goal. NXTM has implemented a number of initiatives to support this level of improving growth, including: (1) Recent and upcoming new product approvals, including the high-flow System One S and Nx2Me Connected Health Platform; (2) DTC programs in 50 regions in 2013 and expanding to 150 in 2014; (3) ramping Clinical Centers of Excellence that drive adoption through raising awareness and providing access; and longer term (4) more clinical data to help drive increasing awareness about the clinical and economic benefits of home hemodialysis therapy versus in-center. While barriers to adoption remain high, NXTM is working to gradually break these down and drive HHD adoption higher from current penetration levels of just ~1.6%. We believe more consistent execution throughout 2014 can help restore management credibility and continue to push the shares higher. Reiterate OP rating and raise price target to \$18 from \$15.
- Cash Position Seems Sufficient to Absorb Ramping Clinical Centers of Excellence.
- 2014 Outlook: In-Center Sales Push Estimates Slightly Lower.
- **2014E EPS/REV:** (\$0.27) / \$292.0 → (\$0.40) / \$285.1 *Revenues in \$MM.*

### Insulet Corporation (PODD) | Danielle Antalffy

#### 2014 Outlook: A 4Q Beat Reinforces Confidence in Sustainable Growth Profile

Outperform | Market Cap: \$2,474.6M | Price: \$45.24 | Price Target: \$55.00 | Methodology: ~8x EV/ &#39:15E sales of \$366M

- Bottom Line: Last night after market close, PODD delivered a solid 4Q13 sales beat -- with sales of \$68.5M (+18.5%, + over 25% ex. Neighborhood Diabetes) vs. us an the Street at \$67.9M and \$67.8M respectively. To us, this beat is notable beacuse -- while PODD has always consistently delivered results safely within the company's guidance range -- this represents the first quarter of sales outperformance relative to Street expectations since 1Q12. The three key takeaways from management's commentary on the post-earnings conference call were: (1) PODD exited the year with strong momentum across all metrics, but notably new patient adds and overall OmniPodd growth, and that momentum is set to continue into 2014; (2) PODD is now running at significantly increased manufacturing capacity, producing 2.5M OmniPods in Q4 -- up 50% vs. Q3 production levels; and (3) PODD achieved operating profitability for the first time ever in 4Q13. Management also highlighted the OmniPod's potential as a platform technology, noting significant sales potential in 2015 for the recently-signed AMGN partnership. All of this leaves us encouraged that PODD can drive sustainable sales growth at or near 20% over the next few years while now achieving profitability by 2H14.
- · Reiterate OP; Price Target of \$55.
- · Strong Momentum Heading into 2014.
- · Outlook Basically Unchanged.
- 2014E EPS/REV: \$0.07 / \$300.4 → \$0.02 / \$301.2

Revenues in MMs.

### Salix Pharmaceuticals, Ltd. (SLXP) | Jason M. Gerberry, JD

### 4Q13: Another Beat & Raise, But Benefit on Tax Appears Limited to Near Term

Outperform | Market Cap: \$7,579.0M | Price: \$109.84 | Price Target:  $$115.00 \rightarrow $126.00$  | Methodology: 17x 2015E EPS

- Bottom Line: Following yesterday's market close, SLXP reported another beat & raise quarter with EPS and sales coming in \$0.08 & ~\$9M above Street estimates. respectively. Strength in the quarter was driven by higher purgatives sales and a change in the booked non-GAAP tax rate (now using the cash tax rate), which was partly offset by weaker GM (mix shift). We are encouraged by continued strength in Apriso, potential upside to SNTS synergies, and continued efforts to strengthen Xifaxan IP ahead of TARGET-3 label expansion data in 1Q15, but we note that a '14 guidance raise appears to be largely predicated on lower cash tax related to the SNTS acquisition. We remain OP-rated and raise our price target to \$126/share (from \$115), based on 17x '15E EPS of \$7.42.
- Apriso appears poised for another strong year in '14.
- · Better-than-expected '14 guidance driven by change in booked non-GAAP tax rate offset by mix shift.
- · Mgmt expects Relistor Adcom "near end of May."
- **2014E EPS/REV:**  $\$6.09 / \$1,615.0 \rightarrow \$6.59 / \$1,610.0$
- 2015E EPS/REV: \$7.64 / \$1,963.0 → \$7.42 / \$1,965.0 Revenue in \$MM. Non-GAAP EPS (ex non-cash depreciation, amortization, stock-based comp, convertible debt expense).

Biopharma | Howard Liang, Ph.D.

### Takeaways from our Global Healthcare Conference - The Hepatitis C Panel

Outperform: GILD, JNJ

• Bottom Line: We hosted a discussion at Leerink Global Healthcare Conference with a diverse panel consisting of two MEDACorp hepatology key opinion leaders (KOLs) including a transplant specialist, an infectious disease doctor and a payor representative to gauge the market dynamics following initial approval of Sovaldi (GILD [OP]) and Olysio (JNJ [OP] / Medivir), as well as upcoming IFN-free all-oral regimens. Our panel discussion supports

rapid HCV market expansion and suggests initial robust launch for Sovaldi likely to continue into Sovaldi/ledipasvir launch (anticipated later in 2014), while pricing and access could be a key determinant of future market dynamics. In addition, HCV screening appears to have seen limited adoption so far, and this will likely be an important trend to watch for all HCV players.

- Reimbursement has been a significant barrier for Sovaldi.
- Published 2.27.14.

### Alkermes (ALKS) | Michael Schmidt, Ph.D.

### Top- and Bottom-line Beat in 4Q13; Guidance Reflective of Pipeline Growth

Outperform | Market Cap: \$8,043.5M | Price: \$52.71 | Price Target:  $\$58.00 \rightarrow \$60.00$  | Methodology: Sum of the parts DCF

- Bottom Line: ALKS reported 4Q13 earnings and beat top-line and bottom-line Street estimates driven by solid Manufacturing and Royalty revenue. ALKS provided 2014 expense guidance that surprised the Street, but confirmed our expectations for higher operating expenses in 2014 due to increased clinical activities and preparation for the potential aripiprazole lauroxil launch. We continue to believe positive pipeline news flow and potential partnerships will be sources of upside with four data readouts expected over next 12 months. Raising adjusted 2014 EPS estimate to account for better 2014 revenue guidance than we had expected, partially offset by an increase in our GAAP SG&A estimate. Maintain Outperform rating, PT now \$60 from \$58.
- Top-line and bottom line beat in 4Q13 driven by solid Manufacturing and Royalty business.
- 2014 guidance reflective of continued investment into the pipeline, while maintaining cash flow generation.
- Four key data readouts over the next 12 months plus a potential partnership should generate additional upside.
- · Published 2.27.14, intra-day.
- 2014E EPS/REV: \$0.45 / \$587.8 → \$0.50 / \$596.1
- 2015E EPS/REV: \$0.28 / \$694.6 → \$0.43 / \$707.3

  2012: fiscal year 12 months ending March 31, 2013, 2013: Stub year 9-months ending Dec. 31, 2013, 2014: 12-months ending Dec. 31, 2014.

### Ophthotech Corporation (OPHT) | Joseph P. Schwartz

### 4Q Recap: Extensive Development Programs for Fovista and Zimura Underway

Outperform | Market Cap: \$1,288.4M | Price: \$36.09 | Price Target:  $$37.00 \rightarrow $45.00$  | Methodology: DCF, 12% WACC, 2% Terminal Growth Rate

- Bottom Line: This morning OPHT provided an update on the ongoing Fovista Phase III and its plans to develop Zimura in both wet and dry AMD. OPHT will provide more details on the underlying science and its development strategy at an R&D day on March 7th. Overall 10 clinical trials are ongoing or expected to be initiated in 2014, leading to data beginning to emerge in 2015. We are updating our model to reflect 4Q13 results. Reiterate OP on OPHT, raising PT to \$45 from \$37 to reflect Fovista and Zimura pipeline advancement.
- US dosing and enrollment were initiated in August 2013 in the two Lucentis combination studies in the Fovista Ph III programs after the protocols for the 3 studies were submitted to the FDA in July.
- OPHT received written confirmation from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) indicating that it is in agreement with the company's proposed Phase III trial design and dosing regimen.
- In addition to the strong safety and efficacy signals seen to date, which should de-risk the Phase III program, we believe that OPHT shares remain attractive
- OPHT is advancing development of Zimura (C5 inhibitor) in both dry and wet AMD.
- · Published 2.27.14, intra-day.
- 2014E EPS/REV: ( 2.78) /  $0.0 \rightarrow$  ( 2.89) / 0.0

2Q13 Revenue and EPS are 1H13 Actuals, since quarterly numbers not provided in S1. GAAP EPS presented.

### Sarepta Therapeutics, Inc. (SRPT) | Joseph P. Schwartz

### 4Q13 EPS: 1Q14 FDA Meeting Could Answer Some Outstanding Questions

Market Perform | Market Cap: \$1,216.3M | Price: \$29.45 | Price Target:  $$17.00 \rightarrow $31.00$  | Methodology: sum-of-the-parts DCF, 12% discount rate, 2.5% terminal growth rate

- Bottom Line: This morning SRPT reported 4Q13 EPS of (\$0.23) versus our estimate of (\$0.59) as the company spent more than we had modeled but unexpectedly recognized a ~\$24MM non-cash gain on the change in the valuation of outstanding warrants. The company continues to work with the FDA to firm up an eteplirsen confirmatory trail design and expects to meet with the Agency in again in March. We are increasing our valuation based on a positive readthrough from recently presented RNA (OP) drisapersen data and SRPT's expansion of its platform, continued discussion with the FDA and advancement of its other DMD exon-skipping therapeutics. We updated our model to reflect 4Q13 results. Reiterate MP increasing PT to \$31 from \$17.
- Recent FDA inquiries may be more encouraging for eteplirsen's ultimate approval prospects,
- SRPT is now most focused on working with the Agency to agree on a confirmatory trial design,
- Our updated valuation assumes a 20% probability of accelerated approval and now attributes more value to SRPT's exon skipping pipeline,
- Published 2.27.14, intra-day.
- **2014E EPS/REV:** (\$3.64) / \$20.0 → (\$3.68) / \$20.0

Revenues in millions; GAAP EPS.

### Healthcare |

### Top Catalysts for 1H14 - Catalyst Tracker - 2.26.14

Outperform: A, ACHN, ACOR, ACT, AEGR, AET, AGIO, ALKS, ALNY, ALXN, AMAG, AMBI, ANTH, ARNA, ARQL, ARRY, ATRC, AUXL, AZN, BAX, BIIB, BMRN, BMY, CBST, CELG, CEMP, CI, CLDX, CLVS, CNC, COV, CVS, CYNO, CYTK, DRNA, ELOS, ENDP, ENTA, EPZM, ESRX, EW, FRX, GILD, GMED, GWPH, HOLX, HTWR, HUM, IDIX, IMGN, INSM, JAZZ, JNJ, KBIO, KPTI, KYTH, LLY, LMNX, MDCO, MDVN, MGNX, MNTA, MRTX, MYL, NBIX, NPSP, NSTG, NUVA, NVS, NXTM, OGXI, ONTX, OREX, PBYI, REGN, RIGL, RNA, RPTP, SGEN, SHPG, SLXP, SNSS, SPHS, STJ, SYK, THOR, THRX, TMO, TSRO, VCYT, VRTX, VVUS, WLP, XNCR, XNPT, ZGNX, ZLTQ, ZMH | Market Perform: AGN, AKRX, AMGN, AMRN, ARIA, BCR, BDX, BSX, DNDN, DXCM, DYAX, ECYT, EXEL, FOLD, HNT, HSP, IPXL, IRWD, ISIS, ISRG, ITMN, MDT, MNK, MOH, MRK, MYGN, PCYC, PFE, PRGO, QSII, SNY, SRPT, TEVA, TFX, UAM, UNH, UTHR, VOLC, WCG

- Bottom Line: Binary events in healthcare can be key determinants of investor performance. We provide a roadmap for the biggest binary events in our universe for 1H14. For Managed Care, final Medicare Advantage rates on April 7 are key to HUM, and also highly relevant for UNH, WCG, UAM, CI, AET, HNT, WLP. In large cap biopharma, we highlight BMY, VRTX. Among mid caps, ALKS, SLXP. And in small caps, CLVS, CYTK, EXEL, INSM, ITMN, MNTA, OGXI. Our sortable Excel sheet has timelines and stock impacts, both Up/Down (calendar).
- For Managed Care, final Medicare Advantage rates on April 7 are key to HUM (OP), which we see as the preferred way to play the undervalued MA growth story and also highly relevant for UNH (MP), WCG (MP), UAM (MP), CI (OP), AET (OP), HNT (MP), WLP (OP).
- In large pharma, 2014 is a pivotal year in immuno-oncology (IO) led by top pick BMY.
- In Biotech, VRTX (OP) Ph III cystic fibrosis (CF) data in mid-2014 could be a binary event associated with the one of biggest market cap changes in recent history of biotech.
- For ALKS (OP), we place an 80% probability of positive Phase III aripiprazole lauroxil (schizophrenia) data in 1H14, setting the stage for potential launch April 2015.
- For SLXP (OP), we see 80% probability of success for mid-2014 Xifaxin retreatment study, Up/down +5-10%/-20%.
- For CLVS (OP), we expect preliminary Ph I data at ELCC, 3/26-29, on CO-1686 in NSCLC.
- For CYTK (OP), we see Up/down of +150%/-60% for Tirasemtiv Ph IIb top line results in ALS.
- Based on due diligence with MEDACorp KOLs, we place a 50% probability of success on MP-rated EXEL's COMET-1 trial, top-line data due in 2014.

- We est. INSM (OP) has Up/down of +/-60% upon 1Q14 release of top-line Ph II TARGET data in NTM (non-tuberculous mycobacteria).
- OP-rated MNTA's launch of g-Copaxone expected upon patent expiry on 5/24/14 could lead to as much as \$10-15 upside to our DCF, depending on 3 scenarios.
- By mid 2014, we expect OGXI (OP)/ TEVA (MP) Ph III data in prostate cancer.
- Published 2.26.14.

### **Research Previews**

### February 28 - Earnings - Before the Open

### AUXL - OP - Joe Schwartz

Close: \$30.84,Mkt Cap: \$914M

4Q13E Leerink EPS/Rev: \$0.31 / \$110.5M
4Q13E Street EPS/Rev: \$0.21 / \$111.8M
2014E Leerink EPS/Rev: \$1.33 / \$457.5M
2014E Street EPS/Rev: \$1.18 / \$489.0M

· CC: 9:00am EST; Dial-in: 877 546 5018, Access Code: Auxilium

#### DYAX - OP - Joe Schwartz

Close: \$10.20,Mkt Cap: \$346M

4Q13E Leerink EPS/Rev: -\$0.03 / \$16.5M
4Q13E Street EPS/Rev: -\$0.04 / \$15.5M
2014E Leerink EPS/Rev: -\$0.23 / \$59.6M
2014E Street EPS/Rev: -\$0.21 / \$63.5M

· CC: 8:30am EST; Dial-in: 877 674-2415, Access Code: --

### **ENDP – OP – Jason Gerberry**

Close: \$81.49,Mkt Cap: \$2,906M

4Q13E Leerink EPS/Rev: \$0.90 / \$626.0M
4Q13E Street EPS/Rev: \$0.92 / \$620.2M
2014E Leerink EPS/Rev: \$3.43 / \$2,309.0M
2014E Street EPS/Rev: \$3.88 / \$2,537.3M

· CC: 8:30am EST; Dial-in: 866 515-2910, Access Code: 79916686

#### ISIS - MP - Joe Schwartz

Close: \$56.37,Mkt Cap: \$1,059M

4Q13E Leerink EPS/Rev: -\$0.16 / \$35.0M
4Q13E Street EPS/Rev: -\$0.21 / \$29.3M
2014E Leerink EPS/Rev: -\$0.81 / \$159.6M
2014E Street EPS/Rev: -\$0.85 / \$103.2M

· CC: 11:30am EST; Dial-in: 866 652-5200, Access Code: --

### March 3 – Earnings – Before the Open

### AKRX - MP - Jason Gerberry

Close: \$26.80,Mkt Cap: \$1,280M

- $\cdot$  4Q13E Leerink EPS/Rev: \$0.14 / \$87.0M
- · 4Q13E Street EPS/Rev: \$0.14 / \$83.5M
- · Recently, mgmt was bullish on the strategic fit of acquisition HITK; the deal is expected to close in March.
- · 2014E Leerink EPS/Rev: \$0.72 / \$380M (stand-alone)

- · 2014E Street EPS/Rev: \$0.91 / \$626.4M
- · CC: 10:00am EST; Dial-in: 888 461-2024, Access Code: 3623707

#### BMRN - OP - Joe Schwartz

Close: \$83.28,Mkt Cap: \$10,093M

- · 4Q13E Leerink EPS/Rev: -\$0.47 / \$144.7M
- · 4Q13E Street EPS/Rev: -\$0.43 / \$145.4M
- · On 2/18, BMRN reported 4Q13 preliminary revenues of \$146.9M, which beat our and consensus estimates of \$140.1M and \$143.6M.
- · Vimizim's anticipated net price (\$380K), 2014 top-line guidance of \$650-\$680M, and new LT tax guidance of ~20% were better than our estimates.
- · 2014E Leerink EPS/Rev: -\$1.94 / \$670.0M
- · 2014E Street EPS/Rev: -\$1.78 / \$671.1M
- · CC: 8:00am EST; Dial-in: 877 303-6313, Access Code: 59525165

#### CLDX - OP - Howard Liang

Close: \$31.59,Mkt Cap: \$432M

- · 4Q13E Leerink EPS/Rev: -\$0.29 / \$0.4M
- · 4Q13E Street EPS/Rev: -\$0.29 / \$0.3M
- · More details about combination strategies for CDX-1127 will be provided on the call.
- · 2014E Leerink EPS/Rev: -\$1.11 / \$1.7M
- · 2014E Street EPS/Rev: -\$1.13 / \$0.7M
- · CC: 8:30am EST; Dial-in: 866 743-9666, Access Code: 39634623

#### **DNDN - MP - Howard Liang**

Close: \$2.97,Mkt Cap: \$817M

- · 4Q13E Leerink EPS/Rev: -\$0.36/ \$70.0M
- · 4Q13E Street EPS/Rev: -\$0.37 / \$75.3M
- · 2014E Leerink EPS/Rev: -\$1.04 / \$285.6M
- · 2014E Street EPS/Rev: -\$0.97 / \$309.9M
- · CC: 9:00am EST; Dial-in: 877 548-9590, Access Code: 57711925

### March 3 - Earnings - After the Close

#### FOLD - MP - Joe Schwartz

Close: \$2.65,Mkt Cap: \$133M

- · 4Q13E Leerink EPS/Rev: -\$0.37 / \$0.0M
- · 4Q13E Street EPS/Rev: -\$0.31 / \$0.0M
- · 2014E Leerink EPS/Rev: -\$1.12 / \$0.0M
- · 2014E Street EPS/Rev: -\$1.23 / \$0.0M
- · CC: 5:00pm EST; Dial-in: 877 303-5859, Access Code: --

### **GEVA - MP - Howard Liang**

Close: \$118.42,Mkt Cap: \$1,133M

- · 4Q13E Leerink EPS/Rev: -\$0.93 / \$4.5M
- · 4Q13E Street EPS/Rev: -\$1.02 / \$2.1M
- · 2014E Leerink EPS/Rev: -\$2.96 / \$13.0M
- · 2014E Street EPS/Rev: -\$4.31 / \$6.6M
- · CC: 4:30pm EST; Dial-in: 877 445-4603, Access Code: --

### NUVA - OP - Rich Newitter

### Close: \$37.10,Mkt Cap: \$675M

- · 4Q13E Leerink EPS/Rev: \$0.34 / \$189.2M
- · 4Q13E Street EPS/Rev: \$0.33 / \$184.8M
- · In mid-Jan., NUVA preannounced 4Q sales of \$189M (+14% y/y), which came in above our and the consensus' \$176M estimate.
- · Management's \$725M 2014 sales outlook reflects +6.1% growth off a now higher 2013 base.
- · 2014E Leerink EPS/Rev: Reference Error / \$729.3M
- · 2014E Street EPS/Rev: \$1.28 / \$722.7M
- · CC: 5:30pm EST; Dial-in:877 407-9039, Access Code: --

### March 4 - Earnings - Before the Open

#### GTXI - MP - Howard Liang

Close: \$1.78,Mkt Cap: \$264M

- · 4Q13E Leerink EPS/Rev: -\$0.17 / \$0.0M
- · 4Q13E Street EPS/Rev: -\$0.16 / \$0.0M
- · 2014E Leerink EPS/Rev: -\$0.28 / \$0.0M
- · 2014E Street EPS/Rev: -\$0.41 / \$0.0M
- · CC: 9:00am EST; Dial-in: 877 280-4957, Access Code: 86747366

#### March 5 - Earnings - Before the Open

### ARQL - OP - Howard Liang

Close: \$2.28,Mkt Cap: \$174M

- · 4Q13E Leerink EPS/Rev: -\$0.14 / \$2.0M
- · 4Q13E Street EPS/Rev: -\$0.13 / \$2.2M
- · 2014E Leerink EPS/Rev: -\$0.48 / \$14.2M
- · 2014E Street EPS/Rev: -\$0.42 / \$12.3M
- · CC: 9:00am EST; Dial-in: 877 548-9590, Access Code: 57711925

#### **KPTI – OP – Michael Schmidt**

Close: \$41.06,Mkt Cap: \$1,208M

- · 4Q13E Leerink EPS/Rev: -\$0.33 / \$0.2M
- · 4Q13E Street EPS/Rev: -\$0.34 / \$0.1M
- · 2014E Leerink EPS/Rev: -\$1.33 / \$1.0M
- · 2014E Street EPS/Rev: -\$1.56 / \$0.6M
- · CC: 8:00am EST; Dial-in: 855 437-4406, Access Code: 6470393

#### March 5 - Earnings - After the Close

### ZGNX - OP - Jason Gerberry

Close: \$4.56,Mkt Cap: \$134M

- · 4Q13E Leerink EPS/Rev: -\$0.11 / \$8.0M
- · 4Q13E Street EPS/Rev: -\$0.10 / \$8.0M
- · 2014E Leerink EPS/Rev: -\$0.37 / \$65.3M
- · 2014E Street EPS/Rev: -\$0.29 / \$76.3M
- · CC: 4:30pm EST; Dial-in: 877 474-9506, Access Code: 23384178

### March 7 - OPHT Investor Meeting - NYC

### **OPHT - OP - Joe Schwartz**

#### Close: \$34.98,Mkt Cap: \$1,188M

- · OPHT will provide details on the underlying science and its development strategy for the ongoing Fovista Phase III and its plans to develop Zimura in both wet and dry AMD.
- · Overall 10 clinical trials are ongoing or expected to be initiated in 2014, leading to data beginning to emerge in 2015.

### **Previous Notes**

### February 27

Life Science Tools and Diagnostics/Diligence, Study Support Large Market Opportunity, Dan Leonard Specialty Pharmaceuticals/APS Preview: Specialist's Views on Key Spec Pharma Regulatory Controversies, Jason Gerberry

AZN/Oncology and Resp. Pipeline Fuel 'Dark Horse' Heading into ATS and ASCO/Outperform, Seamus Fernandez BMY/Updating Forecasts to Better Reflect HCV Oppt'y & More IO Tumors/Outperform, Seamus Fernandez DYAX/Downgrading to Market Perform Due to Lack of Near-Term Catalysts/Market Perform, Joe Schwartz GMED/A Strong Quarter for Margins; Sales/Profit Drivers In Place for '14/Outperform, Rich Newitter MDAS/4Q:13 Results Beat, 2014 Guidance Brackets Expectations/Market Perform, Dave Larsen MRK/Raising PT to \$57/Share on Improving Near-Term Pipeline Prospects/Market Perform, Seamus Fernandez NSTG/2014 Catalysts Remain on Track/Outperform, Dan Leonard XLRN/Catalyst-rich 2014 & Dalantercept Soon Moving into HCC; Reiterate OP/Outperform, Marko Kozul

**Updated Models:** AEGR - Model, ARIA - Model, AZN - Model, BMY - Model, DYAX - Model, GMED - Model, MDAS - Model, MRK - Model, NSTG - Model, XLRN - Model, ZLTQ - Model

### Leerink Partners 2014 Global Healthcare Conference

Medical Supplies and Devices/Takeaways from 2014 Leerink Global Healthcare Conference, Antalffy/Newitter Pharmaceuticals / Major/Takeaways from Our 2014 Global Healthcare Conference, Seamus Fernandez Biopharma/Takeaways from Our 2014 Global Healthcare Conference: Immuno-oncology, Fernandez/Liang/Schmidt Biopharma/Takeaways from our Global Healthcare Conference - The Hepatitis C Panel, Howard Liang Biotechnology/Takeaways from our 2014 Global Healthcare Conference, Michael Schmidt Biotechnology/Takeaways from our 2014 Global Healthcare Conference, Joe Schwartz Biotechnology/Takeaways from our 2014 Global Healthcare Conference, Marko Kozul Specialty Pharmaceuticals/Takeaways from our 2014 Global Healthcare Conference, Jason Gerberry Life Science Tools and Diagnostics/Takeaways from our 2014 Global Healthcare Conference: Clinical NGS, Dan Leonard

HCIT/Takeaways from our 2014 Global Healthcare Conference, Dave Larsen

### **Deep Dive Analyses**

BMRN/Deep Dive on BMN111: Compelling Logic/Early Efficacy Warrant Investor Attention/Outperform, Joseph Schwartz

DYAX/Deep Dive on DX2930: Value Warranted for Novel HAE Monoclonal Antibody/Outperform, Joseph Schwartz SHPG/Deep Dive on Ph. III Pipe; Feedback on Lifitegrast Surprisingly Constructive/Outperform, Jason Gerberry

#### **Recent Surveys**

Managed Care/Broker Survey Points to Stable Market for 2014, Shifts to Exchanges in 2015, Ana Gupte KYTH/Survey Reactions to ATX-101 Validate Expectations for Successful 2015 Launch/Outperform, Seamus Fernandez

Medical Supplies and Devices/Hemophilia Physician Survey: BAX Share Loss Likely in Line with Estimates, Danielle Antalffy

AGN/4Q:13 Cosmetic Survey Highlights Stable Growth Trends, Cont'd AGN Strength/Market Perform, Seamus Fernandez

Medical Devices-Orthopedics/Hip/Knee Survey Highlights 4Q Seasonality & Possible Positive Sign for 1H14, Richard Newitter

EW/TAVR Survey Suggests Weak 4Q13, Supports Long-Term Market Potential/Outperform, Danielle Antalffy Pharmaceuticals/ Major/MEDACorp Diabetes Survey: Solid SGLT2 Prospects & A Tough Januvia (MRK) Outlook, Seamus Fernandez

Life Science Tools and Diagnostics/Survey Offers Mixed Feedback on Forthcoming Sequencing Demand, Dan Leonard

#### **White Papers**

Specialty Pharmaceuticals/ Generics/Future of Respiratory Generics - Next Major Generic Product Class, Jason Gerberry, JD

Medical Devices-Orthopedics/The Future Of Growth & Innovation In US Extremities Ortho Reconstruction, Rich Newitter

Biotechnology/Future of Dynamic Multiple Sclerosis Landscape, Marko Kozul, M.D.

Healthcare IT & Distribution/The Future of HCIT Population Health Management, Dave Larsen

Life Science Tools and Diagnostics/The Future of DNA Sequencing, Dan Leonard - Life Science Tools and Diagnostics

Biopharma/Future of Cancer Treatment - Immuno-Oncology, Seamus Fernandez

Biopharma/Future of Orphan Drugs: Neuro Nirvana, Joseph P. Schwartz - Biotechnology

Biopharma/Future of Hepatitis C Treatment, Howard Liang, Ph.D. - Biotechnology

Medical Devices-Cardiology/Future of Atrial Fibrillation, Danielle Antalffy - Medical Devices

# **Upcoming Healthcare Events**

### **Regulatory Events**

3/3: FDA AdCom: Pediatrics

White House schedule to release FY15 budget

### **Medical Meetings**

2/25-3/1: Association of Academic Psychiatrists

2/26-3/2: American Society for Blood and Marrow Transplantation

2/27-3/2: American Glaucoma Society

2/28-3/4: American Academy of Allergy, Asthma, and Immunology

3/2-6: Pittsburgh Conference on Analytical Chemistry and Applied Spectroscopy

3/6-7: Future of Genomic Medicine

**3/8-9**: American Academy of Pain Medicine **3/6-10**: European Congress of Radiology

### **Corporate Events**

3/5: WAG monthly sales

3/6: A Investor meeting

3/7: OPHT Investor meeting

3/7: MDCO Investor meeting

# Disclosures Appendix Analyst Certification

Each analyst certifies that the views expressed in this report accurately reflect their views and that no part of their compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

	Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13 IB Serv./Past 12 Mos.			
Rating	Count	Percent	Count	Percent
BUY [OP] HOLD [MP]	118 65	64.50 35.50	30 2	25.00 3.00
SELL [UP]	0	0.00	0	0.00

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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.

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