

# **COMPANY UPDATE**

September 9, 2014

Stock Rating:

# **OUTPERFORM**

12-18 mo. Price Target	\$95.00
OPHT - NASDAQ	\$39.36

NM
\$47.99-\$22.61
33.3M
17.8M
\$1,318.0M
255,137
NA/NM
NA
Dec
NA
NA
NA
\$179M
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	0.47	0.43	(0.92)	NM
Prior (E)			(0.72)	1.82	(0.73)	NM
2015E					(2.19)	NM
Prior (E)					(1.99)	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	40.1	40.1	80.2	NM
Prior (E)			0.0	86.7	86.7	NM
2015E					36.6	NM
Prior (E)					43.3	NM

HEALTHCARE/BIOTECHNOLOGY

# **Ophthotech Corporation**

Novartis Milestones Rolling in Ahead of Schedule

#### **SUMMARY**

An earlier than expected initial \$50M Phase 3 enrollment milestone from Novartis 1) highlights an underappreciated stock driver with another \$80M in enrollment milestones coming (we model next \$50M in 4Q14), 2) demonstrates that recent suggestions of Phase 3 enrollment timelines possibly slipping are unwarranted, 3) reflects solid execution on the Fovista Phase 3 program, and 4) reaffirms timelines for Phase 3 data in 2016. Ophthotech has now achieved ~40% of the \$130M in Phase 3 enrollment milestones achievable from Novartis, a goal we had only assumed would be met in 4Q14. Management indicated the current \$50M milestone reflects progress across the entire Phase 3 program and is not tied to completion of enrollment in any particular trial (Exhibit 1).

#### **KEY POINTS**

- Next Novartis milestone could be around corner. Confirmed enrollment progress with the first Novartis milestone earned supports our YE14 projection for enrollment completion in the two Fovista+Lucentis trials, triggering another ~ \$50M (our assumption). These two trials (N=622 each) started August 2013 and with >225 sites, ~3 patients/site/trial over ~1.5 years looks achievable.
- **2H data catalysts.** This Friday, Ophthotech will present additional Phase 2b data at the Retina Society Annual Meeting (September 12) on biomarkers associated with subretinal fibrosis and neovascular regression. Next month, we expect data at the American Academy of Ophthalmology (AAO) Meeting (October 21) reporting a subgroup analysis from the Phase 2b on fibrosis reduction.
- Fovista Phase 2b response durability. Some investors may have missed a new Fovista data point at the American Society of Retinal Specialists meeting last month (page 3). In patients with visual loss or significant gain at 4 weeks, continued anti-VEGF only yielded visual stabilization at 24 weeks, whereas Fovista+anti-VEGF drove continued gains (Exhibit 2).
- Model changes. We had assumed ~2/3 or ~\$87M of the \$130M in Novartis milestones would hit in 4Q14 when the Fovista/Lucentis trials reach full enrollment. We now assume another ~\$50M in 4Q14, with the remaining ~\$30M in 2015 tied to enrolling the Fovista/Eylea/Avastin Phase 3. 3Q14 EPS to \$0.47 from (\$0.72) with the \$50M milestone.

## Stock Price Performance

# 1 Year Price History for OPHT 48 40 32 24 22 23 31 2014 Created by BlueMatrix

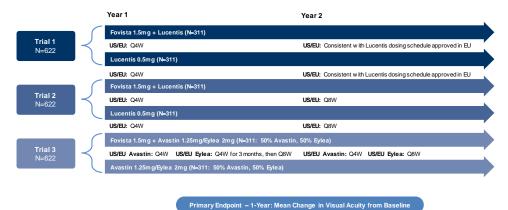
## **Company Description**

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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# Exhibit 1

# Fovista Phase 3 Program in wAMD



Sources: Ophthotech Reports, Oppenheimer Research. Under certain circumstances suggesting visual decline (> 5 decrease in ETDRS or negative findings on fluorescein angiography or SD-OC), treatment at the alternate month visit is permitted on the Q8W schedule.

Exhibit 2

ASRS data demonstrate increased durability of Fovista+Lucentis vs. Lucentis

monotherapy at 24 weeks

Patients with significant visual gain at 4 weeks (≥ 15 letters)	Fovista+Lucentis	Lucentis
Letters gained	2.0	0.2
% additional patients with complete resolution of subretinal hyperreflective material (SHRM)	21%	0%
Patients with visual loss at 4 weeks (≥ 0 Letter Loss)	Fovista+Lucentis	Lucentis
Letters gained	3.4	0.3
% of patients with increased fibrous	24%	47%

Sources: Peter Kaiser, MD. Outcomes in Patients with Visual Gain or Any Loss 1 Month after Combination Therapy of anti-PDGF-β Plus Ranibizumab Compared to Ranibizumab Monotherapy.



Exhibit 3

#### **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 1.642 Fovista US Revenue 111 729 1.700 1.755 1.806 Fovista EU Revenue (Novartis Royalty) 55.6 95.9 112.7 115.9 111.3 105.4 98.3 Novartis Milestones 80.2 36.6 6.6 206.6 0.0 0.0 0.0 50.0 50.0 50.0 50.0 Fovista WW Revenue 80.2 36.6 6.6 326.6 784.5 1,288.8 1,626.5 1,807.9 1,861.4 1,910.7 1,954.6 4849.0% 140.2% 64.3% 26.2% 11.2% 3.0% 2.6% 2.3% % change v-o-v Operating expenses: Cost of sales 21.2 138.5 226.6 287.6 312.0 323.0 333.5 343.2 112.9 Drug COGS 8.9 56.5 89.5 109.8 114.9 114.8 114.1 7.8% 7.5% 7.3% 7.0% 6.8% 6.3% 8.0% 6.5% 137.2 177.9 197.0 208.3 219.4 230.3 Rovalties 12.2 82.0 COGS + Royalties % of revenue 0.0% 0.0% 0.0% 6.5% 17.7% 17.6% 17.7% 17.3% 17.4% 17.5% 17.6% 1,611.4 **Gross Profit** 0.0 0.0 0.0 80.2 36.6 6.6 305.5 646.0 1,062.1 1,338.9 1,496.0 1,538.4 1,577.2 Gross margin 100.0% 100.0% 100.0% 93.5% 82.3% 82.7% 82.6% 82.5% 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 n.a n.a. n.a n.a. n.a. n.a 29.6% 11.9% 7.0% 5.4% 4.7% 4.4% 4.2% 4.0% 25.6 General and administrative 5.7 6.9 51.9 113.3 115.9 118.7 121.7 124.8 128.0 131.4 14.2 24.4 90.7 % of revenue n.a n.a. n.a n.a. n.a. n.a 27.8% 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% 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 n.a n.a. 138.3% 309.9% 2335.2% 63.9% 44.0% 33.6% 30.4% 28.7% 28.5% 28.3% 28.3% Operating income/(loss) (19.6)(13.7)(47.4) (30.7 (76.8 (147.5 96.9 300.9 629.3 845.1 977.6 1.008.3 1.035.7 1.058.9 Operating margin n.a n.a. n.a 38.3% -209.9% -2235.2% 29.7% 38.4% 48.8% 52.0% 54.1% 54.2% 54.2% 54.2% (0.5)(1.5)0.0 Interest income/(expense) 0.0 0.1 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 1.5 1.2 0.9 2.6 4.8 7.7 11.1 14.6 18.2 Other income 0.0 0.0 0.0 1.3 Other gain (loss) (0.0)(0.4)(1.2)0.0 Change in fair value related to investor rights liability 0.0 98.2 631.9 1.077.0 Net income/(loss) before Taxes (19.7)(14.6)(51.1) (30.6)(75.3)(146.4 301.8 849.9 985.3 1.019.4 1.050.3 377.0 Income Tax 1.0 0.0 0.0 0.0 0.0 0.0 0.0 21.9 221.2 297.5 344.9 356.8 367.6 Effective tax rate (%) 5.2% 0.0% 0.0% 0.0% 0.0% 0.0% 0.0% 35.0% 35.0% 35.0% 35.0% 35.0% 35.0% Net income/(loss) (18.6)(14.6)(51.1) (30.6 (75.3) (146.4 98.2 323.7 410.7 552.4 640.4 662.6 682.7 700.1 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 Net income/(loss) to common shareholders (25.5)(21.6)(57.0)(30.6 (75.3)(146.4 98.2 323.7 410.7 552.4 640.4 662.6 682.7 700.1 Basic EPS (\$18.27) (\$14.89) (\$6.34) (\$0.92) (\$2.19) (\$4.08) \$2.67 **\$8.70** 37.2 \$10.90 \$14.51 \$16.67 \$17.13 \$17.57 \$17.94 35.4 38.4 43.2 44.4

33.2

37.1

39.4

Sources: Oppenheimer Research Estimates, Ophthotech Filings.

Diluted number of shares

9

43.7

Exhibit 4

# **Ophthotech Balance Sheet**

in millions of \$ except per share values

in millions of \$ except per share values		1	-		-	-		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
ASSETS														
Current assets														
Cash and cash equivalents	6.4	4.3	210.6	301.4	232.2	262.5	178.7	522.8	962.8	1,543.3	2,218.7	2,913.3	3,632.0	4,372.7
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	499.1	430.8	314.4	234.9	590.0	1,037.0	1,622.4	2,299.7	2,995.2	3,714.9	4,456.5
Property, plant and equipment, net	0.1	0.0	0.0	2.0	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	536.3	469.6	355.1	277.5	634.2	1,082.1	1,668.1	2,345.7	3,041.3	3,760.9	4,502.6
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)				219.8	213.2	206.6	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	256.7	221.5	217.2	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	340.0	304.8	300.5	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)	(219.4)	(281.2)	(412.7)	(298.2)	43.4	473.9	1,048.1	1,712.5	2,401.5	3,113.1	3,845.0
Accumulated other comprehensive income				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	196.2	164.7	54.6	181.6	532.7	977.0	1,560.5	2,237.2	2,932.4	3,651.7	4,392.9
Total liabilities & Shareholders equity	7.7	4.9	217.7	536.3	469.6	355.1	277.5	634.2	1,082.1	1,668.1	2,345.7	3,041.3	3,760.9	4,502.6

Sources: Oppenheimer Research Estimates, Ophthotech Filings.



Exhibit 5
Ophthotech Cash Flow Statement

in millions of US\$ 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 Cash Flow Statement Operating cash flows (18.6) (14.6 (51.1 (42.8) (75.3) (146.4) 98.2 323.7 410.7 552.4 640.4 662.6 682.7 700.1 Net loss Adjustments Depreciation 0.0 0.0 0.0 0.4 0.8 1.3 2.0 2.7 3.4 3.8 4.1 4.3 4.4 Amortization of debt issuance costs 0.0 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 Changes in operating assets & liabilities 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) Prepaid expense and other current assets Other receivables 0.0 (0.7 1.0 0.0 0.0 0.0 0.0 0.0 0.0 0.1 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 Security deposits (0.1 Accrued clinical drug supplies and trial costs (0.8) 0.6 (0.1) (0.2)(0.1 (0.1) (0.1) Accounts payable and accrued expenses 0.2 (0.2 2.4 0.5 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 219.8 (6.6) (6.6) (206.6) 0.0 0.0 0.0 0.0 0.0 0.0 0.0 Deferred rent (0.0 (0.0) 0.0 (48.8 Net cash provided by (used in) operating activities (19.1)(13.1)199.3 (97.1) (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 (2.3) (2.4) (3.9) (4.3) Purchase of property, equipment (0.0) (0.0) (3.2 (4.4 (4.3)(4.3)(4.4)(4.4 (4.4 3.4 0.0 146.8 (4.4) (4.4) (4.4) (4.4) Net cash provided by (used in) investing activities (0.0) (207.2) (2.4) (3.9) (4.3) (4.3) (4.3) Financing cash flows Payment of debt issuance costs Proceeds from issuance of common stk 0.0 0.0 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 Proceeds from royalty purchase agreement 41.7 41.7 15.0 11.0 255.1 98.6 30.3 12.5 13.8 12.3 9.4 Net cash provided by (used in) financing activities 21.3 9.5 9.3 6.2 7.6 206.3 90.8 (69.2 30.3 344 1 440.0 694.6 718.7 740.7 Net change in cash & cash equivalents (0.7 (2.1 2,218.7 Net cash & cash equivalents - opening balance 6.4 4.3 210.6 301.4 232.2 262.5 178.7 522.8 1,543.3 2,913.3 3,632.0 Net cash & cash equivalents - ending balance 962.8 1,543.3 2,913.3 4,372.7

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.

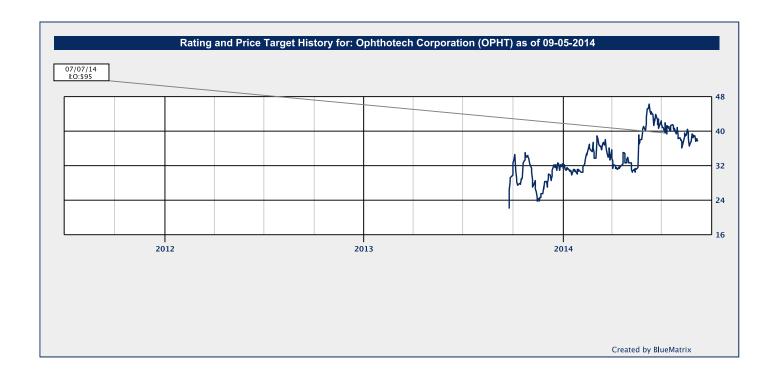
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Perform (P) - Stock expected to perform in line with the S&P 500 within the next 12-18 months.

Underperform (U) - Stock expected to underperform the S&P 500 within the next 12-18 months.

Not Rated (NR) - Oppenheimer & Co. Inc. does not maintain coverage of the stock or is restricted from doing so due to a potential conflict of interest.

# Oppenheimer & Co. Inc. Rating System prior to January 14th, 2008:

**Buy -** anticipates appreciation of 10% or more within the next 12 months, and/or a total return of 10% including dividend payments, and/or the ability of the shares to perform better than the leading stock market averages or stocks within its particular industry sector.

**Neutral** - anticipates that the shares will trade at or near their current price and generally in line with the leading market averages due to a perceived absence of strong dynamics that would cause volatility either to the upside or downside, and/or will perform less well than higher rated companies within its peer group. Our readers should be aware that when a rating change occurs to Neutral from Buy, aggressive trading accounts might decide to liquidate their positions to employ the funds elsewhere.

**Sell** - anticipates that the shares will depreciate 10% or more in price within the next 12 months, due to fundamental weakness perceived in the company or for valuation reasons, or are expected to perform significantly worse than equities within the peer group.

	Dis	tribution	of Rating
		IB Serv/Pa	st 12 Mos.
Count	Percent	Count	Percent
317	52.14	145	45.74
281	46.22	97	34.52
10	1.64	3	30.00
	317 281	Count         Percent           317         52.14           281         46.22	317 52.14 145 281 46.22 97

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