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Stock Rating
Overweight

Industry View
In-Line

Ophthotech Corp

3Q: EU Fovista Updates and Path Forward a Focus

3Q earnings contained incremental updates on Fovista's clinical progress, particularly EU updates.

3Q earnings contained incremental information about Fovista and pipeline progress. Our thoughts are below.

Fovista US: OPHT is currently enrolling US pts in their two Ph 3 Fovista + Lucentis trials, with data expected in '16. The company expects to begin a Ph 3 trial of Fovista + Eylea or Avastin in the US in 1Q14. In our model, the US represents ~2/3^{rds} of WW Fovista sales in 2022 and >90% of OPHT 2022 revenues as we currently assume an EU partner who would pay OPHT a royalty.

Fovista EU: Enrollment of Ph 3 appears to be gated by recent CHMP feedback as follows. **1)** The CHMP wants to discuss the pre-clinical tox data/rationale for Fovista + Eylea or Avastin, which the company expects to provide prior to trial start. We see this as likely a smaller issue. **2)** Since Avastin is not labeled in EU, it will likely be hard to have a claim with Avastin in an EU label. We do not see this as a major issue as Avastin use is minimal in the EU and they would still have the trial data on the label most likely (if a positive trial). **3)** The CHMP wants to discuss the Lucentis and Eylea dosing schedules given different labels in the US vs. EU (first yr of Ph 3 trial is in line with US labeled dosing). The main question to us is whether OPHT would need to run any add'l studies to settle this issue. Mgmt does not expect, as of now, that a potential bridging study or add'l Ph 3 trials are needed. A reasonable bridging design in our view, if needed, would be comparing the two different dosing regimens head to head in a moderate sized add on trial. **Importantly, even if add'l work is needed, we would not expect a major timeline shift as the larger Ph 3 studies have just gotten underway, providing a 2 yr time buffer to settle any EU specific work.** We note that logically, it seem somewhat inconsistent for the EMEA to be uncomfortable with non-labeled dosing in combo vs. monotherapy as that is not an uncommon approach in other areas such as oncology. The Ph 3 study for Eylea was modified to dose Eylea every 8 weeks.

Other indications: OPHT intends to initiate Ph 2 trials in other indications (wet AMD pts failing anti-VEGF therapy, proliferative vitreoretinopathy, and/or von Hippel-Lindau disease) in 2014 with data in 2015.

What's New: Ophthotech reported 3Q earnings.

Key Ratios and Statistics

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

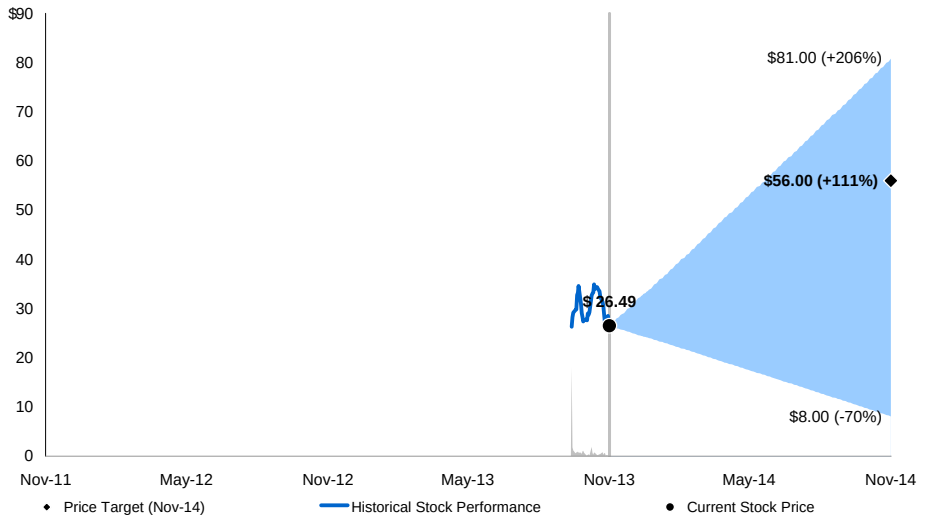
Price target	**$56.00**
Shr price, close (Nov 12, 2013)	$26.49
Mkt cap, curr (mm)	$801
52-Week Range	$36.60-23.00

Fiscal Year ending	**12/12**	**12/13e**	**12/14e**	**12/15e**
ModelWare EPS ($00s)	**(0.02)**	**(0.07)**	**(0.03)**	**(0.03)**
Prior ModelWare EPS ($)	**-**	**(2.87)**	**(2.85)**	**(3.32)**
P/E	**NM**	**NM**	**NM**	**NM**
Consensus EPS ($)§	**-**	**(2.47)**	**(2.45)**	**(2.77)**
Div yld (%)	-	0.0	0.0	0.0
EPS ($)**	(2.52)	(6.86)	(3.11)	(3.50)

Unless otherwise noted, all metrics are based on Morgan Stanley ModelWare framework (please see explanation later in this note).
§ = Consensus data is provided by Thomson Reuters Estimates.
**** =** Based on consensus methodology
e = Morgan Stanley Research estimates

Risk-Reward Snapshot: Ophthotech (OPHT, OW, PT $56)

Fovista's Success Drives Risk-Reward



Source: Morgan Stanley Research estimates, Thomson Reuters

Price Target $56		We derive our PT from a discounted cash flow analysis that uses a WACC of 15% 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 Case $81	**DCF**	**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 ~40% share of the overall wet AMD market. This share is driven by Fovista use in ~45% of Lucentis treated eyes, 33% of Eylea treated eyes and ~40% 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 >$2bn with ~$1.5bn in sales in the US.
Base Case $56	**DCF**	**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 ~40% of Lucentis treated eyes, ~33% of Eylea treated eyes at peak and ~30% 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 ~$1.7bn with >$1bn sales in the US.
Bear Case $8	**Cash Based Value**	**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 >$1.5bn.
- 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 combinations show different results than the Lucentis + Fovista combination and/or 2) the FDA or EMEA could require add'l data for these less well studied combinations.

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	($47)	($90)	($105)	($2)	($67)	$310	$405	$512	$524	$530	$516	$513	$512	$512	$410	$328	$262
YoY Growth		*89%*	*16.8%*	*-98.2%*	*3438%*	*-561.9%*	*30.8%*	*26.3%*	*2.3%*	*1.2%*	*-2.6%*	*-0.7%*	*-0.2%*	*0.0%*	*-20.0%*	*-20.0%*	*-20.0%*
Net Cash Proxy for Dilution	($2.54)	($4.29)	($5.5)	($6.8)	($8.2)	($9.8)	($8.0)	($8.2)	($8.8)	($9.1)	($8.8)	($8.8)	($8.8)	($8.8)	($8.8)	($8.8)	($8.8)
Free Cash Flow for DCF	($49.9)	($94.0)	($110.3)	($8.7)	($75.3)	$300.0	$397.3	$503.5	$514.8	$521.1	$507.5	$504.2	$503.4	$503.1	$400.8	$318.8	$253.3
Present Value of Free Cash Flow	($55.4)	($90.8)	($92.6)	($6.4)	($47.8)	$165.7	$190.7	$210.2	$186.9	$164.5	$139.3	$120.3	$104.5	$90.8	$62.9	$43.5	$30.1

Source: Company data, Morgan Stanley Research estimates

Exhibit 2
DCF Valuation Suggests Upside

Valuation Date	**2013.75**
Discount Rate	**15%**
Terminal Growth Rate	0%
Terminal Value Year	2029
Sum of Discounted FCF	$1,258
Discounted Terminal Value	$200
Net Cash	$236
Equity Value	**$1,695**
Equity Value/Sh	**$56**
Shares Outstanding (Basic)	30.4

Source: Company Data, Morgan Stanley Research estimates

$56 PT includes Fovista in wet AMD.
We derive our PT from a discounted cash flow (DCF) analysis that uses a WACC of 15% 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 15% to development stage companies that have a fair amount of risk.
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 WW rights to Fovista. We assume an EU/ROW partner. We model royalties to Ophthotech in the low 20s on EU sales and high singe digits on ROW sales. 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 be relatively stable through 2017. We expect a sig. increase in 2017+ as Ophthotech builds a US infrastructure to market Fovista.

Financings: We model a $100mn upfront from a partner and a ~$145mn financing in 2016.

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 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

Upcoming Catalysts

Drug	Type	Event	Expected Timing
Fovista	Product Advancement	Begin potential exploratory trials in other indications	2014
Fovista	Clinical Data	Data from potential exploratory trials in other indications	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

Exhibit 4

Variance Analysis

	1Q13 Actual	2Q13 MS Est	2Q13 Actual	Variance from Est.	*Q/Q % chg*
Revenues:					
Total Revenues	**$0.0**	**$0.0**	**$0.0**		
Operating Expenses:					
Cost of Sales	$0.0	$0.0	$0.0		
R&D	$4.1	$5.0	$10.3	107.0%	*152%*
SG&A	$3.1	$3.5	$3.8	7.7%	*21%*
Total Operating Expenses	**$7.2**	**$8.5**	**$14.1**	66.1%	***95%***
Operating Income (Loss)					
Interest expense	$0.0	$0.0	$0.0		*-100%*
Interest and other income	($1.1)	$0.0	$0.0		*-100%*
Net Income (Loss), excl. options expense	**($9.7)**	**($8.5)**	**($15.0)**	76.3%	***55%***
Net Income (Loss), inc. options expense	**($10.0)**	**($8.9)**	**($16.1)**	81.6%	***61%***
EPS, Non GAAP, diluted (excludes options)	**($6.85)**	**($1.02)**	**($8.99)**	779.2%	*31%*
EPS, diluted- GAAP	**($8.13)**	**($1.02)**	**($10.26)**	904.1%	*26%*
Consensus EPS		**($1.00)**			
Diluted Shares Outstanding	1	9	1.80	-79.3%	*23%*

Source: Company Data, Morgan Stanley Research

Exhibit 5

Changes to Model

	2012	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
US Fovista												
Current	0	0	0	0	0	80	562	886	1,111	1,115	1,129	1,104
Prior	0	0	0	0	0	80	562	886	1,111	1,115	1,129	1,104
EU Fovista												
Current	0	0	0	0	0	0	73	252	392	470	440	415
Prior	0	0	0	0	0	0	73	252	392	470	440	415
Total Revenues ($mn)												
Current	0	0	0	0	100	80	579	947	1,207	1,230	1,237	1,205
Prior	0	0	0	0	100	80	579	947	1,207	1,230	1,237	1,205
COGS ($mn)												
Current	0	0	0	0	0	12	92	179	226	237	235	228
Prior	0	0	0	0	0	12	92	179	226	237	235	228
SG&A ($mn) - ex-ESOs												
Current	6	12	14	14	14	61	107	123	135	135	135	135
Prior	6	12	12	12	13	57	102	117	129	135	135	135
R&D ($mn) -ex-ESOs												
Current	7	29	79	91	87	73	64	55	55	55	55	55
Prior	7	23	73	88	83	71	64	54	54	54	54	54
Interest and Other Income, net												
Current	(1)	(1)	0	0	0	0	1	2	3	4	6	7
Prior	(1)	(1)	0	0	0	1	1	2	3	4	6	7
Net Income ($MM) - Non GAAP, excludes options												
Current	(14)	(45)	(93)	(105)	(1)	(66)	317	411	516	525	531	517
Prior	(14)	(37)	(85)	(100)	4	(59)	322	402	521	525	531	517
EPS Non-GAAP, Diluted, excludes options												
Current	($1.62)	($6.74)	($3.05)	($3.43)	($0.02)	($1.94)	$7.12	$8.80	$10.59	$10.28	$9.91	$9.14
Prior	($1.62)	($2.82)	($2.79)	($3.26)	$0.11	($1.74)	$7.24	$8.60	$10.68	$10.29	$9.91	$9.14
EPS - GAAP												
Current	($2.52)	($6.86)	($3.11)	($3.50)	($0.08)	($2.03)	$7.00	$8.70	$10.48	$10.17	$9.81	$9.05
Prior	($2.52)	($2.87)	($2.85)	($3.32)	$0.06	($1.82)	$7.12	$8.50	$10.57	$10.18	$9.81	$9.05
Basic Shares Outstanding												
Current	9	7	30	31	32	34	35	35	36	36	37	37
Prior	9	13	30	31	32	34	35	35	36	36	37	37
Dilluted Shares Outstanding												
Current	9	7	30	31	32	34	45	47	49	51	54	57
Prior	9	13	30	31	40	34	45	47	49	51	54	57

Source: Company Data, Morgan Stanley Research

Exhibit 6

Quarterly Income Statement

($ in millions)	1Q13A	2Q13A	3Q13A	4Q13E	2013E
Other Revenue					0
Revenues	$0.0	$0.0	$0.0	$0.0	$0.0
Operating Expenses:					
Cost of Sales					0
% total product sales	*NA*	*NA*	*NA*	*NA*	*NA*
R&D	2.4	4.1	10.3	12.0	29
% of revenue	*na*	*na*	*na*	*na*	*na*
SG&A	1.6	3.1	3.8	3.5	12
% of revenue	*na*	*na*	*na*	*na*	*na*
Total Operating Expenses	$4.0	$7.2	$14.1	$15.5	$40.9
Operating Income (Loss)	($4.0)	($7.2)	($14.1)	($15.5)	($40.9)
Operating Margin	*na*	*na*	*na*	*na*	*na*
Interest income	$0.00	$0.00	$0.0	$0.0	$0.00
Interest expense	($0.36)	($1.10)	$0.0	$0.0	($1.45)
Fx gain (loss)	($0.00)	$0.00	$0.0		($0.00)
Other loss	($0.13)	($1.32)	($0.9)		($2.32)
Change in fair value related to inv. rights liabil					
Pretax Income (Loss)	($4.51)	($9.7)	($15.0)	($15.5)	($44.6)
Provision for Income Taxes	0				
Effective tax rate					
Net Income (Loss)	($4.51)	($9.65)	($14.99)	($15.50)	($44.65)
EPS, basic	($3.09)	($6.61)	($8.35)	($0.71)	($6.74)
EPS, diluted	($3.09)	($6.61)	($8.35)	($0.71)	($6.74)
Options Expense	0.11	0.35	1.15	1.24	2.85
% of operating expense	*2.7%*	*4.9%*	*8.1%*	*8.0%*	*7.0%*
Tax benefit from options					
Net Income (inc. options expense)	($4.62)	($10.01)	($16.1)	($16.7)	($47.5)
EPS, diluted (inc. options expense)	($3.16)	($6.85)	($8.99)	($0.77)	($7.17)
Basic Shares Outstanding	1.46	1.46	1.80	21.80	6.63
Diluted Shares Outstanding	1.46	1.46	1.80	21.80	6.63
One times	($1.74)	($1.86)	($2.29)	$0.0	($5.9)
GAAP Net Income (incl. options)	($6.360)	($11.864)	($18.424)	($16.7)	($53.4)
GAAP EPS (dil, incl. options)	($4.36)	($8.13)	($10.26)	($0.77)	($8.1)

Source: Company Data, Morgan Stanley Research

Exhibit 7

Annual Income Statement

($ in millions)	2011A	2012A	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
US Fovista			**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$79.6**	**$561.8**	**$886.5**	**$1,111.5**	**$1,115.4**	**$1,129.4**	**$1,104**
EU Fovista			$0.0	$0.0	$0.0	$0.0	$0.0	$73.0	$252.1	$392.0	$470.0	$439.6	$415.0
ROW Fovista			$0	$0	$0	$0	$0	$15	$63	$118	$141	$132	$124
Total WW Fovista Sales			**$0**	**$0**	**$0**	**$0**	**$80**	**$649**	**$1,202**	**$1,621**	**$1,726**	**$1,701**	**$1,644**
EU Fovista Royalties			$0.0	$0.0	$0.0	$0.0	$0.0	$16.1	$55.5	$86.2	$103.4	$96.7	$91.3
ROW Fovista Royalties			$0	$0	$0	$0	$0	$1	$5	$9	$11	$11	$10
Total Fovista Royalties			**$0**	**$0**	**$0**	**$0**	**$0**	**$17**	**$60**	**$96**	**$115**	**$107**	**$101**
Other Revenue						**100**							
Total Revenue	$0	$0	$0	$0	$0	$100	$80	$579	$947	$1,207	$1,230	$1,237	$1,205
Operating Expenses:						0%	0%	3%	6%	8%	9%	9%	8%
Cost of Sales	$0	$0	$0	$0	$0	$0	$12	$92	$179	$226	$237	$235	$228
% of US sales- Drug cost	*NA*	*NA*	*NA*	*NA*	*NA*	*7%*	*7%*	*7%*	*6%*	*5%*	*5%*	*5%*	*5%*
% of US sales - total COGS (incl. royalties)							*16%*	*16%*	*20%*	*20%*	*21%*	*21%*	*21%*
R&D	$14	$7	$29	$79	$91	$87	$73	$64	$55	$55	$55	$55	$55
YoY growth	*-6%*	*-51%*	*325%*	*175%*	*15%*	*-5%*	*-25%*	*-18%*	*-15%*	*0%*	*0%*	*0%*	*0%*
% of revenue	*na*	*na*	*na*	*na*	*na*	*87%*	*92%*	*11%*	*6%*	*5%*	*4%*	*4%*	*5%*
SG&A	$5	$6	$12	$14	$14	$14	$61	$107	$123	$135	$135	$135	$135
YoY growth	*27%*	*14%*	*92%*	*15%*	*2%*	*2%*	*325%*	*75%*	*15%*	*10%*	*0%*	*0%*	*0%*
% of revenue	*na*	*na*	*na*	*na*	*na*	*14%*	*77%*	*18%*	*13%*	*11%*	*11%*	*11%*	*11%*
Total Operating Expenses	$19.39	$13.04	$41	$93	$105	$101	$146	$263	$357	$416	$427	$425	$418
Operating Income (Loss)	-$19	-$13	-$41	-$93	-$105	-$1	-$67	$316	$590	$791	$803	$812	$788
Operating Margin	*na*	*na*	*na*	*na*	*na*	*na*	*na*	*55%*	*62%*	*66%*	*65%*	*66%*	*65%*
Interest income	$0.002	$0.0	$0.0	$0.4	$0.3	$0.4	$0.5	$0.8	$1.7	$3.0	$4.3	$5.8	$7.2
Interest expense	$0.00	-$0.5	-$1.5	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Fx transaction gain (loss)	-$0.02	$0.0	$0.0										
Non cash change in fair value (inv. rights liabil.)	$0.0	-$0.4	-$2.3										
Pretax Income (Loss)	($19.41)	($13.9)	($44.6)	($92.8)	($105.1)	($0.7)	($66.43)	$316.89	$591.74	$794.38	$807.46	$817.39	$794.97
Provision for Income Taxes	(1.03)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	180.3	278.0	282.6	286.1	278.2
Effective Tax Rate		0%	0%	0%	0%	0%	0.0%	0%	30%	35%	35%	35%	35%
Net Income (Loss)	($18.38)	($13.9)	($44.6)	($93)	($105)	($1)	($66)	$317	$411	$516	$525	$531	$517
EPS, basic	($2.23)	($1.62)	($6.74)	($3.05)	($3.43)	($0.02)	($1.94)	$9.17	$11.75	$14.54	$14.54	$14.46	$13.79
EPS, diluted	($2.23)	($1.62)	($6.74)	($3.05)	($3.43)	($0.02)	($1.94)	$7.12	$8.80	$10.59	$10.28	$9.91	$9.14
EPS, diluted (incl. ESOs)	($2.26)	($1.70)	($6.86)	($3.11)	($3.50)	($0.08)	($2.03)	$7.00	$8.70	$10.48	$10.17	$9.81	$9.05
Basic Shares Outstanding	8.23	8.6	6.6	30.4	30.6	32.4	34.2	34.6	35.0	35.5	36.1	36.7	37.5
Diluted Shares Outstanding	8.23	8.6	6.6	30.4	30.6	32.4	34.2	44.5	46.7	48.8	51.0	53.6	56.5
One time items	($6.8)	($7.1)											
GAAP Net Income	($25.47)	($21.6)	($45.5)	($94.7)	($107.2)	($2.7)	($69.4)	$311.6	$406.5	$510.9	$519.3	$525.8	$511.3
GAAP EPS, diluted (includes ESOs)	($3.10)	($2.52)	($6.86)	($3.11)	($3.50)	($0.08)	($2.03)	$7.00	$8.70	$10.48	$10.17	$9.81	$9.05

Source: Company Data, Morgan Stanley Research estimates

Exhibit 8

Balance Sheet

($ in millions)	2011A	2012A	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Assets													
Cash and cash equivalents	$6.4	$4.3	$211	$167	$67	$217	$160	$484	$912	$1,453	$2,015	$2,595	$3,176
Marketable securities	$0.0	$0.2	$0.2	$0.2	$0.2	$0.2	$0.2	$0.2	$0.2	$0.2	$0.2	$0.2	$0.2
Other receivables	$1.0	$0.0	$1.1	$1.2	$1.3	$1.3	$1.6	$5.8	$9.5	$12.1	$12.3	$12.4	$12.1
Prepaid expenses and other deposits	$0.1	$0.0	$0	$0	$0	$0	$1	$6	$9	$12	$12	$12	$12
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**	**$213**	**$168**	**$68**	**$219**	**$163**	**$496**	**$931**	**$1,478**	**$2,040**	**$2,620**	**$3,200**
Property, plant and equipment, net	$0.07	$0.0	$0	$0	$0	$1	$2	$3	$4	$6	$7	$8	$9
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**	**$212.7**	**$168.1**	**$68.8**	**$220.3**	**$164.7**	**$498.6**	**$935.3**	**$1,483.2**	**$2,046.9**	**$2,627.8**	**$3,208.5**
Liabilities and stockholders' equity													
Notes payable	$0.0	$11	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Accrued drug supply and trial cost	$1.5	$1	$1	$2	$3	$3	$2	$2	$2	$2	$2	$2	$2
Accounts payable	$0.9	$1	$1	$3	$3	$3	$4	$8	$11	$12	$13	$13	$13
Accrued compensation	$0.8	$1	$1	$1	$1	$1	$1	$1	$1	$1	$1	$1	$1
Deferred rent	$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
Investor rights liability	$0.2	$1	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Total current liabilities	**$3.3**	**$14.4**	**$2.6**	**$5.7**	**$6.4**	**$6.2**	**$7.1**	**$10.3**	**$12.9**	**$14.6**	**$15.0**	**$14.9**	**$14.7**
Other long-term liabilities	$0.0	$0	$42	$83	$83	$83	$83	$83	$83	$83	$83	$83	$83
Total liabilities	**$3.3**	**$14.4**	**$44**	**$89**	**$90**	**$89**	**$90**	**$94**	**$96**	**$98**	**$98**	**$98**	**$98**
Cumulative Series A	$65.3	$69.47	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Cumulative Series A-1	$8.0	$8.46	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Cumulative Series B	$33.1	$35.5	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Cumulative Series B-1	$0.5	$1	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Preferred Stock	$3.0	$3	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Common stock	$0.0	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01	$0.01
APIC	$0.0	$0	$340	$346	$353	$507	$520	$539	$567	$602	$646	$701	$771
Accumulated deficit	($105)	($126)	($172)	($267)	($374)	($376)	($446)	($134)	$272	$783	$1,303	$1,828	$2,340
Accumulated other comprehensive income	$0.0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0
Total stockholders' equity	**$4**	**-$10**	**$168**	**$79**	**-$21**	**$131**	**$74**	**$405**	**$839**	**$1,385**	**$1,949**	**$2,530**	**$3,110**
Total liabilities and stockholder's equity	**$7.74**	**$4.9**	**$213**	**$168**	**$69**	**$220**	**$165**	**$499**	**$935**	**$1,483**	**$2,047**	**$2,628**	**$3,208**

Source: Company Data, Morgan Stanley Research estimates

Exhibit 9

Cash Flow Statement

($ in millions)	2011A	2012A	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Net loss	($18.6)	($14.6)	($45.5)	($94.7)	($107.2)	($2.7)	($69.4)	$311.6	$406.5	$510.9	$519.3	$525.8	$511.3
Depreciation	$0.03	$0.03	$0.0	$0.0	$0.1	$0.1	$0.2	$0.3	$0.4	$0.6	$0.7	$0.9	$1.0
Amortization and accretion	$0.00	$0.1	$0.0	$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	$0.0	$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	($0.97)	$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	$0.0	$1.9	$2.1	$2.0	$2.9	$5.3	$5.0	$5.4	$5.6	$5.5	$5.4
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	($0)	($0)	($0)	($0)	($1)	($5)	($4)	($3)	($0)	($0.1)	$0
Other receiveables	($0.74)	$1.0	($1)	($0)	($0)	($0)	($0)	($4)	($4)	($3)	($0)	($0.1)	$0
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)	($0)	$2	$0	($0)	($0)	($0)	($0)	$0	$0	$0.0	$0
Accounts payable and accrued expenses	($0.05)	$0.0	$0	$2	$0	($0)	$1	$3	$3	$2	$0	($0.1)	($0)
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
Net cash used in operating activities	**($19.12)**	**($13.10)**	**($47)**	**($90)**	**($104)**	**($1)**	**($66)**	**$311**	**$407**	**$513**	**$525**	**$532.0**	**$518**
Investing Activities:													
Purchases of property, plant and equipment	($0.00)	$0.0	($0.0)	($0.0)	($0.5)	($1.0)	($0.8)	($1.4)	($1.9)	($1.8)	($1.8)	($1.9)	($1.8)
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	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0.0	$0
Sales and maturities of marketable securities	$3.40	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0	$0.0	$0
Net cash used in investing activities	**$3.4**	**$0.0**	**($0.0)**	**($0.0)**	**($0.5)**	**($1.0)**	**($0.8)**	**($1.4)**	**($1.9)**	**($1.8)**	**($1.8)**	**($1.9)**	**($1.8)**
Financing activities:													
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	$11.01	($11)	$0	$0	$0	$0	$0	$0	$0	$0	$0.0	$0
Proceeds from common stock and options	$0.0	$0	$173	$4	$5	$152	$10	$14	$19	$25	$34	$45	$59
Proceeds from issuance of preferred stock	$15.0	$0	$50	$0	$0	$0	$0	$0	$0	$0	$0	$0.0	$0
Tax Benefits related to employee stock options	$0	$0	$0	$0	$0	$0	$0	$0	$3	$4	$5	$4.9	$5
Net cash provided by financing activities	**$14.99**	**$11.01**	**$254.3**	**$45.2**	**$5.0**	**$152.4**	**$10.0**	**$13.8**	**$22.6**	**$29.8**	**$38.5**	**$49.7**	**$64.2**
Effect of exchange rate on changes in cash													
Increase in cash and cash equivalents	($0.73)	($2.1)	$206.96	($44.58)	($99.77)	$150.48	($57.12)	$323.59	$427.84	$541.50	$562.07	$579.8	$580.54
Cash and equivalents at beginning of year	$7.1	$6.4	$4	$211	$167	$67	$217	$160	$484	$912	$1,453	$2,015.3	$2,595
Cash and equivalents at end of year	**$6.396**	**$4.3**	**$211**	**$167**	**$67**	**$217**	**$160**	**$484**	**$912**	**$1,453**	**$2,015**	**$2,595.1**	**$3,176**

Source: Company Data, Morgan Stanley Research estimates

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Total	**2,934**		**1076**		

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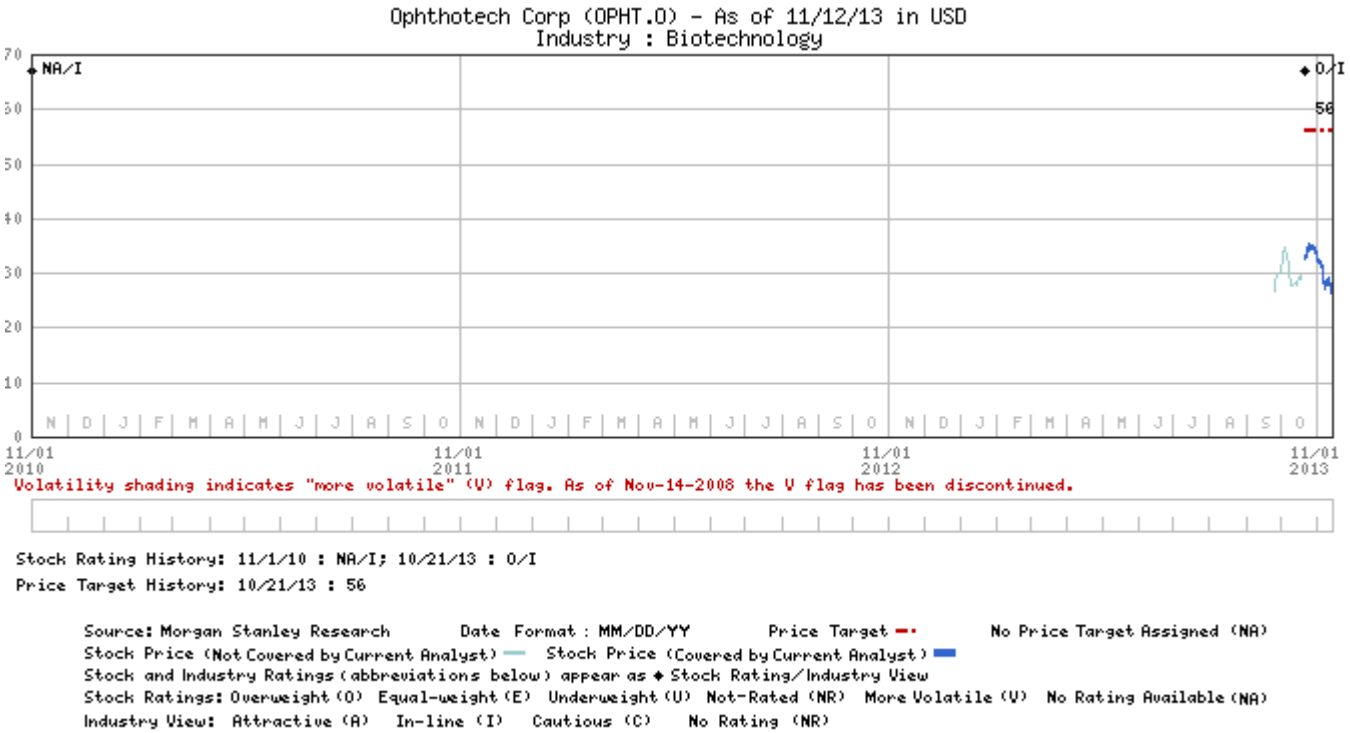
Stock Price, Price Target and Rating History (See Rating Definitions)



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Industry Coverage:Biotechnology

Company (Ticker)	Rating (as of)	Price* (11/12/2013)
David Friedman, M.D.		
AMAG Pharmaceuticals, Inc. (AMAG.O)	E (11/21/2011)	$24.55
Alexion Pharmaceuticals (ALXN.O)	O (09/07/2010)	$115.72
Alnylam Pharmaceuticals (ALNY.O)	O (06/11/2013)	$54.19
Auxilium Pharmaceuticals (AUXL.O)	E (05/03/2013)	$19.05
Chimerix Inc (CMRX.O)	O (05/06/2013)	$14.96
Cubist Pharmaceuticals Inc. (CBST.O)	O (11/13/2013)	$62.38
Elan Corporation PLC (ELN.N)	++	$17.59
Idenix Pharmaceuticals, Inc. (IDIX.O)	E (03/18/2011)	$4.4
Incyte Corporation (INCY.O)	U (01/23/2013)	$40.9
InterMune (ITMN.O)	E (09/07/2010)	$12.88
Ironwood Pharmaceuticals, Inc. (IRWD.O)	E (04/24/2013)	$9.23
Lexicon Pharmaceuticals, Inc. (LXRX.O)	U (06/11/2013)	$2.17
NPS Pharmaceuticals (NPSP.O)	O (10/03/2012)	$25.75
Ophthotech Corp (OPHT.O)	O- (10/21/2013)	$26.49
Portola Pharmaceuticals Inc (PTLA.O)	O (06/17/2013)	$27.48
Synageva Biopharma Corp (GEVA.O)	O (04/20/2012)	$52
Theravance Inc (THRX.O)	U (07/22/2013)	$34.4
Vertex Pharmaceuticals (VRTX.O)	E (05/08/2012)	$60.18
XenoPort Inc (XNPT.O)	U (06/11/2013)	$5.2
Yigal Nochomovitz, Ph.D.		
ImmunoGen Inc. (IMGN.O)	U (11/11/2013)	$14.7
Infinity Pharmaceuticals Inc (INFI.O)	O (02/19/2013)	$13.73
Pharmacyclics Inc. (PCYC.O)	E (03/19/2013)	$119.67
Tesaro Inc. (TSRO.O)	O (07/23/2012)	$33.59
Sara Slifka		
Neurocrine Biosciences Inc (NBIX.O)	O (10/03/2012)	$8.93

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* Historical prices are not split adjusted.