

OPKO Health (OPK - \$6.33)

OPK/PFE's Pediatric GHD in Leading Position Following Competitor's Failure in Pivotal Trial

The failure of the Phase III trial evaluating Versartis' long-acting growth hormone, VRS-317, in pediatric GHD leaves OPK's hGH-CTP in Phase III development in the leading position. We expect top-line data readout possibly in 2019.

- Details.** Yesterday after the market close, Versartis (VSAR – NR) reported the failure of their VRS-317 in pediatric growth hormone deficiency (GHD) Phase III (VELOCITY) trial, which missed the primary endpoint. The study intended to show non-inferiority of annual height velocity, needed to demonstrate that VRS-317 exhibited an outcome no less than the lower bound of a two-sided 95% CI (confidence interval) compared to the once daily administrated Genotropin. The intent to treat (ITT) results showed that the height velocity (HV) at 12 months for receiving VRS-317 was 9.44 cm vs. 10.70 cm for those receiving Genotropin. As for per protocol (PP) assessment, VRS-317 did demonstrate non-inferiority with 12-month HV of 9.71 cm for VRS-317 vs. 10.63 cm for Genotropin. In other news, another long-acting growth hormone competitor, Ascendis Pharma A/S (ASND – NR) recently indicated that patient recruitment of one of their Phase III trials (HEIGHT) for TransCon hGH (ACP-001) in pediatric GHD could complete in 4Q17 and we estimate the topline data readout could occur in early 2019.
- Implications.** The success or failure of competitor's drug cannot fundamentally change the clinical outcome of another drug. However, the reduced likelihood of VSAR immediately advancing VRS-317, in our opinion, has placed hGH-CTP in pediatric GHD development in a leading position with the topline data readout potentially available in early 2019. We have been more confident on the outlook of hGH-CTP, even though VRS-317 seemingly has a more favorable dosing regimen (1x/2 weeks vs. once weekly). We believe PFE had examined all available long-acting growth hormone development options before partnering with OPK. Should the hGH-CTP in pediatric GHD Phase III results be positive, we are more bullish for its market success vs. competitors given the strong presence of PFE in this space.
- Action.** We are reiterating our Buy rating and \$19 target 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.

Healthcare/Biotechnology

Ticker:	OPK
Rating:	Buy
Price Target:	\$19.00

Trading Data:

Last Price (9/21/2017)	\$6.33
52-Week High (12/15/2016)	\$12.15
52-Week Low (8/17/2017)	\$5.85
Market Cap. (MM)	\$3,625
Shares Out. (MM)	550.847

Earnings Estimates: (per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
FY-17E	-0.06A	-0.03A	-0.05	-0.04	-0.18	NM
FY-16A	-0.02	0.03	-0.03	-0.02	-0.05	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

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Source: Laidlaw & Company estimates

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Anticipated milestones in 2017 and beyond

Product	Indication	Event	Timing	Importance
Rayaldee	SHPT in CKD Stage 3-4 patients	Potential include in for formulary of healthcare plans	2017/2018	****
		Potential EMEA filing	2H17	***
		Potential EU approval	1H18	****
	SHPT in ESRD patients	Start Phase III study	4Q17	***
Rolapitant (VARUBI)	Chemotherapy -induced nausea and vomiting (CINV)	Potential approval of the IV version	2H17	****
hGH-CTP (MOD-4023)	hGH deficiency	FDA feedback and additional adult GHD Phase III data analysis	2H17	***
		Potential file BLA for adult GHD	2H17	****
		Potentially report of ACP-001 pediatric Phase III study top-line results by Ascendis	1H19	***
		Report of Phase III pediatric study top-line results	1H19	****
4Kscore test	Prostate cancer diagnostics	Novitas reimbursement decisions	2017	****
		Potential more private payer reimbursement decision	2017	****
Claros 1 testosterone test	POC testosterone test	Potential 510(k) filing	2H17	***
		Potential approval	2018	****
Claros 1 PSA test	POC PSA test	Modular PMA filing	3Q17	***
		Potential approval	2018	****
Claros 1 vitamin D test	POC vitamin D test	Potential 510(k) filing	2H17	****
		Potential approval	2018	****
MOD-5014 (IV)	Hemophilia A/B with inhibitors	Potentially report Phase I/IIa study top-line results	2H17	****
		Potentially start Phase II/III study	2018	***
Potentially report Phase II/III study results		2020	****	
MOD-5014 (s.c)		Potentially report Phase I study results	2H17	****
	Potentially start Phase II/III study	2018	***	
	Potentially report Phase II/III study results	2020	***	
NK-1 inhibitor (OPK88002)	Pruritus	Potentially start Phase IIa study	2H17	***
OPK88004	Obesity	Start Phase IIb study	4Q17	***
OPK88003	Benign prostate hypertrophy	Start Phase II study	4Q17	***
		Potentially report Phase II study results	Mid-2018	****
OPK88001	Dravet Syndrome	Potentially start Phase I/II trial	4Q17	***

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

Source: Laidlaw & Company estimates and company presentation.

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 successful 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.

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.

Figure 1: Income Statement

OPKO Health – Income Statement												
(\$'MM)	2013	2014	2015	2016	1Q17	2Q17	3Q17E	4Q17E	2017E	2018E	2019E	2020E
Revenue												
Products (Pharmaceuticals)	68.2	77.0	80.1	83.5	22.2	29.0	31.8	41.3	123.5	222.3	399.6	520.8
Revenue from services (Diagnostics)	11.7	8.7	329.7	1,012.1	255.3	256.7	278.0	270.7	1,059.1	1,231.0	1,425.9	1,645.1
Revenue from transfer of intellectual property	16.7	5.5	81.9	126.0	18.6	28.5	17.5	22.9	87.5	88.3	89.2	90.1
Total revenue	96.5	91.1	491.9	1,221.7	296.1	314.2	327.3	334.8	1,270.1	1,541.6	1,914.7	2,256.0
Costs of revenues	48.9	48.0	260.0	611.4	154.8	157.4	169.5	167.5	647.4	781.6	922.8	1,080.1
Gross Incomes	47.7	43.1	231.9	610.2	141.3	156.8	157.7	167.4	623.2	760.0	991.9	1,175.9
Selling, general and administrative	55.3	57.9	196.6	490.9	136.7	128.3	129.5	132.0	526.5	599.0	682.9	778.1
Research and development	53.9	83.6	99.5	111.2	26.0	32.6	38.1	40.0	136.8	143.6	149.4	155.4
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	17.0	2.4	4.4	1.9	1.8	10.5	10.5	10.5	10.5
Amortization of intangible assets	11.1	10.9	28.0	64.4	17.9	18.0	18.0	19.0	72.9	72.9	72.9	72.9
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	683.5	183.0	183.3	187.5	192.9	746.7	826.1	915.7	1,016.9
Total costs and expenses	176.2	236.9	615.0	1,294.9	337.8	340.7	357.0	360.4	1,394.1	1,607.7	1,838.5	2,097.0
Operating Incomes (losses)	(79.6)	(145.8)	(98.5)	(73.3)	(41.7)	(26.5)	(29.8)	(25.5)	(124.0)	(66.0)	76.2	159.0
Interest income	0.4	0.8	0.3	0.5	-	-	0.1	0.1	0.3	0.3	0.3	0.3
Interest expense	(13.8)	(12.3)	(8.4)	(7.4)	-	-	(2.3)	(2.3)	(4.6)	(4.6)	(4.6)	(4.6)
Fair value changes of derivative instruments, net	(45.9)	(10.6)	(39.1)	2.8	-	-	(4.4)	1.8	(2.6)	(2.6)	(2.6)	(2.6)
Other income (expense), net	34.8	(3.1)	7.7	3.9	5.9	3.6	(4.5)	3.0	8.0	8.0	8.0	8.0
Total Other Income, net	<u>(24.6)</u>	<u>(25.2)</u>	<u>(39.5)</u>	<u>(0.3)</u>	<u>5.9</u>	<u>3.6</u>	<u>(11.1)</u>	<u>2.6</u>	<u>1.1</u>	<u>1.1</u>	<u>1.1</u>	<u>1.1</u>
Income before tax	(104.2)	(171.0)	(138.0)	(73.5)	(35.8)	(22.9)	(40.8)	(22.9)	(123.0)	(64.9)	77.4	160.1
Tax	(1.7)	(0.0)	113.7	56.1	6.9	11.0	16.2	0.0	34.1	25.0	(28.6)	(59.2)
Loss before investment losses	(105.9)	(171.1)	(24.3)	(17.4)	(28.9)	(11.9)	(24.6)	(22.9)	(88.3)	(39.9)	48.7	100.9
Loss from investments in investees	(11.5)	(3.6)	(7.1)	(7.7)	(2.1)	(5.6)	(0.9)	(2.0)	(10.6)	(3.0)	(3.0)	(3.0)
Net income (loss)	(117.3)	(174.6)	(31.4)	(25.1)	(31.0)	(17.5)	(25.5)	(24.9)	(98.9)	(42.9)	45.7	97.9
Net loss attributable to noncontrolling interests	(2.9)	(3.0)	(1.4)	0.0	-	-	0.0	-	0.0	0.0	0.0	0.0
Net Income (Loss) Applicable to Common Shareholders	(114.8)	(171.7)	(30.0)	(25.1)	(31.0)	(17.5)	(25.5)	(24.9)	(98.9)	(42.9)	45.7	97.9
Net Earnings (Losses) Per Share—Basic and Diluted	(\$0.32)	(\$0.41)	(\$0.06)	(\$0.05)	(\$0.06)	(\$0.03)	(\$0.05)	(\$0.04)	(\$0.18)	(\$0.08)	\$0.08	\$0.17
Shares outstanding—basic	355.1	422.0	488.1	550.8	550.0	559.3	563.3	567.3	560.0	558.8	568.0	566.8
Shares outstanding—diluted	355.1	422.0	488.1	550.8	550.0	564.2	568.2	572.2	563.6	558.8	571.6	566.8
Margin Analysis (% of Sales/Revenue)												
Costs of goods	61%	56%	63%	56%	56%	55%	55%	54%	55%	54%	51%	50%
Gross margin	39%	44%	37%	44%	44%	45%	45%	46%	45%	46%	49%	50%
R&D	56%	92%	20%	9%	9%	10%	12%	12%	11%	9%	8%	7%
MG&A	57%	64%	40%	40%	46%	41%	40%	39%	41%	39%	36%	34%
Operating Income (loss)	-82%	-160%	-20%	-6%	-14%	-8%	-9%	-8%	-10%	-4%	4%	7%
Net Income	-119%	-188%	-6%	-2%	-10%	-6%	-8%	-7%	-8%	-3%	2%	4%
Financial Indicator Growth Analysis (YoY%)												
Products (Pharmaceuticals)	50%	13%	4%	4%	12%	27%	55%	104%	48%	80%	80%	30%
Revenue from services (Diagnostics)	567%	-26%	3705%	207%	1%	-4%	7%	15%	5%	16%	16%	15%
Revenue from transfer of intellectual property	N.A.	-67%	1395%	54%	0%	-58%	-5%	11%	-31%	1%	1%	1%
Total Revenue	105%	-6%	440%	148%	2%	-12%	10%	22%	4%	21%	24%	18%
R&D	176%	55%	19%	12%	37%	4%	56%	45%	23%	5%	4%	4%
SG&A	99%	5%	239%	150%	7%	9%	4%	10%	7%	9%	9%	8%
Operating income (loss)	114%	83%	-32%	-26%	51%	-194%	26%	-49%	69%	-47%	-215%	109%
Total Other Income, net	-15001%	3%	57%	-99%	-325%	-30%	5%	-66%	-499%	3%	3%	0%
Net Income	267%	49%	-82%	-16%	158%	-213%	71%	82%	294%	-57%	-207%	114%
EPS	206%	26%	-85%	-26%	156%	-210%	67%	94%	288%	-57%	-205%	114%

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

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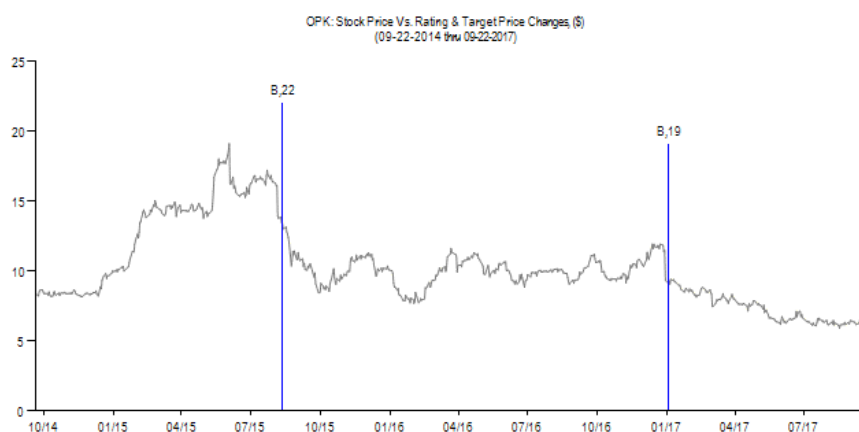
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3 Year Rating Change History

Date	Rating	Closing Price (\$)
08/12/2...	Buy (B)	13.45

3 Year Price Change History

Date	Target Price (\$)	Closing Price (\$)
08/12/2...	22.00	13.45
01/03/2...	19.00	9.09

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ADDITIONAL COMPANIES MENTIONED

Versartis (VSAR – Not Rated)
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