

# **COMPANY UPDATE**

August 6, 2014

Stock Rating:

# **OUTPERFORM**

12-18 mo. Price Target	\$95.00
OPHT - NASDAQ	\$37.95

3-5 Yr. EPS Gr. Rate	NM
52-Wk Range	\$47.99-\$22.61
Shares Outstanding	33.3M
Float	17.3M
Market Capitalization	\$1,270.8M
Avg. Daily Trading Volume	397,794
Dividend/Div Yield	NA/NM
Book Value	\$6.22
Fiscal Year Ends	Dec
2014E ROE	NM
LT Debt	NA
Preferred	NA
Common Equity	\$207M
Convertible Available	No

EPS Diluted	Q1	Q2	Q3	Q4	Year	Mult.
2013A	(4.33)	(8.07)	(10.26)	(0.65)	(6.34)	NM
2014E	(0.64)A	(1.57)A	(0.72)	1.82	(0.73)	NM
Prior (E)		(0.62)	(0.65)	(0.69)	(2.60)	NM
2015E					(1.99)	NM
Prior (E)					0.84	NM
Revenue (\$/mil)	Q1	Q2	Q3	Q4	Year	Mult.
2013A	0.0	0.0	0.0	0.0	0.0	NM
2014E	0.0A	0.0A	0.0	86.7	86.7	NM
Prior (E)		1.3	2.6	2.6	6.6	NM
2015E					43.3	NM
Prior (E)					140.5	NM

HEALTHCARE/BIOTECHNOLOGY

# **Ophthotech Corporation**

Catalysts on the Way in 2H14

#### SUMMARY

The 2Q14 updates were relatively modest (as anticipated) but nonetheless positive. A key 2H catalyst for the stock is data at the American Academy of Ophthalmology (AAO) Meeting on October 21, 2014. Ophthotech will present a subgroup analysis on reduction of sub-retinal fibrosis from their positive Phase 2b trial for Fovista and Lucentis in wet AMD. We expect significant interest in this oral presentation from retinal specialists attending the meeting as current therapies (anti-VEGFs) do not address the fibrotic aspect of disease progression in wet AMD. With the Phase 2a in sub-retinal fibrosis now underway, and additional wAMD expansion trials nearing initiation (anti-VEGF resistant, anti-VEGF burden), we see 2015 as offering multiple catalysts ahead of the 2016 Phase 3 data.

#### **KEY POINTS**

- 3Q14 Catalysts Ahead of AAO. We have identified two catalysts worth noting not widely appreciated by investors (see Ex. 1): 1) August 12, American Society of Retinal Specialists oral presentation on Phase 2b response durability, 2) September 12, The Retina Society Annual Meeting, oral presentation on biomarkers associated with visual gain in Phase 2b.
- AAO Oral Presentation. An external expert (prominent anti-VEGF investigator) has evaluated in a blinded fashion imaging data from a subgroup in the completed Phase 2b (presentation at AAO). We expect these findings to support Ophthotech's hypothesis that Fovista may reduce sub-retinal fibrosis. The new Phase 2a should provide additional important insights into this question.
- 2015 Catalysts. Ophthotech is executing on a strategic plan to develop Fovista as a potential backbone of therapy in wet AMD vs. the current (and only) option of an anti-VEGF. We see the new anti-fibrosis trial as underpinning this plan, complemented by additional planned expansion trials (VEGF-resistant, anti-VEGF injection burden) that create important 2015 catalysts.
- Next Novartis Milestones. Near-term enrollment milestones for the Fovista Phase 3 program may be underappreciated. The replicate Fovista+Lucentis trials (N=622 each) commenced August 2013, and with >225 sites, implies ∼3 patients/ site/trial. Completion of enrollment for these 2 trials in 4Q14 triggering ∼\$87M (∼2/3 of \$130M, our assumption) seems achievable.
- Model Changes. Refined 2014E EPS. See page 2 for details.

### **Stock Price Performance**

# 1 Year Price History for OPHT 48 40 40 40 22 Q2 Q3 Q1 Q1 Q2 Q3 16 Created by Blumbatrs.

### **Company Description**

Ophthotech is a biotechnology company focused on the development and commercialization of novel therapeutics to treat eye diseases. The company's most advanced asset is Fovista, a PDGF inhibitor being tested in combination with anti-VEGF therapy for wet agerelated macular degeneration (wAMD), a disease characterized by progressive retinal damage and vision loss.

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Yigal Nochomovitz, Ph.D. 212 667-8163 Yigal.Nochomovitz@opco.com **Model Changes.** We have brought forward ~\$87M of the \$130M in projected Novartis enrollment milestones to 4Q14 from 2015. Changes to our 2014E EPS estimate reflect higher than expected R&D in 2Q14 (~\$20M milestone to Nektar) offset by our more aggressive assumption on additional Novartis milestone revenue.

# Exhibit 1. 3Q14 Medical Meeting Catalysts

Meeting	Location	Date	Time	Session	Speaker	Affiliation	Title
American Society of Retinal Specialists	Hilton Bayfront Hotel, San Diego, CA	8/12/14	2:27 PM	AMD Neovascular 2	Peter Kaiser, MD	Cleveland Clinic	Outcomes in Patients with Visual Gain or Any Loss 1 Month After Combination Therapy of Anti-PDGF-B Plus Ranibizumab Compared to Ranibizumab Monotherapy
The Retina Society	Union League, Philadelphia, PA	9/12/14	3:27 PM	Age-related Macular Degeneration 1	Glenn Jaffe, MD	Duke University	Morphological Biomarkers Associated with Visual Acuity Gain and Loss in Eyes with Neovascular Age-Related Macular Degeneration Treated with Dual Antagonism of PDGF (Fovista 1.5mg)/VEGF (Lucentis 0.5 mg)
Canadian Ophthalmological Society	Westin Hotel, Ottawa, Ontario	9/18/14	ТВА	Global Impact of Retinal Innovations	Samir Patel, MD	Ophthotech, President	ТВА

Sources: Oppenheimer Research, http://www.retinasociety.org/files/2014\_meeting.pdf, https://www.eyefox.com/2908/46th-Sally-Letson-Symposium-2014.html, https://www.asrs.org/content/documents/annualmeetingschedforweb.pdf



Exhibit 2

# **Ophthotech Income Statement**

in millions of \$ except per share values FY:11 FY:12 FY:13 FY:14E FY:15E FY:16E FY:17E FY:18E FY:19E FY:20E FY:21E FY:22E FY:23E FY:24E Income Statement GAAP - as reported 1.193 1.514 Fovista US Revenue 111 729 1.642 1.700 1.755 1.806 Fovista EU Revenue (Novartis Rovalty) 55.6 95.9 112.7 115.9 111.3 105.4 98.3 Novartis Milestones 86.7 43.3 0.0 200.0 0.0 0.0 0.0 50.0 50.0 50.0 50.0 1.626.5 Fovista WW Revenue 86.7 43.3 0.0 320.0 784.5 1.288.8 1.807.9 1,861.4 1,910.7 1.954.6 % change y-o-y 145.1% 64.3% 26.2% 11.2% 3.0% 2.6% 2.3% Operating expenses: Cost of sales 21 2 138.5 226.6 287 6 312.0 323.0 333.5 343.2 Drug COGS 8.9 56.5 89.5 109.8 114.9 114.8 114.1 112.9 8.0% 7.8% 7.5% 7.3% 7.0% 6.8% 6.5% 6.3% Royalties 12.2 82.0 137.2 177.9 197.0 208.3 219.4 230.3 COGS + Royalties % of revenue 6.6% 17.7% 17.6% 17.7% 17.3% 17.4% 17.5% 17.6% Gross Profit 0.0 0.0 0.0 86 7 43 3 0.0 298 9 646.0 1,062.1 1,338.9 1.496.0 1.538.4 1,577.2 1.611.4 Gross margin 100.0% 100.0% 93.4% 82.4% 82.7% 82.6% 82.4% Research and development 13.9 6.8 33.2 86.5 87.8 102.2 96.7 93.4 90.3 87.4 84.7 82.3 80.0 77.9 % of revenue 30.2% 11.9% 7.0% 5.4% 4.7% 4.4% 4.2% 4.0% General and administrative 5.7 6.9 14.2 24.4 25.6 51.9 90.7 113.3 115.9 118.7 121.7 124.8 128.0 131.4 % of revenue n.a. n.a. n.a. 28.4% 14.4% 9.0% 7.3% 6.7% 6.7% 6.7% 6.7% Others % of revenue 0.0% 0.0% 0.0% 0.0% 0.0% 0.0% 0.0% 0.0% 0.0% 0.0% Total operating expenses 19.6 13.7 47.4 110.9 113.4 154.1 208.6 345.1 432.8 493.8 518.4 530.0 541.5 552.5 % of revenue 44.0% 33.6% 30.4% 28.7% 28.5% 28.3% 28.39 Operating income/(loss) (19.6) (13.7) (47.4) (24.2) (70.1) (154.1) 90.3 300.9 629.3 845.1 977.6 1,008.3 1,035.7 1,058.9 Operating margin 27.9% 161.7% 28.2% 38.4% 48.8% 52.0% 54.1% 54.2% 54.2% 54.2% n.a n.a. Interest income/(expense) 0.0 (0.5) (1.5) 0.1 0.0 0.0 0.0 0.0 0.0 0.0 0.0 0.0 0.0 0.0 Gain/(loss) on extinguishment of debt (1.1) 0.0 0.0 0.0 0.0 0.0 1.2 1.3 0.9 2.6 4.8 7.7 11.1 14.6 18.2 1.4 (0.0) Other gain (loss) (0.4) (1.2) 0.0 Change in fair value related to investor rights liability 0.0 Net income/(loss) before Taxes (19.7) (14.6) (51.1) (24.1 (68.6) (153.0) 301.8 631.9 849.9 985.3 1,019.4 1,050.3 1,077.0 Income Tax 1.0 0.0 0.0 0.0 0.0 0.0 0.0 21.8 221.2 297.5 344.9 356.8 367.6 377.0 Effective tax rate (%) 5.2% 0.0% 0.0% 0.0% 0.0% 0.0% 0.0% 7.2% 35.0% 35.0% 35.0% 35.0% 35.0% 35.0% (14.6) (153.0) 323.6 Net income/(loss) (18.6) (51.1) (24.1) (68.6) 91.6 410.7 552.4 640.4 662.6 682.7 700. Accretion of preferred stock dividends (6.8 (7.1)(5.9) 0.0 0.0 0.0 0.0 0.0 0.0 0.0 0.0 0.0 0.0 0.0 323.6 640.4 662.6 Net income/(loss) to common shareholders (25.5) (21.6) (57.0) (24.1) (68.6) (153.0) 91.6 410.7 552.4 682.7 700.1 \$17.94 Basic EPS (\$18.27) (\$14.89) (\$6.34) (\$0.73) (\$1.99) (\$4.26) \$2.49 \$8.69 \$10.90 \$14.51 \$16.67 \$17.13 \$17.57 Basic number of shares 37.2 37.7 38.4 38.7 38.8 39.0

35.4

37.1

38.4

39.4

40.5

41.5

42.4

43.2

43.7

44.4

9.0

33.2

Sources: Oppenheimer Research Estimates, Ophthotech Filings.

Diluted number of shares

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Exhibit 3

# **Ophthotech Balance Sheet**

in millions of \$ except per share values

in millions of \$ except per share values												1		
Balance Sheet	FY:11	FY:12	FY:13	FY:14E	FY:15E	FY:16E	FY:17E	FY:18E	FY:19E	FY:20E	FY:21E	FY:22E	FY:23E	FY:24E
<u>ASSETS</u>														
Current assets														
Cash and cash equivalents	6.4	4.3	210.6	288.0	232.0	262.4	178.5	522.6	962.6	1,543.1	2,218.5	2,913.1	3,631.8	4,372.5
Prepaid expenses and other current assets	0.1	0.0	6.8	8.2	9.1	12.3	16.7	27.6	34.6	39.5	41.5	42.4	43.3	44.2
Inventory	0.0	0.0	0.0	-	-	-	-	-	-	-	-	-	-	-
Other receivables	1.0			-										
Other Assets		0.3	0.0	188.7	188.7	38.7	38.7	38.7	38.7	38.7	38.7	38.7	38.7	38.7
Deferred tax assets				0.8	0.8	0.8	0.8	0.8	0.8	0.8	0.8	0.8	0.8	0.8
Security deposits		0.2	0.0	-	-	-	-	-	-	-	-	-	-	-
Total current assets	7.5	4.8	217.4	485.7	430.7	314.3	234.8	589.8	1,036.8	1,622.2	2,299.6	2,995.0	3,714.7	4,456.3
Property, plant and equipment, net	0.1	0.0	0.0	2.1	3.6	5.5	7.5	9.1	10.0	10.6	10.8	10.9	10.9	11.0
Security deposits (Non-current)	0.2	0.0	0.3	0.3	0.3	0.3	0.3	0.3	0.3	0.3	0.3	0.3	0.3	0.3
Deferred tax assets (Non-current)				19.6	19.6	19.6	19.6	19.6	19.6	19.6	19.6	19.6	19.6	19.6
Other long term assets				15.3	15.3	15.3	15.3	15.3	15.3	15.3	15.3	15.3	15.3	15.3
Total assets	7.7	4.9	217.7	503.3	449.8	335.3	257.7	614.4	1,062.3	1,648.3	2,325.9	3,021.5	3,741.1	4,482.8
Total assets		4.0	217.7	505.5	440.0	000.0	201.1	014.4	1,002.0	1,040.0	2,020.0	0,021.0	0,141.1	4,402.0
LIABILITIES & SHAREHOLDERS' EQUITY														
Accrued clinical drug supplies & trial costs	1.5	1.0	2.5	3.1	3.5	4.1	3.9	3.7	3.6	3.5	3.4	3.3	3.2	3.1
Accounts payable and accrued expenses	1.6	1.4	3.8	4.3	4.8	6.5	8.8	14.5	18.2	20.7	21.8	22.3	22.7	23.2
Accrued bonuses				0.0										
Loans payable, current portion	0.0	0.0	0.0											
Notes payable		11.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Income Tax Payable				29.5										
Warrant liability	0.2	1.0	0.0	0.0										
Deferred Revenue (Novartis)				200.0	200.0	200.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Deferred Rent	0.0			0.0										
Total current liabilities	3.3	14.4	6.3	236.9	208.3	210.6	12.6	18.2	21.8	24.2	25.2	25.6	25.9	26.3
Loans payable, less current portion	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Royalty purchase liability		0.0	41.7	83.3	83.3	83.3	83.3	83.3	83.3	83.3	83.3	83.3	83.3	83.3
Total liabilities	3.3	14.4	48.0	320.2	291.6	293.9	96.0	101.6	105.1	107.6	108.5	108.9	109.3	109.7
Preferred stock														
Series A	65.3	69.5	0.0	0.0										
Series A-1	8.0	8.5	0.0	0.0										
Series B	33.1	35.5	0.0	0.0										
Series B-1	0.5	0.6	0.0	0.0										
Series C			0.0	0.0										
Stockholders' equity (deficit)														
Junior Series A Convertible Preferred Stock	3.0	3.0	0.0	0.0										
Common stock	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Additional paid-in capital		0.0	352.7	415.6	445.9	467.2	479.7	489.2	503.0	512.3	524.6	530.9	538.5	547.9
Accumulated surplus/(deficit)	(105.5)	(126.5)	(183.1)	(213.0)	(268.1)	(406.2)	(298.3)	43.4	473.9	1,048.1	1,712.4	2,401.4	3,113.0	3,844.9
Accumulated other comprehensive income	()	(.=5.0)	(/)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total stockholders' equity/(deficit)	(102.5)	(123.5)	169.7	202.7	177.9	61.1	181.5	532.6	976.9	1,560.4	2,237.1	2,932.3	3,651.6	4,392.8
Total liabilities & Shareholders equity	7.7	4.9	217.7	522.9	469.5	355.0	277.5	634.1	1,082.0	1,668.0	2,345.6	3,041.2	3,760.8	4,502.5

Sources: Oppenheimer Research Estimates, Ophthotech Filings.



Exhibit 4
Ophthotech Cash Flow Statement

in millions of US\$ except per share values	=>/ //	=>/ +=		=>/ / / =		=>/ / -=				=======================================		=>/=		
Cash Flow Statement	FY:11	FY:12	FY:13	FY:14E	FY:15E	FY:16E	FY:17E	FY:18E	FY:19E	FY:20E	FY:21E	FY:22E	FY:23E	FY:24E
Operating cash flows														
Net loss	(18.6)	(14.6)	(51.1)	(36.3)	(68.6)	(153.0)	91.6	323.6	410.7	552.4	640.4	662.6	682.7	700.1
<u>Adjustments</u>														
Depreciation	0.0	0.0	0.0	0.3	0.8	1.3	2.0	2.7	3.4	3.8	4.1	4.3	4.4	4.4
Amortization of debt issuance costs		0.0	0.1											
Accretion of debt discount		0.1	0.1											
Amortization of premium and discounts on securities				0.8										
Non-cash change in fair value of warranty liability	0.0	0.4	1.2											
Non-cash chg in fair value of investor rights liability														
Loss on extinguishment of debt			1.1											
Deferred tax provision				(19.2)										
Share-based compensation	0.2	0.6	2.9	12.3	13.5	14.9	16.3	18.0	19.8	21.8	23.9	26.3	29.0	31.8
Pref stk issued for acquired tech & licenses	0.5													
Excess tax benefits from share-based compensation				(1.2)										
Accrued int. expense converted to pref stk				(1.2)										
Changes in operating assets & liabilities														
Prepaid expense and other current assets	0.1	0.0	(6.8)	(1.4)	(0.9)	(3.3)	(4.4)	(10.9)	(7.0)	(4.9)	(2.0)	(0.9)	(0.9)	(0.9)
Other receivables	(0.7)	1.0		0.1	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Inventory	0.0	0.0	0.0		0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Security deposits			(0.1)											
Accrued clinical drug supplies and trial costs	(0.8)	(0.5)	1.5	0.6	0.4	0.6	(0.2)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)	(0.1)
Accounts payable and accrued expenses	0.2	(0.2)	2.4	0.5	0.5	1.7	2.3	5.7	3.7	2.6	1.0	0.5	0.5	0.5
Accrued bonuses														
Income Tax payable				29.5	(29.5)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Deferred revenue				200.0	0.0	0.0	(200.0)	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Deferred rent	(0.0)	(0.0)	0.0											
Net cash provided by (used in) operating activities	(19.1)	(13.1)	(48.8)	185.9	(83.9)	(137.7)	(92.3)	339.0	430.5	575.5	667.4	692.7	715.4	735.8
Investing cash flows														
Purchase of marketable securities				(244.8)										
Maturities of marketable securities	3.4			40.0		150.0								
Purchase of property, equipment	(0.0)		(0.0)	(2.3)	(2.4)	(3.2)	(3.9)	(4.4)	(4.3)	(4.3)	(4.3)	(4.4)	(4.4)	(4.4)
Net cash provided by (used in) investing activities	3.4	0.0	(0.0)	(207.2)	(2.4)	146.8	(3.9)	(4.4)	(4.3)	(4.3)	(4.3)	(4.4)	(4.4)	(4.4)
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Financing cash flows														
Payment of debt issuance costs		(0.4)	(0.0)	0.0										
Proceeds from issuance of common stk	0.0	0.0	0.1	0.3	30.3	21.3	12.5	9.5	13.8	9.3	12.3	6.2	7.6	9.4
Proceeds from initial public offerings, net	0.0	0.0	175.6	0.0										
Proceeds from follow-on public offering, net			0.0	55.4										
Excess tax benefits from share-based compensation				1.2										
(Repayments on) proceeds from notes payables		11.4	(11.9)	0.0										
Repayment of loan payable	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Proceeds from issuance of preferred stk	15.0		49.7	0.0				1						
Proceeds from royalty purchase agreement			41.7	41.7										
Net cash provided by (used in) financing activities	15.0	11.0	255.1	98.6	30.3	21.3	12.5	9.5	13.8	9.3	12.3	6.2	7.6	9.4
Net change in cash & cash equivalents	(0.7)	(2.1)	206.3	77.4	(55.9)	30.3	(83.8)	344.1	440.0	580.5	675.4	694.6	718.7	740.7
Net cash & cash equivalents - opening balance	7.1	6.4	4.3	210.6	288.0	232.0	262.4	178.5	522.6	962.6	1,543.1	2,218.5	2,913.1	3,631.8
Net cash & cash equivalents - ending balance	6.4	4.3	210.6	288.0	232.0	262.4	178.5	522.6	962.6	1,543.1	2,218.5	2,913.1	3,631.8	4,372.5

Sources: Oppenheimer Research Estimates, Ophthotech Filings.

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#### **Investment Thesis**

We believe Ophthotech's PDGF inhibitor Fovista being developed for wet AMD has high chances of success in an ongoing Phase 3 program (data 2016), with expected FDA approval to follow in 2017. Our conviction is based on solid proof-of-concept from randomized Phase 2 data that showed clear benefits in combination with an approved VEGF inhibitor (standard of care). Upon its commercialization, we see Fovista eventually capturing ~25%/~10% share of US/EU wAMD patients on VEGF therapy and generating peak US/EU sales of ~\$1.75B/\$500M. Indications for Fovista beyond wAMD (not modeled) and/or proof-of-concept for Phase 2 asset Zimura (we do not value) could generate additional upside.

# **Price Target Calculation**

We value Ophthotech using a discounted cash flow (DCF) analysis with a WACC of 12.5% and a 0% terminal growth rate post-2030, yielding a \$95 price target (terminal value of \$405M). Our valuation framework utilizes a 12.5% discount rate for pre-commercial stage companies that have achieved clear Phase 2 proof-of-concept.

# **Key Risks to Price Target**

1) Universal failure across all Phase 3 trials or a range of split outcomes could jeopardize FDA and/or EMA approval, and/or require additional clinical work that delays cash flows and/or creates an unexpected financing gap. 2) Although members of management have experience with launching back-of-the eye ophthalmic products (CEO David Guyer was CEO of EyeTech, which developed and launched Macugen), Ophthotech as an independent corporation has yet to establish a commercial infrastructure, distribution capabilities, or market any drug. 3) Data only in 2016 could mean the stock retains a large discount to our estimated intrinsic valuation, creating risks around opportunity cost. 4) Share gains among anti-VEGF treated patients could be weaker than projected if cost over Lucentis and Eylea and Avastin and the requirement for a second intravitreal injection emerge as greater headwinds than we currently believe. 5) More rapid development and approval of competitive PDGF inhibitors, including products possibly co-formulated with existing anti-VEGF agents, could pressure Fovista share. 6) Regulators could require additional non-clinical trials or require changes to the Phase 3 clinical program for Fovista (endpoints, enrollment criteria, selection of anti-VEGF drugs) that could delay progress of the Phase 3 program relative to the 2016 timeframe for top-line data, magnifying the opportunity cost.

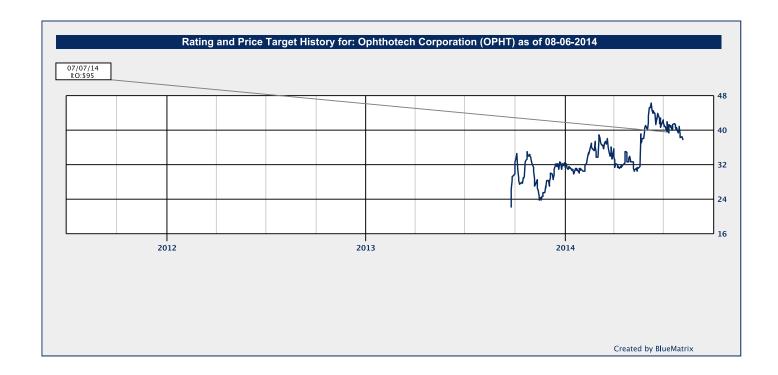
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			IB Serv/Pa	st 12 Mos.
Rating	Count	Percent	Count	Percent
OUTPERFORM [O]	312	51.66	141	45.19
PERFORM [P]	282	46.69	98	34.75
UNDERPERFORM [U]	10	1.66	3	30.00
• •				

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