

Reason for report:

EARNINGS

## OPHTHOTECH CORPORATION

## 2Q14 Recap: on Heels of Big NVS Deal, 10 Trials to Be Underway Shortly

• **Bottom Line:** We are updating our model to reflect 2Q financial results reported today before the open. Our estimates are being revised to reflect higher R&D expense this quarter, due to the payment to licensors of a share of the upfront payment from NVS (OP). All in, OPHT has 10 clinical trials ongoing or about to start, tripling the number last year. OPHT exited the quarter in very solid financial shape, with \$452.5MM, including \$200MM in upfront cash from new partner NVS. As the Fovista Phase III enrolls, OPHT stands to earn an additional \$130MM from NVS in enrollment-based milestones. OPHT may also earn approval and commercial milestones of up to \$300mm and \$400mm respectively, plus royalties which are in the mid-30% range. The Fovista Phase III program is on track to produce top-line data in around 1,866 patients in 2016; OPHT has already activated over 225 sites worldwide. **Reiterate OP and \$61 PT in 12 months.**

• **During 2Q OPHT entered into a partnership with NVS to develop Fovista ex-US and potentially proprietary anti-VEGF (Vascular Endothelial Growth Factor) and-PDGF (Platelet Derived Growth Factor) combination products worldwide.** The Fovista development strategy remains agnostic with respect to physicians' choice of anti-VEGF agent, although OPHT and NVS may develop co-formulation products and a prefilled syringe to give physicians additional choices. OPHT has begun meeting with NVS planning teams to set priorities ahead of a potential Fovista launch. We view NVS as an extremely strong partner with established distribution to retinal surgeons around the world.

• **OPHT is on track to produce data from additional expansion trials of Fovista in wet AMD beginning in 2015.** These trials include the investigation of Fovista administered with anti-VEGF therapy for the reduction in the treatment burden, treatment-resistance and reduction of sub-retinal fibrosis in wet age-related macular degeneration (AMD) patients, the latter of which began enrollment yesterday. A paper will be presented on the improvement in long-term visual outcomes for VEGF experienced patients with sub-retinal fibrosis treated with Fovista anti-PDGF therapy at the AAO meeting on October 21.

• **We recently obtained positive feedback from 43 surveyed MEDACorp ophthalmologists (LINK)** who project a robust market opportunity for OPHT's Fovista in both treatment-naïve and not-well-controlled anti-VEGF-treated patients, the former of which presents upside to our \$747MM peak Fovista US risk-adjusted sales estimate in 2026. We were also favorably surprised to learn that almost 40% of physicians already consider fibrosis and geographic atrophy as key markers of disease progression, which we believe supports the Fovista value proposition.

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2013A	--	0.0	0.0	0.0	0.0	--	(12.39)	(10.26)	(0.65)	(6.34)	NM
2014E - New	0.0A	0.0A	0.0	0.0	0.0	(0.64)A	(1.57)A	(0.74)	(0.80)	(3.75)	NM
2014E - Old	0.0A	0.0A	0.0	0.0	0.0	(0.64)A	(0.69)	(0.73)	(0.76)	(2.82)	NM
2015E - New	0.0	0.0	0.0	0.0	0.0	--	--	--	--	(3.38)	NM
2015E - Old	0.0	0.0	0.0	0.0	0.0	--	--	--	--	(3.01)	NM

Source: Company Information and Leerink Partners LLC Research

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

## Key Stats:

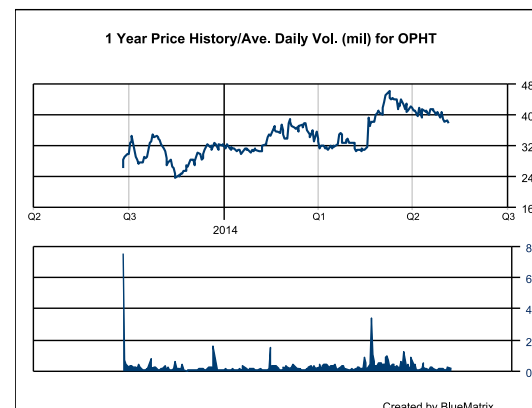
(NASDAQ:OPHT)

S&P 600 Health Care Index: 1,286.70  
Price: \$37.90  
Price Target: \$61.00

Methodology: DCF, 12% WACC, 2% Terminal Growth Rate

52 Week High: \$47.99  
52 Week Low: \$22.61  
Shares Outstanding (mil): 33.4  
Market Capitalization (mil): \$1,265.9  
Cash Per Share: \$12.73  
Dividend (ann): \$0.00  
Dividend Yield: 0.0%

General: fully diluted shares outstanding, net cash per share YE13



## INVESTMENT THESIS

We rate OPHT shares Outperform. Ophthotech is a late stage biopharmaceutical company that is primarily focused on the development of Fovista, an anti-Platelet Derived Growth Factor (PDGF) agent that has shown a statistically significant capacity to augment vision in wet Age-Related Macular Degeneration (AMD) patients. In Phase IIb, a combination of Fovista and Roche/Genentech's Lucentis was able to provide patients with a ~62% additional benefit over Lucentis monotherapy on the Early Treatment Diabetic Retinopathy Study (ETDRS), a widely-used standardized chart of vision testing. A Phase III Fovista pivotal trial is underway that we believe to be de-risked based on its strong similarities to Phase II, greater statistical powering, and longer duration, the latter of which could be beneficial to OPHT since the delta between the Fovista add-on arm and the Lucentis monotherapy arm seemed to be widening at the end of the Phase IIb trial. While anti-VEGF (vascular endothelial growth factor) treatments such as Lucentis and REGN's (OP) Eylea have achieved rapid commercial success and strong uptake in wet AMD, we see an opportunity for Fovista to upgrade the wet AMD standard-of-care, especially in patients who begin to plateau or even decline after receiving currently available treatments for multiple years. Our view is corroborated by the results of the Lucentis long-term visual outcomes HORIZON extension study which showed that after 5 years of therapy, the aggregate visual capacity of Lucentis-treated patients returned to baseline pre-treatment levels. There are approximately 1.3 million cases of wet AMD in the US, and our belief in the commercial attractiveness of Fovista stems not only from its efficacy but literature estimates that the prevalence of wet AMD is growing rapidly with an estimated ~200,000 additional wet AMD diagnoses per year. We expect Fovista to ultimately be indicated as an add-on therapy to any anti-VEGF treatment, as Fovista is being studied in combination with each of Lucentis, Eylea, and Roche's Avastin in the ongoing Phase III program. OPHT holds strong method of treatment patent protection over Fovista in the US, EU, and Japan that expires in 2027 with extensions.

## VALUATION

We estimate a \$61 per share value for OPHT based on a discounted cash flow analysis that assumes a 12% discount rate and a 2% terminal growth rate. We project Fovista revenue growth from 2018 through 2027 in the US and EU and cut it significantly thereafter at the expiration of OPHT's method-of-treatment patent. We see upside to our valuation from either: (1) less robust competition than we anticipate, or (2) the potential for Fovista to be best-in-class even in the face of anti-PDGF competition from co-formulated or augmented anti-VEGF agents.

## RISKS TO VALUATION

Risks to our OPHT valuation include the possibility of disappointing clinical data, commercial shortfalls, or higher-than-anticipated regulatory hurdles. Since OPHT solely has one product in late-stage development, any of these could impact the stock significantly.

<i>Ophthotech P&amp;L (\$MM except EPS)</i>	2012	1H13	3Q13	4Q13	2013	1Q14	2Q14	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E
Fovista Product Sales by OPHT (p/w)	-	-	-	-	-	-	-	-	-	-	-	-	-	241.8
Royalties on EU Fovista Sales	-	-	-	-	-	-	-	-	-	-	-	-	-	25.9
Zumira (ARC1905) Sales	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Royalties to Novo	-	-	-	-	-	-	-	-	-	-	-	-	-	19.2
Royalties to OSI (eyetech)	-	-	-	-	-	-	-	-	-	-	-	-	-	9.6
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	29.0
R&D	6.8	6.7	11.1	15.4	33.2	14.4	34.7	17.9	20.0	87.0	100.0	102.0	104.0	106.1
SG&A	6.9	5.0	4.2	5.1	14.2	6.3	7.6	6.8	7.0	27.7	29.1	30.6	51.6	53.2
Operating Expenses	13.7	11.7	15.3	20.4	47.4	20.7	42.3	24.7	27.0	114.7	129.1	132.6	155.7	217.2
<b>Operating Income</b>	<b>(13.7)</b>	<b>(11.7)</b>	<b>(15.3)</b>	<b>(20.4)</b>	<b>(47.4)</b>	<b>(20.7)</b>	<b>(42.3)</b>	<b>(24.7)</b>	<b>(27.0)</b>	<b>(114.7)</b>	<b>(129.1)</b>	<b>(132.6)</b>	<b>(155.7)</b>	<b>50.6</b>
Interest income, expense (net)	(0.5)	(1.5)	-	-	(1.5)	0.0	0.1	-	-	0.1	-	-	-	-
Other income, expense (net)	(0.3)	(1.5)	(0.9)	0.1	(2.3)	-	-	-	-	-	-	-	-	-
EBT	(14.5)	(14.6)	(16.1)	(20.4)	(51.1)	(20.7)	(42.2)	(24.7)	(27.0)	(114.6)	(129.1)	(132.6)	(155.7)	50.6
Tax Expense (Benefit)	-	-	-	-	-	-	10.3	-	-	-	-	-	-	-
<b>Net Income</b>	<b>(14.5)</b>	<b>(14.6)</b>	<b>(16.1)</b>	<b>(20.4)</b>	<b>(51.1)</b>	<b>(20.7)</b>	<b>(52.5)</b>	<b>(24.7)</b>	<b>(27.0)</b>	<b>(114.6)</b>	<b>(129.1)</b>	<b>(132.6)</b>	<b>(155.7)</b>	<b>50.6</b>
<b>Diluted EPS</b>	<b>(2.52)</b>	<b>(12.39)</b>	<b>(10.26)</b>	<b>(0.65)</b>	<b>(6.34)</b>	<b>(0.64)</b>	<b>(1.57)</b>	<b>(0.74)</b>	<b>(0.80)</b>	<b>(3.75)</b>	<b>(3.38)</b>	<b>(3.38)</b>	<b>(3.87)</b>	<b>1.20</b>
Basic Shares Outstanding	8.6	1.5	1.8	31.4	9.0	32.3	33.4	33.6	33.8	33.3	38.3	39.3	40.3	41.3
Diluted Shares Outstanding	8.6	1.5	1.8	34.8	12.7	35.7	33.4	33.6	33.8	34.1	39.1	40.1	41.1	42.1

Note: Basic and Diluted Shares Outstanding for 1H13 and 3Q13 are lower since Ophthotech officially became a public company on September 25, 2013 after which we OPHT had 31.4MM basic shares outstanding for 4Q13

<i>Balance Sheet and Cash Flow Statement (\$MM)</i>	2012	1H13	3Q13	4Q13	2013	1Q14	2Q14	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E
Cash & Cash Equivalents	4.3	39.9	236.1	210.6	210.6	290.8	452.5	429.8	496.6	485.8	446.0	447.6	400.4	508.7
Debt	11.0	-	-	-	-	-	-	-	-	-	-	-	-	-

<i>Change in Cash</i>	(2.1)	28.0	196.2	(25.5)	198.8	80.2	150.9	(22.7)	66.9	275.2	(39.8)	1.5	(47.2)	108.3
<b>Cash Flow From Operations</b>	<b>(13.1)</b>	<b>(13.7)</b>	<b>(14.9)</b>	<b>(22.8)</b>	<b>(47.4)</b>	<b>(16.1)</b>	<b>(49.1)</b>	<b>(22.7)</b>	<b>(24.8)</b>	<b>(102.8)</b>	<b>(118.8)</b>	<b>(122.0)</b>	<b>(143.2)</b>	<b>63.3</b>
Net Income	(14.5)	(14.6)	(16.1)	(20.4)	(51.1)	(20.7)	(52.5)	(24.7)	(27.0)	(114.6)	(129.1)	(132.6)	(155.7)	50.6
Stock Option Expense	0.6	0.9	1.2	1.6	3.8	4.3	3.4	2.0	2.2	11.8	10.3	10.6	12.5	12.7
Other	0.8	-	-	(4.0)	-	0.3	-	-	-	-	-	-	-	-
<b>Cash Flow From Investing</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>(2.7)</b>	<b>(2.7)</b>	<b>(225.0)</b>	<b>200.0</b>	<b>-</b>	<b>50.0</b>	<b>25.0</b>	<b>79.0</b>	<b>(1.5)</b>	<b>96.0</b>	<b>45.0</b>
Sale (Purchase) of Marketable Securities	-	-	-	-	-	(224.2)	-	-	-	(224.2)	-	-	-	-
CapEx	-	-	-	-	-	(0.7)	-	-	-	(0.7)	(2.0)	(3.0)	(4.0)	(5.0)
D&A/Other	-	-	-	(2.7)	(2.7)	-	200.0	-	50.0	250.0	81.0	1.5	100.0	50.0
<b>Cash Flow From Financing</b>	<b>11.0</b>	<b>41.7</b>	<b>192.0</b>	<b>-</b>	<b>221.7</b>	<b>97.1</b>	<b>-</b>	<b>-</b>	<b>41.7</b>	<b>138.8</b>	<b>-</b>	<b>125.0</b>	<b>-</b>	<b>-</b>
Equity Issuance (Buyback/Costs)	0.0	-	192.0	-	192.0	55.4	-	-	-	55.4	-	125.0	-	-
Debt Issuance (Buyback/Costs)	11.0	-	-	-	(12.0)	-	-	-	-	-	-	-	-	-
Other	-	41.7	-	-	41.7	41.7	-	-	41.7	83.4	-	-	-	-

Source: SEC Filings and Leerink Partners Estimates

<i>OPTH DCF Analysis, \$MM</i>	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	TV
Free Cash Flow to Equity	(47.4)	(102.8)	(39.8)	(123.5)	(47.2)	108.3	177.3	255.4	331.1	393.3	410.3	432.7	451.1	468.4	471.2	269.9	237.3	
Discount Periods	0	0	0.5	1.5	2.5	3.5	4.5	5.5	6.5	7.5	8.5	9.5	10.5	11.5	12.5	13.5	14.5	
Discounted FCFE	-	(51.4)	(37.6)	(104.3)	(35.6)	73.1	106.9	137.6	159.4	169.2	157.8	148.7	138.5	128.5	115.6	59.2	46.5	478.8

NPV	\$ 1,690.91
Net Cash 2Q14	\$ 452.49
<b>OPHT Per Shr Val</b>	<b>\$ 64.23</b>
Implied Mkt. Cap	\$ 2,143.4

Cost of Equity	12%
TG	2%
Diluted Shrs. Outstanding	33.4

Source: Leerink Partners Estimates

<b><i>OPHT Milestones</i></b>		
<b>Product</b>	<b>Event</b>	<b>Timing</b>
Fovista	Initiate Expansion Trials	2014
Zimura	Clinical Trial Initiation	4Q14/1Q15
Fovista	Data from Smaller AMD Trials	2015
Fovista	Pivotal Phase III Data	Mid-2016
Fovista	NDA/MAA Filings	2H16
Fovista	FDA/EMA Approval	2H17

*Source: SEC Filings and Leerink Partners Estimates*

## Disclosures Appendix

### Analyst Certification

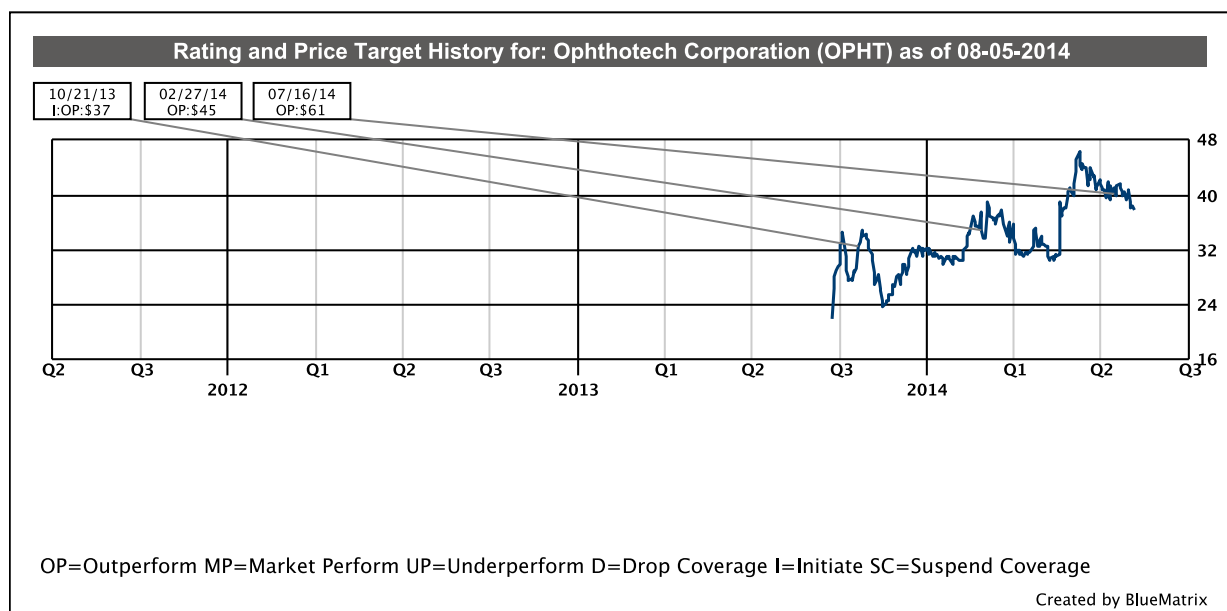
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

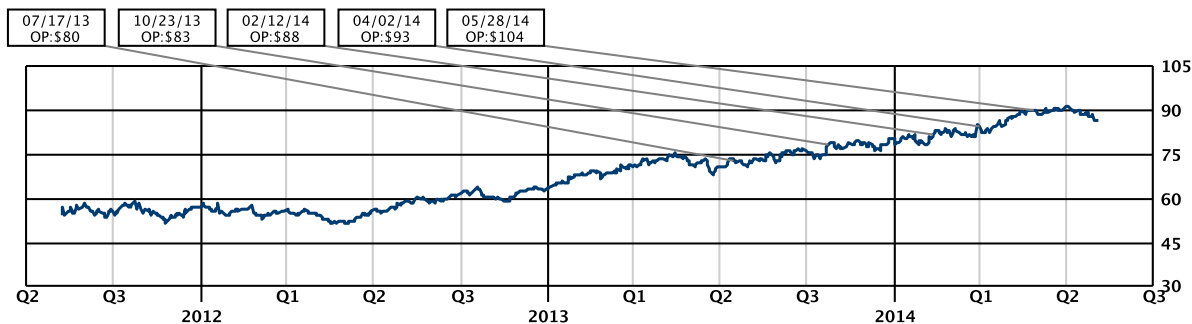
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**Rating and Price Target History for: Novartis AG (NVS) as of 08-05-2014**


Leerink Swann initiated coverage of NVS with an Outperform rating on November 9, 2010. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix

**Rating and Price Target History for: Regeneron Pharmaceuticals, Inc. (REGN) as of 08-05-2014**


Leerink Swann initiated coverage of REGN with an Outperform rating on June 2, 2011. On June 11, 2013, Leerink Swann began a transition to specific price targets for the stocks under its coverage, replacing valuation ranges.

OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage

Created by BlueMatrix

Distribution of Ratings/Investment Banking Services (IB) as of 06/30/14				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OP]	138	69.00	50	36.20
HOLD [MP]	62	31.00	2	3.20
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.

## Important Disclosures

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MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.



In the past 12 months, the Firm has received compensation for providing investment banking services to Ophthotech Corporation .

Leerink Partners LLC makes a market in Ophthotech Corporation and Regeneron Pharmaceuticals, Inc.

Leerink Partners LLC is willing to sell to, or buy from, clients the common stock of Novartis AG on a principal basis.

Leerink Partners LLC has acted as a co-manager for a public offering of Ophthotech Corporation in the past 12 months.

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