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Est. 1842
London
New York
San Francisco
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EQUITY RESEARCH
Company Report

June 21, 2016

# OPKO Health, Inc. (OPK - $ 9.19)

*Healthcare/Biotechnology*

## Rayaldee Received Approval Four Months Ahead of the Scheduled Oct. 22 PDUFA Date

OPK announced this morning that the FDA has approved Rayaldee in secondary hyperparathyroidism (SHPT) in stage 3 or 4 chronic kidney disease (CKD) patients with vitamin D insufficiency. The approval is substantially ahead of the scheduled PDUFA date of Oct. 22, 2016.

- **Details.** OPK reported this morning that the FDA has approved Rayaldee in secondary SHPT in adult with stage 3 or 4 chronic kidney disease (CKD) patients with vitamin D insufficiency (serum 25-hydroxyvitamin D <30 ng/ml). The actual approval date was June 17, 2016 according to the information posted at the FDA website; and it also indicated that there are no therapeutic equivalents to Rayaldee. OPK management recently reported that the FDA has decided that it was unnecessary to inspect Catalent's plant, and we therefore believe a potential Rayaldee approval ahead of its scheduled PDUFA date (Oct. 22, 2016) is possible. OPK reported this morning to potentially launch Rayaldee in 2H16. Further, OPK revealed during recent Analyst day that a plan for a Phase III study that evaluates Rayaldee in secondary hyperparathyroidism (SHPT) in end stage renal disease (ESRD) patients with vitamin D insufficiency is under development with trial potentially to begin in 2017.
- **Implications.** Even though our belief that an earlier Rayaldee approval could be imminent, today's news remains a positive surprise given the fast response by the agency. We view the news bears two positive aspects: it removes an overhang for OPK shares for the possible uncertainty of Rayaldee approval. In addition, an earlier than expected approval provides more time for OPK to start discussions with third party payers to place Rayaldee in the formularies and the reimbursement negotiations. As such, it could have a positive impact on possible faster sales ramp up, in our opinion, by narrowing the time between former product launch and establishment of reimbursement. OPK also reported at the Analyst day the recent hiring of three senior VPs (Sales, Marketing and Market Access, and Medical Science Liaisons) for the Rayaldee commercial team.
- **Action.** We are reiterating our Buy rating and $22 price to reflect our bullish view on progress in OPK's multiple drivers in place, especially the 4Kscore test, Rayaldee, VARUBI (rolapitant) and hGH-CTP (MOD-4023). Our valuation is based on our DCF analyses.

| | |
|---|---|
| Ticker: | **OPK** |
| Rating: | **Buy** |
| Price Target: | **$ 22.00** |

**Trading Data:**

| | |
|---|---|
| Last Price (06/20/2016) | $ 9.19 |
| 52–Week High (7/24/2015) | $ 17.51 |
| 52–Week Low (1/20/2016) | $ 7.12 |
| Market Cap. (MM) | $ 5,031050 |
| Shares Out. (MM) | 547 |

**Earnings Estimates:** (per share)

| (Dec) | 1Q | 2Q | 3Q | 4Q | FY | P/E |
|---|---|---|---|---|---|---|
| **FY-16E** | -0.02A | -0.04 | -0.01 | -0.01 | -0.08 | NM |
| **FY-15A** | -0.26 | -0.09 | 0.26 | 0.00 | -0.06 | NM |
| **FY-14A** | -0.11 | -0.06 | -0.11 | -0.12 | -0.41 | NM |
| **FY-13A** | -0.11 | -0.01 | -0.17 | -0.04 | -0.32 | NM |

*Source: Laidlaw & Company estimates*

**Yale Jen, Ph.D.**
Managing Director /
Senior Biotechnology Analyst
(212) 953-4978
yjen@laidlawltd.com

## Anticipated milestones in 2016 and beyond

| Product | Indication | Event | Timing | Importance |
|---|---|---|---|---|
| Rayaldee | SHPT in CKD Stage 3-4 patients | Build M&S organization | **2016** | *** |
| | | Product launch | **4Q16** | *** |
| | | Potential include in for formulary of healthcare plans | **2017** | **** |
| | | Potential EMEA filing | **1H17** | *** |
| | | Potential EU approval | **1H18** | **** |
| | SHPT in ESRD patients | Start Phase III study | **2017** | *** |
| Rolapitant (VARUBI) | Chemotherapy -induced nausea and vomiting (CINV) | Potential approval of the IV version (PDUFA date) | **Jan. 11, 2017** | **** |
| hGH-CTP (MOD-4023) | hGH deficiency | Report of Phase III study top-line results | **4Q16** | **** |
| | | Potential product approval for adult hGH deficiency | **2H17** | *** |
| | | Potential commencement of pediatric Phase III study | **2H16** | *** |
| | | Report of Phase III pediatric study top-line results | **2018** | **** |
| 4Kscore test | Prostate cancer diagnostics | CMS reimbursement decisions | **2016** | **** |
| | | Potential private payer reimbursement decision | **2016 - 2017** | **** |
| Claros 1 testosterone test | POC testosterone test | Potential 510(k) filing | **2H16** | *** |
| | | Potential approval | **2017** | **** |
| Claros 1 PSA test | POC PSA test | Modular PMA filing | **2H16** | *** |
| | | Potential approval | **2017** | **** |
| Claros 1 vitamin D test | POC vitamin D test | Potential 510(k) filing | **1Q17** | **** |
| | | Potential approval | **2017** | **** |
| MOD-5014 (IV) | Hemophilia A/B with inhibitors | Potentially report Phase I/IIa study top-line results | **4Q16** | **** |
| | | Potentially start Phase II/III study | **1H17** | *** |
| | | Potentially report Phase II/III study results | **2Q18** | **** |
| MOD-5014 (s.c) | | Potentially start Phase I study | **Mid-16** | *** |
| | | Potentially report Phase I study results | **1Q17** | **** |
| | | Potentially start Phase II study | **2H17** | *** |
| | | Potentially report Phase II study results | **Mid-18** | *** |
| MOD-6031 | Obesity | Potentially report Phase I study top-line results | **4Q16** | *** |

***** / ***** Major catalyst event that could impact share price very significantly while *** event is more informative*

*Source: Laidlaw & Company estimates and company presentation.*


Yale Jen, Ph.D.
yjen@laidlawltd.com

## Major risks

**Risks of clinical study failure.** One of the key risks for healthcare product developer is failure of clinical studies that could potentially result in sunk costs in both capital and time lost. OPK currently has several mid- to late stage clinical trials underway. Failures of these studies, especially the Phase III pivotal trials, could have significant negative impact on share value. More specifically, clinical study success of MOD-4023 (hGH-CTP) is very important given it accounts for substantial valuation of OPK share value.

**Regulatory success is important**. Given the company currently has several products (in-house development and partnered) under regulatory agency review, whether to receive positive response and approval could have significant impact on share value. Although clinical study results for the several drugs currently under FDA review are rather robust; it remains possible that the agency may not grant approval or request additional clinical information or studies before considering approval. A scenario of this nature could have significant and immediate negative impact on OPK shareholder value.

**Merger and acquisition risks.** Although acquisition is a faster way to accomplish financial and strategic goals, it bears a number of risks especially post-merger. For example, due to the differences of corporate culture and mentality of operation, there are no assurance a successfully integration can be accomplished immediately.

**Successful reimbursement is critical for commercial success.** Given the high price of medical products, it is important for most patients who will use them only if the diagnostic test or pharmaceutical products are reimbursed by third party payers, such as Medicare or private insurers. There is no certainty that the company's current or future products can be reimbursed by private or public parties. If so, we believe the revenue growth for such drug or diagnostic test could be limited.

**Products may not be approved or reach anticipated sales.** Although OPK's current pipeline products have exhibited the potential to generate positive clinical outcomes from current and future trials; it remains too early to project whether any of these products would be approved by regulatory agencies. Even if the products were to enter the market, sales could be significantly below projections due to the specific product label under approval, physician consensus for prescribing the drug, changes of treatment paradigms, entrance of competitors, and possibly the changes in pricing flexibility and payer reimbursement. A revenue outlook below expectations could also negatively affect OPK shareholder value.

**Ex-U.S. market risks.** Given several OPK operations are out-side of the U.S. and some are in emerging markets, certain risks, such as macroeconomic volatility, geopolitical risk and currency fluctuations could all impact on the revenue generated from and operation in these territories.


Yale Jen, Ph.D.
yjen@laidlawltd.com

**Concentrated insider ownership**. Given senior management and other insiders own near 50% of OPK shares, the insider ownership is very concentrated. As such, insiders could have significant control and therefore, with the potential risk of creating price volatility. Highly concentrated insider ownership could also have impact on delaying or preventing a change in control of the company.


Yale Jen, Ph.D.
yjen@laidlawltd.com

Figure 1: Income Statement

## OPKO Health – Income Statement

| ($'MM) | 2013 | 2014 | 2015 | 1Q16 | 2Q16E | 3Q16E | 4Q16E | 2016E | 2017E | 2018E | 2019E | 2020E |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **Revenue** | | | | | | | | | | | | |
| Products (Pharmaceuticals) | 68.2 | 77.0 | 80.1 | 19.9 | 23.7 | 25.5 | 34.2 | 103.4 | 212.9 | 328.1 | 563.3 | 728.4 |
| Revenue from services (Diagnostics) | 11.7 | 8.7 | 329.7 | 252.5 | 279.3 | 305.1 | 333.7 | 1,171.7 | 1,354.7 | 1,566.1 | 1,817.5 | 2,080.7 |
| Revenue from transfer of intellectual property | 16.7 | 5.5 | 81.9 | 18.6 | 16.0 | 17.0 | 17.9 | 69.5 | 70.2 | 70.9 | 71.6 | 72.3 |
| Total revenue | 96.5 | 91.1 | 491.7 | 291.0 | 319.0 | 347.6 | 385.8 | 1,344.5 | 1,637.8 | 1,965.1 | 2,452.4 | 2,881.4 |
| Costs of revenues | 48.9 | 48.0 | 260.0 | 147.5 | 164.8 | 180.0 | 198.4 | 690.7 | 834.0 | 984.0 | 1,168.3 | 1,351.5 |
| Gross Incomes | 47.7 | 43.1 | 231.7 | 143.5 | 154.2 | 167.6 | 187.4 | 653.8 | 803.8 | 981.1 | 1,284.2 | 1,530.0 |
| Selling, general and administrative | 55.3 | 57.9 | 196.6 | 128.0 | 132.2 | 134.8 | 137.5 | 532.5 | 534.2 | 609.4 | 694.4 | 791.0 |
| Research and development | 53.9 | 83.6 | 99.5 | 27.8 | 29.8 | 31.3 | 32.5 | 121.4 | 129.9 | 136.3 | 141.8 | 147.5 |
| In process research and development | 0.0 | 12.1 | 0.0 | - | - | - | - | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| Contingent consideration | 6.9 | 24.4 | 5.1 | 1.8 | 4.0 | (2.0) | 3.3 | 7.1 | 7.1 | 7.1 | 7.1 | 7.1 |
| Amortization of intangible assets | 11.1 | 10.9 | 28.0 | 13.4 | 13.0 | 13.0 | 13.0 | 52.4 | 52.4 | 52.4 | 52.4 | 52.4 |
| Grant repayment | 0.0 | 0.0 | 25.9 | - | - | - | - | 0.0 | 0.0 | 0.0 | 0.0 | 0.0 |
| **Total Operating Expenses** | 127.3 | 188.9 | 355.0 | 171.0 | 179.0 | 177.1 | 186.3 | 713.4 | 723.6 | 805.2 | 895.7 | 998.0 |
| Total costs and expenses | 176.2 | 236.9 | 615.0 | 318.6 | 343.7 | 357.1 | 384.7 | 1,404.1 | 1,557.5 | 1,789.2 | 2,064.0 | 2,349.4 |
| Operating Incomes (losses) | (79.6) | (145.8) | (98.5) | (27.5) | (24.7) | (9.5) | 1.0 | (59.6) | 80.2 | 175.9 | 388.4 | 532.0 |
| Interest income | 0.4 | 0.8 | 0.3 | 0.0 | 0.2 | 0.2 | 0.2 | 0.6 | 0.7 | 0.8 | 0.9 | 0.9 |
| Interest expense | (13.8) | (12.3) | (8.4) | (1.8) | (2.7) | (2.7) | (2.7) | (9.9) | (9.9) | (9.9) | (9.9) | (9.9) |
| Fair value changes of derivative instruments, net | (45.9) | (10.6) | (39.1) | (1.4) | (1.0) | 2.0 | (4.0) | (4.4) | (6.0) | (6.0) | (6.0) | (6.0) |
| Other income (expense), net | 34.8 | (3.1) | 7.7 | 0.5 | 8.0 | 6.0 | (1.0) | 13.5 | 29.0 | 29.0 | 29.0 | 29.0 |
| Total Other Income, net | (24.6) | (25.2) | (39.5) | (2.6) | 4.5 | 5.5 | (7.5) | (0.1) | 13.8 | 13.9 | 14.0 | 14.0 |
| **Income before tax** | (104.2) | (171.0) | (138.0) | (30.2) | (20.2) | (4.0) | (6.5) | (59.7) | 94.1 | 189.8 | 402.4 | 546.0 |
| Tax | (1.7) | (0.0) | 113.7 | 20.5 | 0.0 | 0.0 | 0.0 | 20.5 | (34.8) | (70.2) | (148.9) | (202.0) |
| Loss before investment losses | (105.9) | (171.1) | (24.3) | (9.6) | (20.2) | (4.0) | (6.5) | (39.2) | 59.3 | 119.6 | 253.5 | 344.0 |
| Loss from investments in investees | (11.5) | (3.6) | (7.1) | (2.4) | (0.7) | (0.6) | (0.8) | (4.5) | (3.0) | (3.0) | (3.0) | (3.0) |
| **Net income (loss)** | (117.3) | (174.6) | (31.4) | (12.0) | (20.9) | (4.6) | (7.3) | (43.7) | 56.3 | 116.6 | 250.5 | 341.0 |
| Net loss attributable to noncontrolling interests | (2.9) | (3.0) | (1.4) | (0.8) | (0.8) | (0.8) | (0.8) | (1.4) | (3.0) | (3.0) | (3.0) | (3.0) |
| Net Income (Loss) Applicable to Common Shareholders | (114.8) | (171.7) | (30.0) | (12.0) | (20.2) | (3.8) | (6.5) | (42.3) | 59.3 | 119.6 | 253.5 | 344.0 |
| Net Earnings (Losses) Per Share—Basic and Diluted | ($0.32) | ($0.41) | ($0.06) | ($0.02) | ($0.04) | ($0.01) | ($0.01) | ($0.08) | $0.12 | $0.21 | $0.50 | $0.61 |
| Shares outstanding—basic | 355.1 | 422.0 | 488.1 | 545.8 | 549.8 | 553.8 | 557.8 | 551.8 | 496.1 | 559.8 | 504.1 | 567.8 |
| Shares outstanding—diluted | 355.1 | 422.0 | 488.1 | 545.8 | 549.8 | 553.8 | 557.8 | 551.8 | 496.1 | 559.8 | 504.1 | 567.8 |
| **Margin Analysis (% of Sales/Revenue)** | | | | | | | | | | | | |
| Costs of goods | 61% | 56% | 63% | 54% | 54% | 54% | 54% | 54% | 53% | 52% | 49% | 48% |
| Gross margin | 39% | 44% | 37% | 46% | 46% | 46% | 46% | 46% | 47% | 48% | 51% | 52% |
| R&D | 56% | 92% | 20% | 10% | 9% | 9% | 8% | 9% | 8% | 7% | 6% | 5% |
| MG&A | 57% | 64% | 40% | 44% | 41% | 39% | 36% | 40% | 33% | 31% | 28% | 27% |
| Operating Income (loss) | -82% | -160% | -20% | -9% | -8% | -3% | 0% | -4% | 5% | 9% | 16% | 18% |
| Net Income | -119% | -188% | -6% | -4% | -6% | -1% | -2% | -3% | 4% | 6% | 10% | 12% |
| **Financial Indicator Growth Analysis (YoY%)** | | | | | | | | | | | | |
| Products (Pharmaceuticals) | 50% | 13% | 4% | 28% | 4% | 23% | 63% | 29% | 106% | 54% | 72% | 29% |
| Revenue from services (Diagnostics) | 567% | -26% | 3705% | 12104% | 14539% | 194% | 50% | 255% | 16% | 16% | 16% | 14% |
| Revenue from transfer of intellectual property | N.A. | -67% | 1395% | 49% | -9% | -7% | -46% | -15% | 1% | 1% | 1% | 1% |
| Total Revenue | 105% | -6% | 440% | 867% | 652% | 143% | 40% | 173% | 22% | 20% | 25% | 17% |
| R&D | 176% | 55% | 19% | 9% | 1% | 65% | 28% | 22% | 7% | 5% | 4% | 4% |
| SG&A | 99% | 5% | 239% | 634% | 531% | 144% | 34% | 171% | 9% | 9% | 9% | 8% |
| Operating income (loss) | 114% | 83% | -32% | -52% | -3% | 16% | -113% | -39% | -235% | 119% | 121% | 37% |
| Total Other Income, net | -15001% | 3% | 57% | -95% | -127% | -88% | -53% | -100% | -11522% | 1% | 1% | 0% |
| Net Income | 267% | 49% | -82% | -90% | -53% | -103% | -505% | 41% | -240% | 102% | 112% | 36% |
| EPS | 206% | 26% | -85% | -92% | -61% | -103% | -498% | 25% | -256% | 79% | 135% | 20% |

**Yale Jen, Ph.D. 212-953-4978**

*Source: Bloomberg LP; Company reports; Laidlaw & Company estimates.*



Yale Jen, Ph.D.
yjen@laidlawltd.com

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***Additional information available upon request.***

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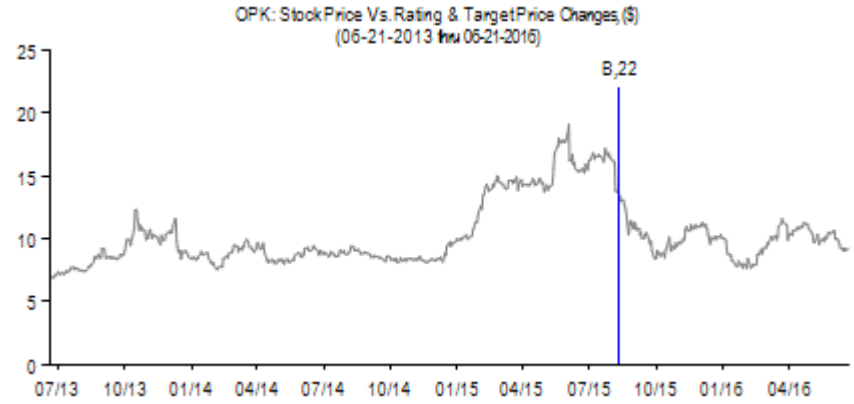


*3 Year Rating Change History*

| Date | Rating | Closing Price ($) |
|---|---|---|
| 08/12/2015 | Buy (B ) | 13.45 |

*3 Year Price Change History*

| Date | Target Price ($) | Closing Price, ($) |
|---|---|---|
| 08/12/2015 | 22.00 | 13.45 |

**Source: Laidlaw & Company** **Created by: Blue-Compass.net**

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|---|---|---|---|---|
| | | | Investment Banking | Brokerage |
| **Strong Buy (SB)** | Expected to significantly outperform the sector over 12 months. | 0.00% | 0.00% | 0.00% |
| **Buy (B)** | Expected to outperform the sector average over 12 months. | 67.57% | 27.03% | 2.70% |
| **Hold (H)** | Expected returns to be in line with the sector average over 12 months. | 0.00% | 0.00% | 0.00% |
| **Sell (S)** | Returns expected to significantly underperform the sector average over 12 months. | 0.00% | 0.00% | 0.00% |

## ADDITIONAL COMPANIES MENTIONED

Catalent (CTLT – Not Rated)

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