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May 20, 2014

Stock Rating
Overweight
Industry View
In-Line

Ophthotech Corp

Fovista ex-US Deal With Novartis a Positive

What's Changed

Price Target

\$79.00 to \$99.00

OPHT licensed ex-US Fovista rights to NVS, with the nice deal terms positively impacting our PT.

OPHT announced an ex-US commercialization agreement with Novartis for Fovista. OPHT retains US rights to Fovista. While we have always modeled that OPHT would partner ex-US rights, this deal is more favorable than we had anticipated with larger milestones and a higher royalty rate.

Deal Terms: We now model OPHT receiving a few tranches of cash from NVS, including a) an upfront of \$200mn, b) Ph 3 enrollment based milestones of up to \$130mn, and c) ex-US approval based milestones of \$300mn. There are also ~\$400mn of sales milestones which we do not model. The royalty rate on ex-US Fovista monotherapy sales will be in the mid-30% (we assume 35%). We view this as a solid deal. OPHT remains financially responsible for the Ph 3 program, but Novartis 1) will pay for all other ex-US clinical development, 2) will provide Lucentis in the ongoing Ph 3 and other trials, and 3) is fully responsible for coformulated product and prefilled syringe development. OPHT will have the option to market the combination product and pre-filled syringe in the US.

Novartis as Good Strategic Partner: Novartis has an established sales force marketing Lucentis ex-US. Additionally, Novartis may potentially develop 1) a prefilled Fovista syringe, and 2) a fixed combination of VEGF + Fovista. We see the fixed combo product as useful for the portion of physicians that would prefer a "closed" combination, but do not view this as critical to Fovista success. A closed product would have a lower royalty rate to OPHT but a higher selling price, implying a net to OPHT similar to the monotherapy royalty rate. Overall, we view NVS as a solid partner for Fovista.

US: OPHT retains US Fovista rights. With this deal, OPHT could opt-in to commercialize a VEGF + Fovista combo product and/or a pre-filled Fovista syringe.

Model Changes: We updated our model for the favorable upfront/milestones, mid-30% royalty, a more detailed salesforce build, and updated tax rate.

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Key Ratios and Statistics

Reuters: OPHT.O Bloomberg: OPHT US Biotechnology / United States of America

\$99.00
\$31.46
\$951
\$42.54-22.61

Fiscal Year ending	12/13	12/14e	12/15e	12/16e
ModelWare EPS (\$)	(5.67)	(2.52)	(2.37)	(2.60)
Prior ModelWare EPS (\$)	-	(2.83)	(3.26)	(0.19)
P/E	NM	NM	NM	NM
Consensus EPS (\$)§	(5.39)	(2.98)	(3.64)	(2.30)
Div yld (%)	0.0	0.0	0.0	0.0

Unless otherwise noted, all metrics are based on Morgan Stanley ModelWare framework (please see explanation later in this note).

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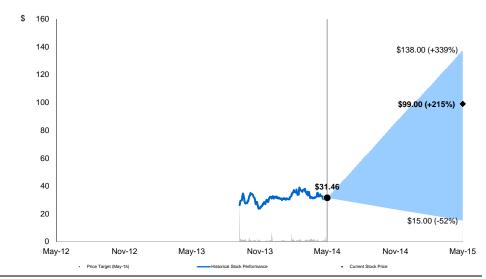
For analyst certification and other important disclosures, refer to the Disclosure Section, located at the end of this report.

^{§ =} Consensus data is provided by Thomson Reuters Estimates.

e = Morgan Stanley Research estimates

Risk-Reward Snapshot: Ophthotech (OPHT, OW, PT \$99)

Fovista's Success Drives Risk-Reward



Source: Morgan Stanley Research estimates, Thomson Reuters

Price Target \$99 We derive our PT from a discounted cash flow analysis that uses a WACC of 12.5% and a 0% terminal growth rate. The revenue driver in our model is the WW launch of Fovista in wet AMD in 2H17 (US) and 2018 (EU/ROW).

Bull	DCF
Case	
\$138	

Fovista gains significant share in the wet AMD market. Limited competition from earlier stage anti-PDGF/VEGF combinations. Our bull case assumes that Fovista gains ~45% share of the overall wet AMD market. This share is driven by Fovista use in ~55% of Lucentis treated eyes, ~30% of Eylea treated eyes and ~45% of Avastin treated eyes. This scenario assumes 1) Regeneron's anti-PDGF/Eylea combination makes it to market by ~2020, but does not take significant share from Fovista, and 2) Fovista is able to gain meaningful traction in combination with Avastin despite its likely high cost per injection. We model WW Fovista sales of ~\$3bn with ~\$1.7bn in sales in the US.

Base DCF Case \$99

Fovista gains decent share in the wet AMD market, but loses some share to competition in 2020 and beyond. Our base case scenario assumes Fovista approval and ~30% peak share of the overall wet AMD market. This share is made up of Fovista use in ~45% of Lucentis treated eyes, ~25% of Eylea treated eyes at peak and ~35% of Avastin treated eyes. This scenario assumes 1) Regeneron's anti-PDGF/Eylea combination launches in 2020 and rapidly takes share from Fovista, and 2) Fovista use in combination with Avastin is less than that with the other two anti-VEGF therapies as patients treated with Avastin are often those that are unable to afford Lucentis/Eylea, and these patients may also be unable to afford the addition of Fovista. We model peak WW Fovista sales of ~\$2bn with ~\$1.2bn sales in the US.

Bear Cash Based Case Value \$15

Fovista fails. Our bear case assumes Fovista fails in its Ph 3 wet AMD trials either due to insufficient efficacy or safety concerns. Given that Fovista is Ophthotech's only late stage asset, we would expect the stock to trade at or near cash in the case of Ph 3 failure. We view this scenario as unlikely given Fovista data to date.

Investment Thesis

- We are OW OPHT as we believe the company's lead asset Fovista has encouraging data in wet AMD to date and WW sales potential of ~\$2bn.
- Fovista, a PDGF inhibitor, is in Ph 3 in combination with anti-VEGF therapy for the treatment of wet AMD. Ph 2b data suggest Fovista + anti-VEGF leads to greater vision improvement than anti-VEGF therapy alone.
- Fovista's Ph 3 program largely mimics its Ph 2b and we expect success in 2016, followed by a 2016 NDA filing and potential launch in 2017.
- While anti-VEGF therapies work well in many pts with wet- AMD, ~20% of pts initiating treatment with these drugs continue to lose vision over the course of a year and most pts have progressive vision loss over time.
- Physician feedback suggests that 1) all pts with wet AMD should be treated, 2) the goal of treatment is to provide the greatest letter benefit upfront, and 3) a gain of even 2-3 letters makes a difference. We model >\$1bn peak sales in the US and ~\$500mn+ ex-US.
- Ophthotech is considering exploratory trials for Fovista in add'l indications such as wet AMD VEGF failures, proliferative vitreoretinopathy, and von Hippel Lindau disease. These indications as well as any potential success from Ophthotech's earlier stage asset, ARC1905, are upside to our model.

Risks to our price target

1) Fovista could fail in Ph 3 either due to insufficient efficacy or a safety issue, 2) Fovista does not yet have data in combination with Eylea or Avastin and it is possible that 1) these combos show different results than the Lucentis + Fovista combination and/or 2) the FDA or EMEA could require add'l data.

Ophthotech Valuation

Exhibit 1

DCF Drives Valuation

	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E
Free Cash Flow	(\$49)	\$115	\$32	(\$105)	\$53	\$457	\$497	\$412	\$478	\$554	\$647	\$675	\$682	\$683	\$547	\$437	\$350
YoY Growth		-336%	-72.6%	-433.9%	-151%	756.8%	8.9%	-17.1%	16.0%	15.8%	16.9%	4.2%	1.1%	0.3%	-20.0%	-20.0%	-20.0%
Net Cash Proxy for Dilution	(\$4.38)	(\$1.79)	(\$2.3)	(\$3.0)	(\$3.9)	(\$4.2)	(\$4.3)	(\$5.3)	(\$6.6)	(\$8.2)	(\$10.2)	(\$10.2)	(\$10.2)	(\$10.2)	(\$10.2)	(\$10.2)	(\$10.2)
Free Cash Flow for DCF	(\$53.2)	\$113.3	\$29.2	(\$108.3)	\$49.4	\$452.5	\$493.1	\$406.8	\$471.6	\$545.7	\$637.1	\$664.4	\$671.5	\$673.3	\$536.6	\$427.3	\$339.8
Present Value of Free Cash Flow		\$113.3	\$26.8	(\$88.2)	\$35.7	\$291.0	\$281.8	\$206.7	\$213.0	\$219.1	\$227.3	\$210.7	\$189.3	\$168.7	\$119.5	\$84.6	\$59.8

Source: Company data, Morgan Stanley Research estimates

Exhibit 2

DCF Valuation Suggests Upside

Valuation Date	2014.25
Discount Rate	12.5%
Terminal Growth Rate	0%
Terminal Value Year	2029
Sum of Discounted FCF	\$2,331
Discounted Terminal Value	\$478
Net Cash	\$491
Equity Value	\$3,300
Equity Value/Sh	\$99
Shares Outstanding (Basic)	33.3

Source: Company Data, Morgan Stanley Research estimates

\$99 PT includes Fovista in wet AMD.

We derive our PT from a discounted cash flow (DCF) analysis that uses a WACC of 12.5% and a terminal growth rate of 0% post 2029. We incorporate the cash cost of stock options

Valuation Methodology: We use a DCF to value Ophthotech as well as most other companies under coverage. We believe a DCF best captures the long-term nature of drug development and commercialization. We do not feel that a multiples analysis accomplishes the same goal, as it only evaluates a company during a snapshot in time.

Discount Rate: We typically apply a discount rate of 12.5% to development stage companies that have good, company-sponsored proof of concept data.

Terminal Growth Rate: Our modeled cash flows extend to 2023. Beyond this point, we grow free cash flows from 2023-27 at 25% of the prior year's growth rate. In 2027-29 we

decline cash flows by 20% per year to account for the potential presence of a generic Fovista after the 2026 patent expiry. Beyond 2029, we use a terminal growth rate of 0%.

Revenue: The revenue driver in our model is Fovista.

Economics: Ophthotech has US rights to Fovista. Ophthotech licensed ex-US rights to Novartis with mid-30s% royalty. Ophthotech also has several low single digit royalty obligations on Fovista sales. 1) For rights to anti-PDGF aptamers, Ophthotech owes OSI a royalty at a low single digit percentage of net sales – we estimate 2.5%. 2) For use of Nektar's technology, Ophthotech is obligated to pay Nektar tiered royalties at low to mid-single digit percentages of net sales – we estimate 2-4%. 3) In conjunction with a May 23rd, 2013 financing, Novo AS gained rights to a low to mid single digit percentage of net sales. This financing can be drawn in three separate tranches with additional royalty obligations, with each tranche – we assume Ophthotech uses 2/3 tranches and a corresponding 4% royalty obligation.

COGS: We assume a drug cost of ~5-7% of sales, which in addition to royalty obligations leads to total COGS of ~20%.

Operating Expenses:

R&D: We expect R&D to increase over the next few years as Ophthotech runs the Fovista Ph 3 program (1 yr data in 2016). Post 2016, we expect R&D to begin to decline.

SG&A: We expect SG&A to modestly increase through 2017. We expect a sig. increase in 2017+ as Ophthotech builds a US infrastructure to market Fovista.

Key Risks Include: 1) Fovista fails to demonstrate sufficient efficacy and safety for approval, 2) the FDA and EMEA deem one trial with Avastin and Eylea as insufficient for approval and require additional data, which could result in an approval delay for these combinations, 3) Ophthotech has difficulty finding an ex-US partner, which could result in additional

MORGAN STANLEY RESEARCH

May 20, 2014 Ophthotech Corp

financing needs, 4) the commercial potential for Fovista is more limited than we expect if a) two injections are logistically difficult in the real-world, b) two injections are a bigger hurdle for patients and/or payors than we anticipate, or c) competition has a larger impact than we expect.

Exhibit 3

Ophthotech Upcoming Catalysts

Drug	Туре	Event	Expected Timing
Fovista	Product Advancement	Begin trial in anti-VEGF resistant patients	2014
Fovista	Product Advancement	Begin trial in subretinal fibrosis wet AMD patients	2014
Fovista	Product Advancement	Begin trial in von Hippel Lindau disease	2014
Zimura (C5)	Product Advancement	Begin Ph 2/3 trial in geographic atrophy (severe form of dry AMD)	Late 2014/Early 2015
Fovista	Product Advancement	Begin trial in proliferative vitreoretinopathy	2015
Fovista	Clinical Data	Data from potential exploratory trials in other indications	2015
Zimura (C5)	Product Advancement	Begin trial in combo with Fovista in anti-VEGF resistant wet AMD pts with complement component	2015
Fovista	Clinical Data	Ph 3 trials with Lucentis, Eylea and Avastin	2016
Fovista	Regulatory	File NDA and MAA for Fovista in wet AMD	2H16
Fovista	Product Advancement	Begin small registrational trial in Japan	2017
Fovista	Regulatory	Fovista approval	2H17

Source: Company Data, Morgan Stanley Research

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May 20, 2014 Ophthotech Corp

Exhibit 4

Ophthotech Changes to Model

Ophthotech C												
	2012	2013	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
US Fovista												
Current	0	0	0	0	0	295	597	755	806	929	1,045	1,199
Prior	0	0	0	0	0	295	597	755	806	929	1,045	1,199
EU Fovista												
Current	0	0	0	0	0	0	229	458	571	557	605	639
Prior	0	0	0	0	0	0	229	458	571	557	605	639
Total Revenu	es (\$mn)										
Current	0	0	11	28	28	324	741	1,024	1,135	1,251	1,388	1,558
Prior	0	0	0	0	100	295	651	865	957	1,076	1,204	1,368
COGS (\$mn)												
Current	0	0	0	0	0	46	113	185	203	220	244	273
Prior	0	0	0	0	0	46	113	185	203	220	244	273
SG&A (\$mn) -	ex-ESO	s										
Current	6	13	24	25	31	112	115	119	123	127	130	133
Prior	6	13	24	24	25	81	142	163	179	179	179	179
R&D (\$mn) -e:	x-ESOs											
Current	7	31	64	74	74	78	78	71	64	57	51	46
Prior	7	31	64	74	74	78	78	71	64	57	51	46
Interest and C	Other Inc	come, n	et									
Current	(1)	(1)	0	1	1	1	2	3	4	5	6	8
Prior	(1)	(1)	0	0	0	1	1	2	3	4	5	7
Net Income (БММ) - N	on GAA	P, exclu	ıdes opti	ions							
Current	(14)	(48)	(77)	(70)	(76)	89	360	424	487	554	630	724
Prior	(14)	(48)	(88)	(98)	2	91	282	328	375	455	536	639
EPS Non-GAA	AP, Dilut	ed, exc	ludes op	otions								
Current	(\$1.62)	(\$5.35)	(\$2.34)	(\$2.16)	(\$2.35)	\$2.63	\$10.61	\$12.35	\$14.04	\$15.77	\$17.71	\$20.07
Prior	(\$1.62)	(\$5.35)	(\$2.65)	(\$3.04)	\$0.04	\$2.48	\$7.62	\$8.77	\$9.96	\$11.94	\$13.90	\$16.36
EPS - GAAP												
Current	(\$2.52)	(\$6.33)	(\$2.52)	(\$2.37)	(\$2.60)	\$2.34	\$10.32	\$12.11	\$13.78	\$15.49	\$17.42	\$19.77
Prior	(\$2.52)	(\$6.33)	(\$2.83)	(\$3.26)	(\$0.19)	\$2.20	\$7.33	\$8.52	\$9.69	\$11.65	\$13.60	\$16.04
Basic Shares	Outstan	ding										
Current	9	9	33	32	32	32	33	33	33	33	33	33
Prior	9	9	33	32	34	35	36	36	36	36	36	36
Dilluted Shar	es Outst	anding										
Current	9	9	33	32	32	34	34	34	35	35	36	36
Prior	9	9	33	32	35	37	37	37	38	38	39	39

Source: Company Data, Morgan Stanley Research

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May 20, 2014 Ophthotech Corp

Exhibit 5 Ophthotech Quarterly Income Statement					
(\$ in millions)	1Q14A	2Q14E	3Q14E	4Q14E	2014E
Other Revenue		2.1	4.2	4.2	10.5
Revenues	\$0.0	\$2.1	\$4.2	\$4.2	\$10.5
Operating Expenses:					
Cost of Sales					
% total product sales	NA	NA	NA	NA	NA
R&D	13.4	15.0	17.0	19.0	64
% of revenue	na	na	na	na	na
SG&A	5.9	6.0	6.0	6.0	24
% of revenue	na	na	na	na	na
Total Operating Expenses	\$19.3	\$21.0	\$23.0	\$25.0	\$88.3
Operating Income (Loss)	(\$19.3)	(\$18.9)	(\$18.8)	(\$20.8)	(\$77.8)
Operating Margin	na	-900.0%	-447.6%	-495.2%	-741.2%
Interest income	\$0.0	\$0.10	\$0.10	\$0.10	\$0.34
Interest expense	\$0.0	\$0.00	\$0.00	\$0.00	\$0.00
Fx gain (loss)	\$0.0	\$0.00	\$0.00	\$0.00	\$0.00
Other loss	\$0.0	\$0.00	\$0.00	\$0.00	\$0.00
Change in fair value related to inv. rights liabil					
Pretax Income (Loss)	(\$19.3)	(\$18.8)	(\$18.7)	(\$20.7)	(\$77.5)
Provision for Income Taxes	0	0	0	0	0
Effective tax rate					
Net Income (Loss)	(\$19.3)	(\$18.80)	(\$18.70)	(\$20.70)	(\$77.48)
EPS, basic	(\$0.60)	(\$0.56)	(\$0.56)	(\$0.62)	(\$2.34)
EPS, diluted	(\$0.60)	(\$0.56)	(\$0.56)	(\$0.62)	(\$2.34)
Options Expense	1.40	1.50	1.50	1.50	5.90
% of operating expense	7.2%	7.1%	6.5%	6.0%	6.7%
Tax benefit from options					
Net Income (inc. options expense)	(\$20.7)	(\$20.30)	(\$20.2)	(\$22.2)	(\$83.4)
EPS, diluted (inc. options expense)	(\$0.64)	(\$0.61)	(\$0.60)	(\$0.66)	(\$2.52)
Basic Shares Outstanding	32.28	33.33	33.43	33.53	33.14
Diluted Shares Outstanding	32.28	33.33	33.43	33.53	33.14
One times	\$0.0	\$0.00	\$0.00	\$0.00	\$0.0
GAAP Net Income (incl. options)	(\$20.682)	(\$20.3)	(\$20.2)	(\$22.2)	(\$83.4)
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Source: Company Data, Morgan Stanley Research

MORGAN STANLEY RESEARCH

May 20, 2014 Ophthotech Corp

(\$ in millions)	2011A	2012A	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
US Fovista			201071		\$0.0	\$0.0	\$295.1	\$597.3	\$755.1	\$806.4	\$928.6	\$1,044.6	\$1,199
EU Fovista					\$0.0	\$0.0	\$0.0	\$229.2	\$457.7	\$571.4	\$557.3	\$604.8	\$639.4
ROW Fovista					\$0	\$0	\$0	\$46	\$114	\$171	\$167	\$181	\$192
Total WW Fovista Sales					\$0	\$0	\$295	\$872	\$1,327	\$1,549	\$1,653	\$1,831	\$2,030
EU Fovista Royalties					\$0.0	\$0.0	\$0.0	\$80.2	\$160.2	\$200.0	\$195.1	\$211.7	\$223.8
ROW Fovista Royalties					\$0	\$0	\$0	\$16	\$40	\$60	\$59	\$64	\$67
Total Fovista Royalties					\$0	\$0	\$0	\$96	\$200	\$260	\$254	\$275	\$291
Other Revenue				11	28	28	28	47	69	69	69	69	69
Total Revenue	\$0	\$0	\$0	\$11	\$28	\$28	\$324	\$741	\$1,024	\$1,135	\$1,251	\$1,388	\$1,558
Operating Expenses:							0%	13%	20%	23%	20%	20%	19%
Cost of Sales	\$0	\$0	\$0	\$0	\$0	\$0	\$46	\$113	\$185	\$203	\$220	\$244	\$273
% of US sales- Drug cost	NA	NA	NA	NA	NA	7.0%	7.0%	6.5%	6.0%	5.0%	5.0%	5.0%	5.0%
% of US sales - total COGS (incl. royalties)							16%	19%	24%	25%	24%	23%	23%
R&D	\$14	\$7	\$31	\$64	\$74	\$74	\$78	\$78	\$71	\$64	\$57	\$51	\$46
YoY growth	-6%	-51%	359%	107%	15%	0%	-5%	-5%	-10%	-10%	-10%	-10%	-10%
% of revenue	na	na	na	613%	260%	260%	24%	11%	7%	6%	5%	4%	3%
SG&A	\$5	\$6	\$13	\$24	\$25	\$31	\$112	\$115	\$119	\$123	\$127	\$130	\$133
YoY growth	27%	14%	114%	79%	5%	25%	256%	3%	3%	3%	4%	2%	2%
% of revenue	na	na	na	228%	88%	110%	35%	16%	12%	11%	10%	9%	9%
Total Operating Expenses	\$19.39	\$13.04	\$45	\$88	\$99	\$105	\$236	\$307	\$374	\$389	\$404	\$426	\$453
Operating Income (Loss)	-\$19	-\$13	-\$45	-\$78	-\$71	-\$77	\$88	\$434	\$650	\$746	\$846	\$962	\$1,106
Operating Margin	na	na	na	na	na	na	27%	59%	63%	66%	68%	69%	71%
Interest income	\$0.002	\$0.0	\$0.0	\$0.3	\$1.1	\$1.0	\$0.9	\$1.6	\$2.8	\$3.9	\$5.1	\$6.4	\$7.9
Interest expense	\$0.00	-\$0.5	-\$1.5	\$0.0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Fx transaction gain (loss)	-\$0.02	\$0.0	\$0.0	\$0.0									
Non cash change in fair value (inv. rights liabil.	,	-\$0.4	-\$2.3	\$0.0									
Pretax Income (Loss)	(\$19.41)	(\$13.9)	(\$48.3)	(\$77.5)	(\$69.6)	(\$76.0)	\$88.63	\$435.77	\$652.66	\$749.60	\$851.55		\$1,113.56
Provision for Income Taxes	(1.03)	0.0	0.0	0.0	0.0	0.0	0.0	75.4	228.4	262.4	298.0	339.1	389.7
Effective Tax Rate		0%	0%	0%	0%	0%	0.0%	17%	35%	35%	35%	35%	35%
Net Income (Loss)	(\$18.38)	(\$13.9)	(\$48.3)	(\$77)	(\$70)	(\$76)	\$89	\$360	\$424	\$487	\$554	\$630	\$724
EPS, basic	(\$2.23)	(\$1.62)	(\$5.35)	(\$2.34)	(\$2.16)	(\$2.35)	\$2.73	\$11.08	\$13.00	\$14.87	\$16.82	\$19.04	\$21.76
EPS, diluted	(\$2.23)	(\$1.62)	(\$5.35)	(\$2.34)	(\$2.16)	(\$2.35)	\$2.63	\$10.61	\$12.35	\$14.04	\$15.77	\$17.71	\$20.07
EDS diluted final ESOO	(\$2.26)	(\$1.70)	(\$5.67)	(\$2.52)	(\$2.37)	(\$2.60)	\$2.34	\$10.32	\$12.11	\$13.78	\$15.49	\$17.42	\$19.77
EPS, diluted (incl. ESOs) Basic Shares Outstanding	8.23	8.6	9.0	33.1	32.3	32.3	32.4	32.5	32.6	32.8	32.9	33.1	33.3
Diluted Shares Outstanding	8.23	8.6	9.0	33.1	32.3	32.3	33.7	34.0	34.3	34.7	35.1	35.6	36.1
One time items	(\$6.8)	(\$7.1)	(\$5.9)	\$0.0									
GAAP Net Income	(\$25.47)	(\$21.6)	(\$57.0)	(\$83.4)	(\$76.6)	(\$84.0)	\$78.6	\$350.5	\$415.8	\$478.1	\$543.8	\$619.3	\$712.8
GAAP EPS, diluted (includes ESOs)	(\$3.10)	(\$2.52)	(\$6.33)	(\$2.52)	(\$2.37)	(\$2.60)	\$2.34	\$10.32	\$12.11	\$13.78	\$15.49	\$17.42	\$19.77

Source: Company Data, Morgan Stanley Research estimates

Exhibit 7 Ophthotech Balance Sheet													
(\$mn)	2011A	2012A	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Assets													
Cash and cash equivalents	\$6.4	\$4.3	\$211	\$423	\$455	\$351	\$405	\$863	\$1,364	\$1,781	\$2,265	\$2,826	\$3,482
Marketable securities	\$0.0	\$0.2	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Other receivables	\$1.0	\$0.0	\$0.0	\$1.2	\$1.3	\$1.3	\$6.5	\$7.4	\$10.2	\$11.4	\$12.5	\$13.9	\$15.6
Prepaid expenses and other deposits	\$0.1	\$0.0	\$7	\$0	\$0	\$0	\$3	\$7	\$10	\$11	\$13	\$14	\$16
Debt issuance costs	\$0.0	\$0.3	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Total current assets	\$7.50	\$4.84	\$217	\$425	\$457	\$352	\$414	\$878	\$1,385	\$1,804	\$2,290	\$2,854	\$3,513
Property, plant and equipment, net	\$0.07	\$0.0	\$0	\$1	\$2	\$3	\$5	\$9	\$13	\$18	\$22	\$27	\$32
Security deposits	\$0.17	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Other long-term assets	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Total assets	\$7.7	\$4.879	\$217.7	\$425.6	\$458.6	\$355.0	\$420.0	\$887.3	\$1,398.3	\$1,821.7	\$2,312.5	\$2,881.1	\$3,545.7
Liabilities and stockholders' equity Notes payable Accrued drug supply and trial cost Accounts payable Accrued compensation Deferred rent Investor rights liability Deferred Revenue (current) Total current liabilities Deferred Revenue (Non-current) Other long-term liabilities Total liabilities	\$0.0 \$1.5 \$0.9 \$0.8 \$0.0 \$0.2 \$0 \$3.3 \$0 \$0.0	\$11 \$1 \$1 \$1 \$0.0 \$1 \$0 \$14.4 \$0 \$0 \$14.4	\$0 \$2 \$4 \$0 \$0.0 \$0 \$6.3 \$0 \$42	\$0 \$2 \$3 \$0 \$0.0 \$0 \$28 \$33.1 \$161 \$83	\$0 \$2 \$3 \$0 \$0.0 \$0 \$28 \$33.7 \$263 \$83 \$380	\$0 \$2 \$3 \$0 \$0.0 \$0 \$28 \$33.9 \$234 \$83 \$351	\$0 \$2 \$7 \$0 \$0.0 \$0 \$47 \$56.7 \$187 \$83	\$0 \$2 \$9 \$0 \$0.0 \$69 \$80.2 \$268 \$83	\$0 \$2 \$11 \$0 \$0.0 \$0 \$69 \$82.0 \$350 \$83	\$0 \$2 \$12 \$0 \$0.0 \$0 \$69 \$82.2 \$281 \$83	\$0 \$2 \$12 \$0 \$0.0 \$0 \$69 \$82.5 \$212 \$83	\$0 \$2 \$13 \$0 \$0.0 \$0 \$69 \$83.0 \$144 \$83	\$0 \$1 \$14 \$0 \$0.0 \$0 \$69 \$83.6 \$75 \$83
Preferred Stock	\$3.0	\$3	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Common stock	\$0.0	\$0.01	\$0.03	\$0.03	\$0.03	\$0.03	\$0.03	\$0.03	\$0.03	\$0.03	\$0.03	\$0.03	\$0.03
APIC	\$0.0	\$0	\$353	\$415	\$422	\$431	\$441	\$453	\$465	\$479	\$495	\$512	\$532
Accumulated deficit	(\$105)	(\$126)	(\$183)	(\$266)	(\$343)	(\$427)	(\$348)	\$2	\$418	\$896	\$1,440	\$2,059	\$2,772
Accumulated other comprehensive income	\$0.0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Total stockholders' equity	\$4	-\$10	\$170	\$148	\$79	\$4	\$93	\$455	\$883	\$1,375	\$1,934	\$2,571	\$3,304
Total liabilities and stockholder's equity	\$7.74	\$4.9	\$218	\$426	\$459	\$355	\$420	\$887	\$1,398	\$1,822	\$2,313	\$2,881	\$3,546

Source: Company Data, Morgan Stanley Research estimates

(\$mn)	2011A	2012A	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Net loss	(\$18.6)	(\$14.562)	(\$51.145)	(\$83.4)	(\$76.6)	(\$84.0)	\$78.6	\$350.5	\$415.8	\$478.1	\$543.8	\$619.3	\$712.8
Depreciation	\$0.03	\$0.03	\$0.0	\$0.1	\$0.2	\$0.3	\$0.5	\$0.8	\$1.3	\$1.7	\$2.3	\$2.9	\$3.5
Amortization and accretion	\$0.00	\$0.1	\$0.1	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Unrealized gain on investments	\$0.00	\$0.0	\$0.1	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Non-cash charge in fair value (inv. rights libil.)	\$0.01	\$0.37	\$1.18	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.0	\$0.00
Loss on extinguishment of debt	\$0.00	\$0.00	\$1.1	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.0	\$0.00
Stock-based compensation	\$0.25	\$0.6	\$2.9	\$5.9	\$7.0	\$8.0	\$10.0	\$9.9	\$8.5	\$9.1	\$9.8	\$10.4	\$11.1
Preferred stock issued for tech and licenses	\$0.50	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0	\$0.0
Changes in operating assets and liabilities:													
Prepaid expenses, other current deposits	\$0.1	\$0	(\$7)	\$7	(\$0)	(\$0)	(\$3)	(\$4)	(\$3)	(\$1)	(\$1)	(\$1.4)	(\$2)
Other receiveables	(\$0.74)	\$1.0	\$0	(\$1)	(\$0)	(\$0)	(\$5)	(\$1)	(\$3)	(\$1)	(\$1)	(\$1.4)	(\$2)
Security deposits	(\$0.0)	(\$0)	(\$0)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0.0	\$0
Accrued drug supply and trial cost	(\$0.8)	(\$0.5)	\$1	(\$1)	\$0	\$0	\$0	\$0	(\$0)	(\$0)	(\$0)	(\$0.2)	(\$0)
Accounts payable and accrued expenses	(\$0.05)	\$0.0	\$2	(\$1)	\$0	\$0	\$4	\$2	\$2	\$0	\$0	\$0.7	\$1
Accrued bonuses	\$0.22	(\$0.2)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0.0	\$0
Deferred rent	(\$0.02)	(\$0)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0.0	\$0
Deferred revenue	\$0.00	\$0	\$0	\$190	\$102	(\$28)	(\$28)	\$103	\$81	(\$69)	(\$69)	(\$69)	(\$69)
Net cash used in operating activities	(\$19.12)	(\$13.10)	(\$49)	\$116	\$33	(\$104)	\$56	\$461	\$503	\$418	\$485	\$562	\$656
Investing Activities:													
Purchases of property, plant and equipment	(\$0.00)	\$0.0	(\$0.0)	(\$0.9)	(\$1.1)	(\$1.3)	(\$3.1)	(\$4.3)	(\$5.6)	(\$6.2)	(\$6.9)	(\$7.7)	(\$8.6)
Deposit on purchase of property, plant & equipment	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.00	\$0.0	\$0.00
Purchases of marketable securities	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.00 \$0	\$0.0	\$0.00 \$0
Sales and maturities of marketable securities	\$3.40	\$0 \$0	\$0 \$0	\$0 \$0	\$0.0	\$0 \$0							
Net cash used in investing activities	\$3.40 \$3.4	\$0.0	(\$0.0)	(\$0.9)	(\$1.1)	(\$1.3)	(\$3.1)	(\$4.3)	(\$5.6)	(\$6.2)	(\$6.9)	(\$7.7)	(\$8.6)
•	\$3.4	φυ.υ	(φυ.υ)	(φυ.σ)	(φ1.1)	(φ1.3)	(\$5.1)	(\$4.5)	(\$5.0)	(\$0.2)	(40.9)	(φ1.1)	(\$0.0)
Financing activities:													
Payment of debt issuance costs	\$0.000	(\$0)	(\$0)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0.0	\$0
Proceeds from issuance of stock options	\$0.0	\$0	\$0	\$0	\$0	\$1	\$1	\$1	\$1	\$2	\$2	\$3	\$3
Sale of royalty entitlement to Novo A/S	\$0.000	\$0	\$42	\$42	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Proceeds from issuance of notes payable, net	\$0.000	\$11.39	(\$12)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0.0	\$0
Proceeds from common stock and options	\$0.0	\$0	\$176	\$55	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0
Proceeds from issuance of preferred stock	\$15.0	\$0	\$50	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0.0	\$0
. receded mem leadance of prototred attack													
Tax Benefits related to employee stock options	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$1	\$2 \$3.7	\$3 \$4.6	\$4 \$5.7	\$4.4	\$5 \$8.8

(\$2) \$6 **\$4**

\$206

\$4

\$211

\$213

\$211

\$423

\$32

\$423

(\$105)

\$455

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\$54

\$351

\$405

\$459

\$405 **\$863** \$501

\$863

\$1,364

\$417

\$1,364 **\$1,781** \$484

\$1,781

\$2,265

\$561

\$2,265

\$2,826

\$656

\$2,826

\$3,482

(\$1)

\$7

Source: Company Data, Morgan Stanley Research estimates

Effect of exchange rate on changes in cash

Increase in cash and cash equivalents

Cash and equivalents at beginning of year Cash and equivalents at end of year

MORGAN STANLEY RESEARCH

May 20, 2014 Ophthotech Corp



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Within the last 12 months, Morgan Stanley was received companyation for investment banking services from Akebia Therapeutics Inc. AMAG

Priamaceuticals, Inc., NPS Pharmaceutical Inc, Versartis, Inc..

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May 20, 2014 **Ophthotech Corp**

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(as of April 30, 2014)

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	Coverage U	niverse	Investment	ents (IBC)	
_		% of		% of %	% of Rating
Stock Rating Category	Count	Total	Count	Total IBC	Category
Overweight/Buy	1045	35%	355	38%	34%
Equal-weight/Hold	1301	43%	455	48%	35%
Not-Rated/Hold	110	4%	22	2%	20%
Underweight/Sell	543	18%	109	12%	20%
Total	2,999		941		

Data include common stock and ADRs currently assigned ratings. Investment Banking Clients are companies from whom Morgan Stanley received investment banking compensation in the last 12 months.

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Overweight (O). The stock's total return is expected to exceed the average total return of the analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

Equal-weight (E). The stock's total return is expected to be in line with the average total return of the analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

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analyst's industry (or industry team's) coverage universe, on a risk-adjusted basis, over the next 12-18 months.

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Analyst Industry Views

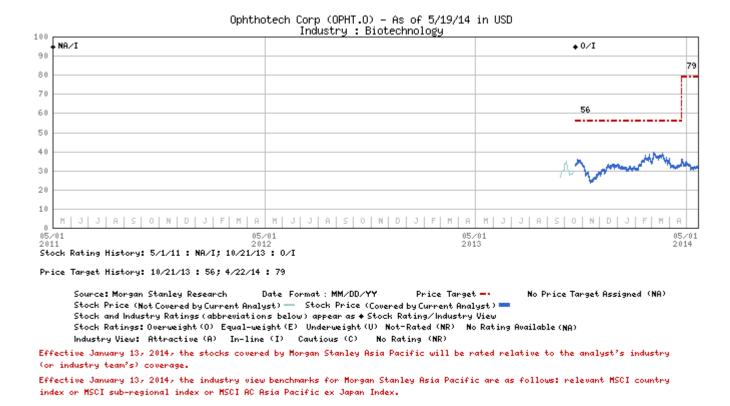
Attractive (A): The analyst expects the performance of his or her industry coverage universe over the next 12-18 months to be attractive vs. the relevant broad market benchmark, as indicated below.

In-Line (I): The analyst expects the performance of his or her industry coverage universe over the next 12-18 months to be in line with the relevant broad market benchmark, as indicated below.

Cautious (C): The analyst views the performance of his or her industry coverage universe over the next 12-18 months with caution vs. the relevant broad market benchmark, as indicated below.

Benchmarks for each region are as follows: North America - S&P 500; Latin America - relevant MSCI country index or MSCI Latin America Index; Europe - MSCI Europe; Japan - TOPIX; Asia - relevant MSCI country index or MSCI sub-regional index or MSCI AC Asia Pacific ex Japan Index.

Stock Price, Price Target and Rating History (See Rating Definitions)



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May 20, 2014 Ophthotech Corp

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Industry Coverage:Biotechnology

Company (Ticker) Rating (as of) Price* (05/19/2014) David Friedman, M.D. AMAG Pharmaceuticals. Inc. E (11/21/2011) \$18.02 (AMAG.O) O (04/14/2014) \$21 14 Akebia Therapeutics Inc (AKBA.O) Alexion Pharmaceuticals (ALXN.O) O (09/07/2010) \$158.42 Alnylam Pharmaceuticals E (01/14/2014) \$58.26 (ALNY.O) Auxilium Pharmaceuticals U (03/06/2014) \$19.79 (AUXL.O) Chimerix Inc (CMRX.O) O (05/06/2013) \$16.82 Cubist Pharmaceuticals Inc. O (11/13/2013) \$66.7 (CBST.O) **GW Pharmaceuticals PLC** O (04/22/2014) \$69.94 (GWPH.O) Idenix Pharmaceuticals, Inc. E (03/18/2011) \$6.04 (IDIX.O) Incyte Corporation (INCY.O) U (01/23/2013) \$49.9 InterMune (ITMN.O) E (09/07/2010) \$38.92 Ironwood Pharmaceuticals, Inc. E (04/24/2013) \$13.75 (IRWD.O) Lexicon Pharmaceuticals, Inc. U (06/11/2013) \$1.42 (LXRX.O) NPS Pharmaceuticals (NPSP.O) O (10/03/2012) \$27.43 E (01/08/2014) Neurocrine Biosciences Inc \$13.35 (NBIX.O) Ophthotech Corp (OPHT.O) \$31.46 O- (10/21/2013) Portola Pharmaceuticals Inc O (06/17/2013) \$20.62 (PTLA.O) Relypsa, Inc. (RLYP.O) O (12/10/2013) \$21.55 Synageva Biopharma Corp O (04/20/2012) \$84.84 (GEVA.O) Theravance Inc (THRX.O) U (07/22/2013) \$27.59 Ultragenyx Pharmaceutical Inc O (02/25/2014) \$33.8 (RARE.O) Versartis, Inc. (VSAR.O) O (04/15/2014) \$27.4 Vertex Pharmaceuticals (VRTX.O) E (05/08/2012) \$67.22 XenoPort Inc (XNPT.O) U (06/11/2013) \$3.72 **Matthew Harrison** Amgen Inc. (AMGN.O) O (03/26/2014) \$112.08 Biogen Idec Inc. (BIIB.O) O (03/26/2014) \$290.75 Celgene Corp (CELG.O) E (03/26/2014) \$147.38 Gilead Sciences Inc. (GILD.O) E (03/26/2014) \$82.05 Pharmacyclics Inc. (PCYC.O) E (03/26/2014) \$99.38 Regeneron Pharmaceuticals Inc. E (03/26/2014) \$302.14 (REGN.O)

Stock Ratings are subject to change. Please see latest research for each company.
* Historical prices are not split adjusted.