October 20, 2013

OUTPERFORM

Reason for report:

INITIATION

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LEERINK SWANN

HEALTHCARE EQUITY RESEARCH

OPHTHOTECH CORPORATION

All Eyes on Fovista; Initiating at Outperform

• **Bottom Line**: We are initiating coverage of OPHT with an Outperform rating and $37 PT in 12 mos. Ophthotech is a late stage biotechnology company developing Fovista, an anti-platelet derived growth factor (PDGF) aptamer that in ph. IIb demonstrated impressive vision benefits to wet Age-Related Macular Degeneration (AMD) pts., when administered in combination with Roche's Lucentis. A ph. III confirmatory trial is underway that if successful, could indicate Fovista treatment for wet AMD in combination with any of Lucentis, Eylea, or Avastin.

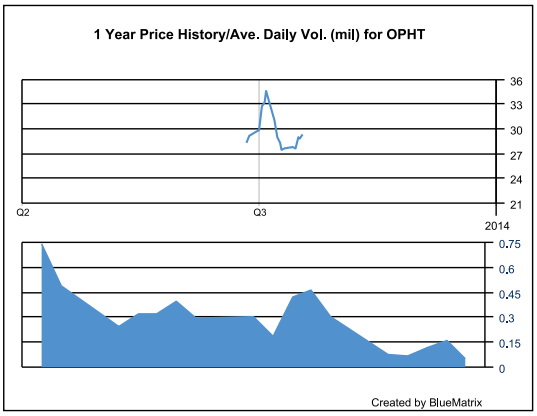
• **In Phase IIb, a combination of Fovista and Lucentis was able to provide patients with a ~62% additional benefit over Lucentis monotherapy** on the Early Treatment Diabetic Retinopathy Scale (ETDRS), a widely-used standardized chart of vision testing. Fovista's additive benefits were both statistically significant, dose dependent, and according to MEDACorp KOLs, clinically relevant. OPHT also performed a variety of sub-group analyses that validate Fovista's efficacy across many different cohorts of wet AMD patients, as well as anatomical assessments that correspond with the relatively rapid biological realization of Fovista's activity.

• **A Phase III Fovista pivotal trial is ongoing that we believe is de-risked based on its strong similarities to Phase II, greater statistical powering, and longer duration,** the latter of which could beneficial to OPHT since the delta between the Fovista add-on arm and the Lucentis monotherapy arm seemed to be widening at the end of the Phase IIb trial. OPHT has employed identical exclusion/inclusion criteria to the ongoing pivotal Phase III to minimize the risk that the Phase IIb results will not be replicated. Phase III results are expected in mid-2016, and we assume 80%/65%/65% probabilities of success that Fovista will demonstrate statistically significant efficacy when administered in combination with Lucentis, Eylea and Avastin respectively. Additional studies may be initiated in 2014 in VEGF-nonresponders and other rarer ocular conditions, which could add to the Fovista opportunity.

• **While anti-VEGF treatments such as Lucentis and REGN's (OP) Eylea have achieved rapid commercial success and strong uptake in wet AMD, we see an opportunity for Fovista to upgrade the wet AMD standard-of-care,** especially in patients that begin to plateau or even decline after receiving currently available treatments for multiple years. Our view is corroborated by the results of the Lucentis long-term visual outcomes HORIZON extension study which showed that after 5 years of therapy, the aggregate visual capacity of Lucentis-treated patients returned to baseline pre-treatment levels. There are approximately 1.3 million cases of wet AMD in the US, and our belief in the commercial attractiveness of Fovista stems not only from its efficacy but literature estimates that the prevalence of wet AMD is growing rapidly with an estimated ~200,000 additional wet AMD diagnoses per year.

Key Stats:	**(NASDAQ:OPHT)**
S&P 600 Health Care Index:	**1,209.56**
Price:	**$29.25**
Price Target:	$37.00
Methodology:	DCF Analysis
52 Week High:	$36.00
52 Week Low:	$23.00
Shares Outstanding (mil):	1.5
Market Capitalization (mil):	$43.0
Book Value/Share:	$0.00
Cash Per Share:	$2.93
Dividend (ann):	$0.00
Dividend Yield:	0.0%

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



Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2012E	--	--	--	--	0.0	--	--	--	--	(2.52)	NM
2013E	--	0.0	0.0	0.0	0.0	--	(0.65)	(0.25)	(0.30)	(1.20)	NM
2014E	0.0	0.0	0.0	0.0	0.0	(0.61)	(0.66)	(0.68)	(0.73)	(2.68)	NM

Source: Company Information and Leerink Swann LLC Research
2Q13 Revenue and EPS are 1H13 Actuals, since quarterly numbers not provided in S1. GAAP EPS presented.

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The Healthcare Investment Bank™

Ophthotech (NASDAQ: OPHT) Initiating at Outperform

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Ophthotech: Investment Thesis

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

OPHT Valuation and Risks to Valuation

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		Cost of Capital				
		10%	11%	12%	13%	14%
Terminal Growth Rate	-1%	$ 44.89	$ 40.19	$ 36.21	$ 32.80	$ 29.85
	0%	$ 46.00	$ 41.00	**$ 36.81**	$ 33.26	$ 30.20
	1%	$ 47.36	$ 41.98	$ 37.53	$ 33.79	$ 30.60
	3%	$ 51.24	$ 44.67	$ 39.44	$ 35.18	$ 31.63
	5%	$ 58.23	$ 49.15	$ 42.45	$ 37.26	$ 33.12

Source: Leerink Swann estimates

- **Valuation: We estimate a ~$37 per share value for OPHT based on a discounted cash flow analysis that assumes an 12% discount rate and a 0% terminal growth rate.** We project Fovista revenue growth from 2018 through 2027 in the US and EU and cut it significantly thereafter at the expiration of OPHT's method-of-treatment patent. We see upside to our valuation from either, 1) less robust competition than we anticipate, 2) the potential for Fovista to be best-in-class *even in the face of anti-PDGF competition*, or 3) the commercial potential of ARC195 which is not currently included in our model.
- **Risks:** Risks to our OPHT valuation include the possibility of disappointing clinical data, commercial shortfalls, or higher than anticipated regulatory hurdles. Since OPHT solely has one product in late-stage development, any of these could impact the stock significantly.

Wet AMD Presents a Significant and Growing Unmet Medical Need

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- Age-related macular degeneration is a progressive chronic disease of the central retina and a leading cause of vision loss worldwide
- The majority of visual loss in AMD occurs in the late stages of the disease: late dry AMD and wet AMD. **Wet AMD specifically is believed to be responsible for 90% of the severe vision loss associated with AMD**
- There are approximately 15MM patients with AMD in the US, 10-15% of which are estimated to be "wet", according to the AMD society, **implying ~1.85MM wet AMD patients in the US.**
- With the increasing prevalence of people above the age of 60, **the prevalence of wet AMD in the US has been increasing by ~200,000 patients per year and is expected to increase at an even faster rate going forward**
- Other behavioral and environmental risk factors listed on the right, such as obesity and cardiovascular risk, show that antecedents to AMD are quite common, relatively speaking

Panel 1: Risk factors for age-related macular degeneration

Environmental and behavioural factors

- Cigarette smoking
- Obesity
- Low dietary intake of vitamins A, C, and E, and zinc
- Low dietary intake of lutein and omega-3 fatty acids
- Unhealthy lifestyle related to cardiovascular risk factors

Genetic

- *CFH* (complement factor H; chr 1)
- *ABCA4* (ATP-binding cassette transporter; chr 1)
- *COL8A1* (collagen type 8 alpha 1 subunit; chr 3)
- *CF1* (complement factor 1; chr 4)
- *VEGFA* (vascular endothelial growth factor A; chr 6)
- *FRK/COL10A1* (fyn-related kinase/alpha chain of type X collagen; chr 6)
- *CFB* (complement factor B [properdin]; chr 6)
- *C2* (complement component 2; chr 6)
- ARMS2/*HTRA1* (HtrA-serinepeptidase1; chr 10)
- *LIPC* (hepatic lipase; chr 15)
- *CETP* (cholesterylester transfer protein; chr 16)
- *APOE* (apolipoprotein E; chr 19)
- *C3* (complement component 3; chr 19)
- *TIMP3* (tissue inhibitor of metalloproteinase 3; chr 22)
- *TNFRSF10A* (tumour necrosis factor receptor superfamily 10a; chr 8)

Other

- Hyperopic refraction

Source: Lancet

The Logic is there for Fovista Therapy: Deficiencies in the anti-VEGF Standard-of-Care

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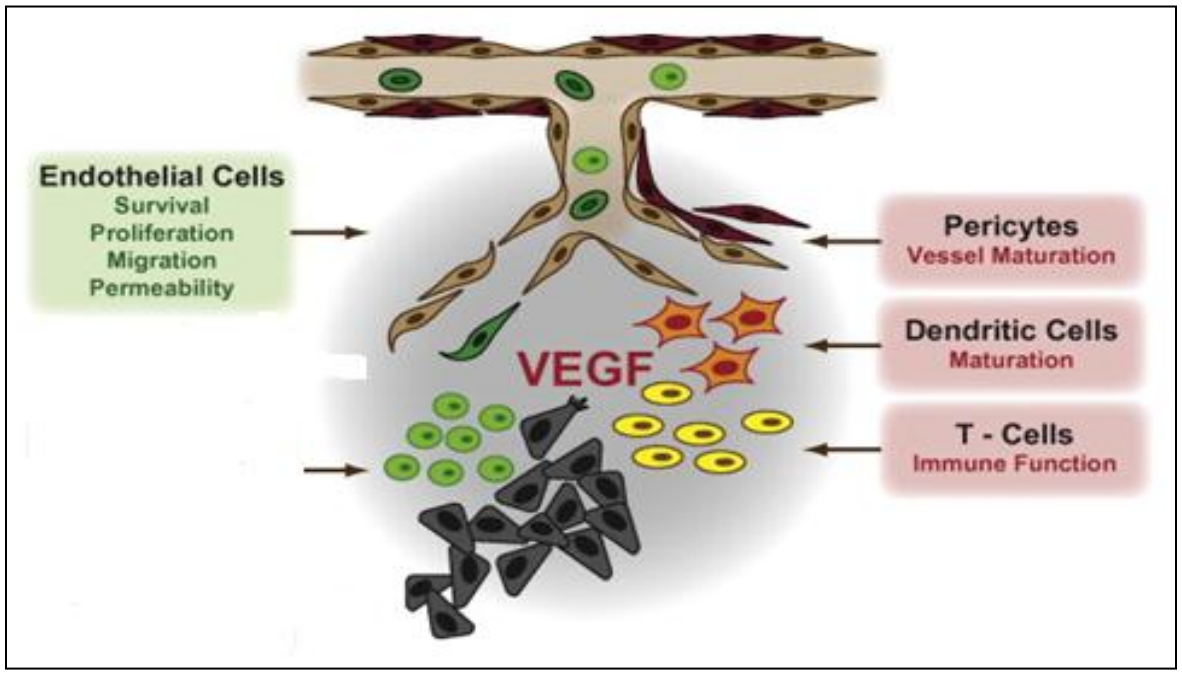
- While very compelling data exist that anti-VEGF monotherapy is effective in the treatment of wet AMD, **long-term outcomes studies show that after ~4 years of anti-VEGF treatment, patients' visual capacity generally returns to pre-treatment baseline, as demonstrated in the Lucentis HORIZON outcomes study**
- **This plateau effect and visual regression seen with anti-VEGF therapies is believed to stem from interference by pericytes** (shown in the diagram below), a group of cells that intimately cover and protect the neovascular complex when new blood vessels develop and mature. Some researchers theorize that the plateau effect seen with anti-VEGF agents is due to the fact that anti-VEGF therapies can only reach the minority of endothelial cells on the neovascular complex that are not protected by a pericyte layer



Source: Science Direct

Thus, OPHT is developing Fovista to target and inhibit the activity of PDGF (Platelet-derived growth factor) which is responsible for pericyte recruitment, survival, and maturation. Such a strategy could render the leaky neovasculature responsible for wet AMD pathogenesis significantly more vulnerable to anti-VEGF treatments

Fovista Add-On Therapy Conferred a Robust Benefit in Phase IIb

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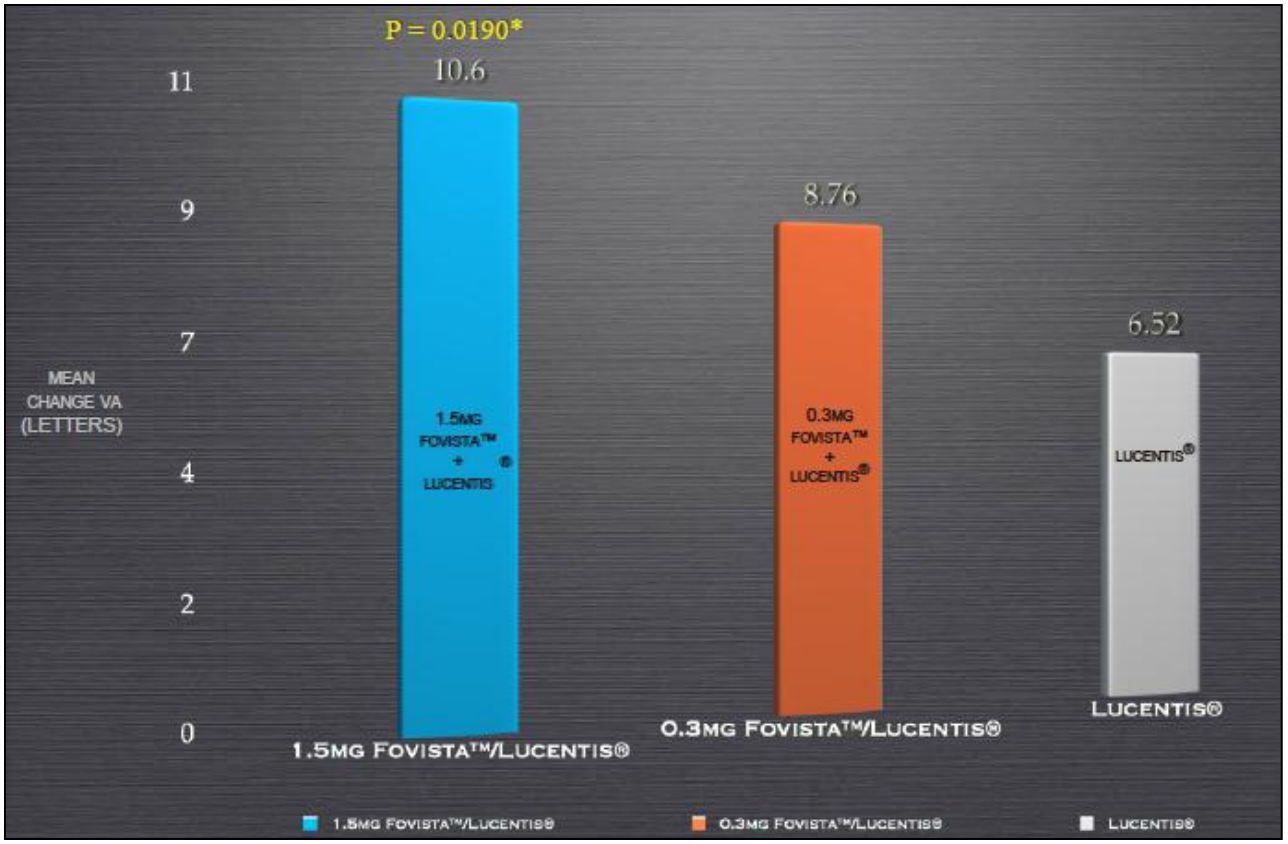
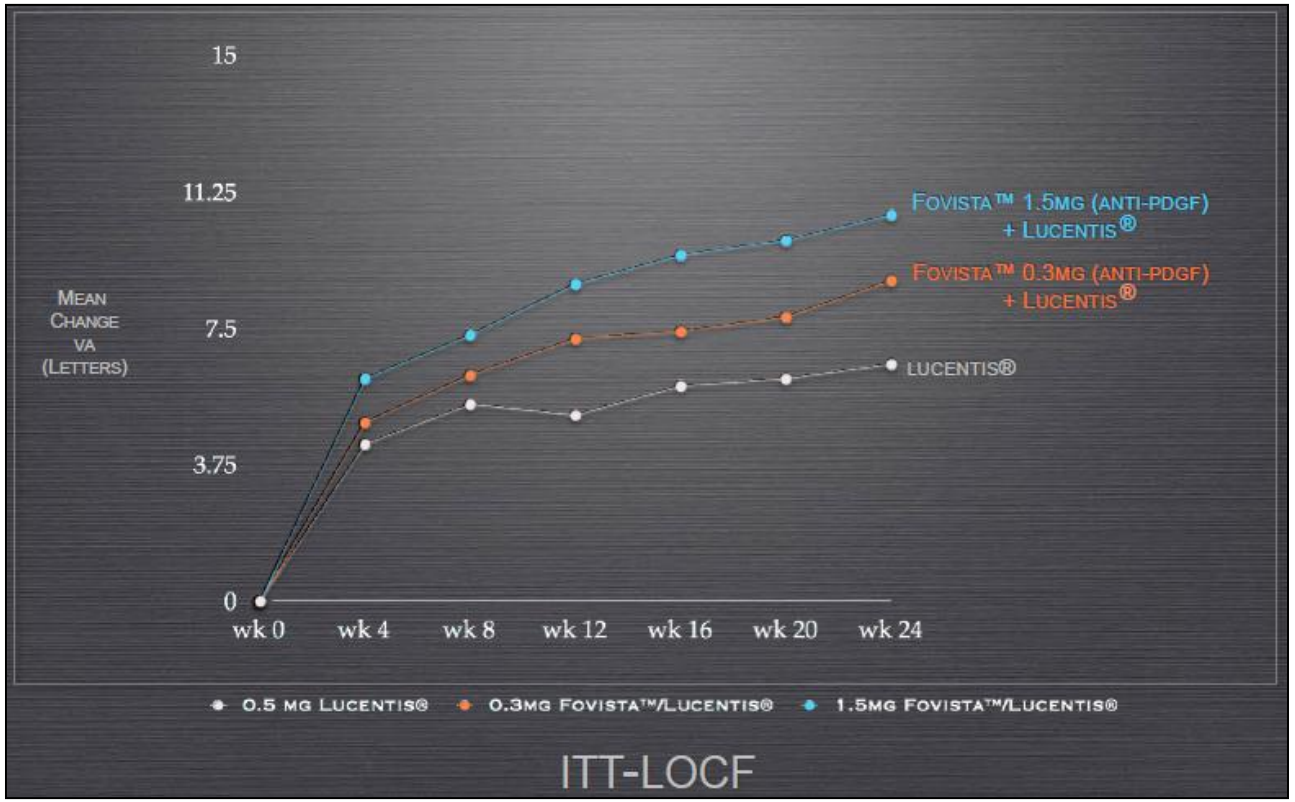
- As an add-on to Lucentis, Fovista therapy was able to confer a 62% additional benefit to treated wet AMD patients (p=0.019) at week 24
- Such a benefit was comprised of an additional ~4 letter improvement on the Early Treatment Diabetic Retinopathy Study (ETDRS), a well-established standardized chart of vision testing used in clinical trials
- Fovista was generally well tolerated in Phase IIb: no cases of eye infection were observed, and only one Fovista treated patient demonstrated severe intraocular inflammation, but this was at the lower 0.3mg dose



Source: Company Presentations

Additional Benefits from Fovista May Peak Later than Week 24

- In Phase IIb, the addition of 1.5mg of Fovista to 0.5mg Lucentis was able to confer a 62% additional benefit to treated patients over 0.5mg of Lucentis monotherapy at week 24. **However, shown in the chart on the right, the difference between these treatment groups broadened throughout the study**
- **Thus, it is possible that Fovista's additional benefit could be greater over longer periods of time, and in Phase III, the change in visual acuity will be evaluated at week 48**



Source: Company Presentations

Fovista Ph. III Similarly Structured, Rendering it De-Risked By Ph. II, We Believe

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- **In Phase III, OPHT have employed identical exclusion criteria to those utilized in the successful Phase II.** The trial is composed of three studies, and in two the treatment group will receive 1.5mg of Fovista + Lucentis**.** A separate study will recruit ~50% more patients to evaluate 3 groups: Fovista + Eylea and Fovista + Avastin, each of which will be compared to Eylea or Avastin control arms. With the strong uptake of Eylea, we believe that the third study group is key, and **expect Fovista's pivotal trial to indicate a label that enables Fovista add-on therapy to combined with any anti-VEGF agent**

OPHT: Phase III Fovista Program

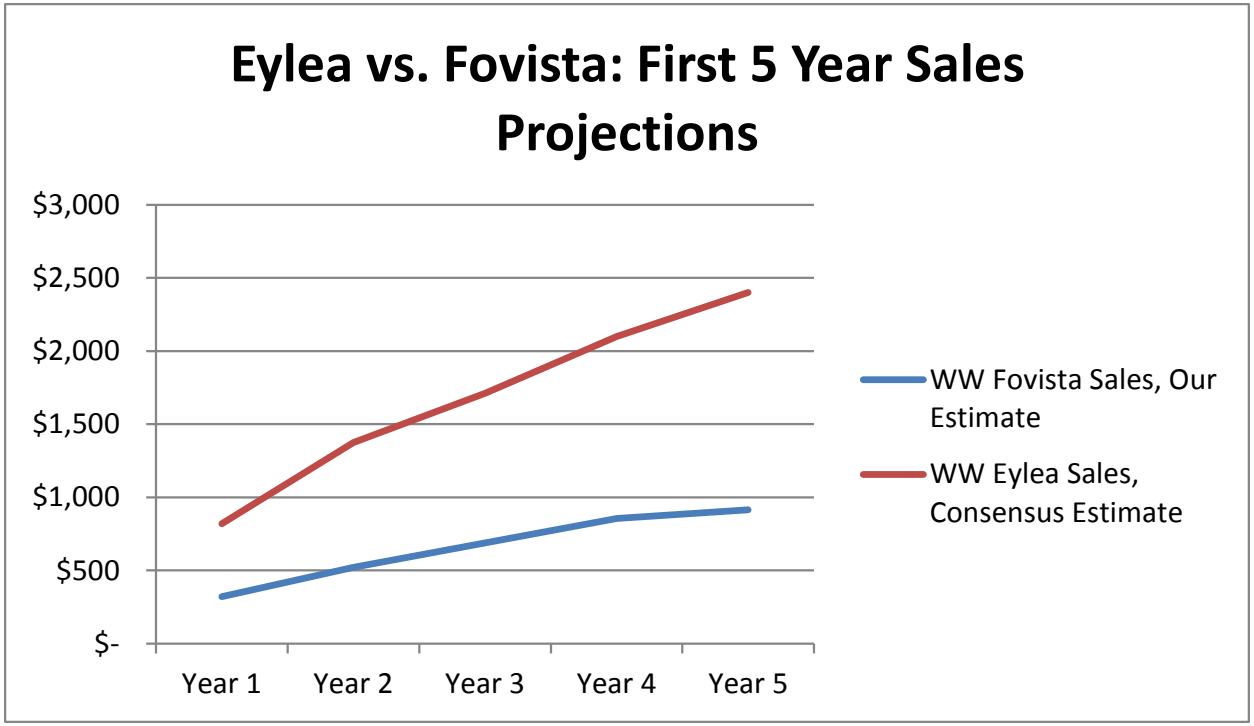
- Strategy – Replicate Phase 2b – 2 Trials vs. Lucentis
  - Primary Endpoint - Mean change in visual acuity from baseline at one year
- Expand Label in a Third Trial to Eylea and Avastin
- Phase 3 Data – Mid 2016
- NDA Filing – 2H 2016

To recruit ~1,866 patients at up to ~225 treatment centers internationally

Source: SEC Filings and Company Presentations

As Evidenced by Eylea Launch, the Wet AMD Market Adopts New Products Very Rapidly

- In its first full year on the market, Eylea product sales were $817.9MM, and are estimated by the Street to exceed $1.35B in 2013, its second full year on the market
- The Street models ~$2.4B for Eylea in 2016 (its 5th full year on the market**). For Fovista, we model slightly less than $1B in worldwide sales in 2022 (Fovista's 5th full year on the market), which holds the potential to be conservative we believe, given the "quick adoption" dynamic of the wet AMD market** and the fact that the prevalence of wet AMD is expected to increase by >1MM patients between 2016-2022



Source: Leerink Swann estimates; FactSet

While the comparison between the two agents is not apples-to-apples, the market's rapid uptake of Eylea is in our view a bullish sign for a Fovista launch. We assume that Fovista generates ~31% of the revenues that Eylea is expected to generate in its first five years on the market, but nonetheless arrive at an OPHT per share price at a 28% premium to where the stock closed on 10/16.

We Model a 75% Probability that Heavy Competition Enters the Market in 2021

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- There are multiple agents that could be potentially competitive to Fovista in the wet AMD pipeline, and thus **we assume a 75% likelihood that Fovista's wet AMD market penetration remains flat starting in 2021**
- **However, we believe our assumption that competition steals potential market share from OPHT could be conservative**, since most agents that could have an anti-PDGF (or dual anti-VEGF/anti-PDGF) effect are considerably less proven than Fovista in the clinic
- **Potentially competing agents include**: AGN's DARPin (Phase II), REGN's Eylea/anti-PDGF (pre-clinical), Xcovery vision's oral anti-VEGF/anti-PDGF (Phase I), and Neurotech and SomaLogic's anti-PDGF therapies (each preclinical)

Fovista IP Protection in US/EU/Japan Until 2027

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- OPHT holds strong intellectual property protection over Fovista including a method-of-treatment patent that expires in the 2027 and covers Fovista in the US, Europe and Japan.
- Other patents/applications include: composition-of-matter patents that expire in 2017 in the US and 2018 in EU/Japan, a US patent covering methods of treating AMD with a combination of an –anti-PDGFR aptamer and an anti-VEGF-A antibody or binding fragment thereof, patent applications in various jurisdictions covering the treatment of wet AMD with a combination of Fovista/Eylea which, if granted, would expire in 2030 in the US.
- **In our model we cut our Fovista sales estimates significantly in the year(s) after such exclusivities expire, but believe this holds the potential to be conservative, given that OPHT could benefit from up to 5 years of patent term extension after expiry in 2027.**

OPHT Management: Strong Team with Industry Experience

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Team Member	Role	Industry Experience
David R. Guyer M.D.	*CEO*	Partner at SV Life Sciences, CEO of Eyetech Pharmaceuticals
Samir Patel M.D.	*President*	Director of the Retinea Service and Residency Program at University of Chicago Department of Ophthalmology
Bruce Peacock	*CFO/CBO*	CEO of Alba Therapeutics, Venture Partner at SV Life Sciences
Richard L. Beckman M.D.	*CMO*	CMO at Neurotech Pharmaceuticals, Positions at Alcon, Lux Biosciences, Danube Pharmaceuticals BDX, AGN
Eveyln Harrison	*COO*	SVP Clinical Research and Development at Eyetech Pharmaceuticals

Source: Company Website

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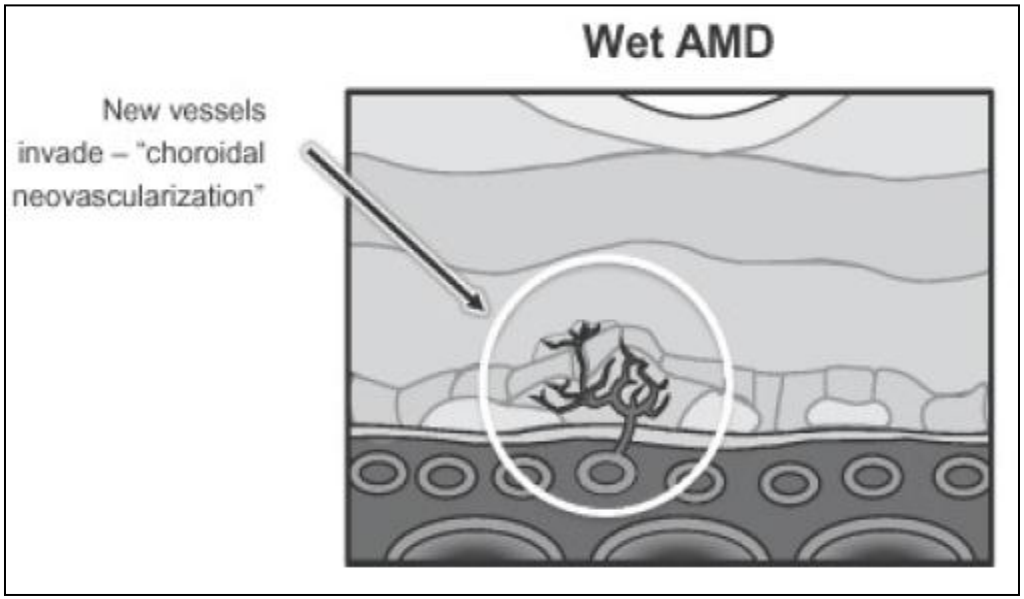
Wet AMD Background

Wet AMD Background

- Age-Related Macular Degeneration (AMD) is a leading cause of vision in people over the age of 50 in the western world
- There are two forms of AMD: dry AMD and wet AMD
- Wet AMD is preceded by dry AMD, and is triggered by the development of new and abnormal blood vessels that invade the retina. These abnormal new blood vessels originate beneath the retina in a layer called the choroid, and invade into the overlying retinal layers via a process called “choroidal neovascularization” (CNV).

- *Shown in the picture on the right, abnormal new blood vessels in Wet AMD patients tend to be fragile and often bleed and leak fluid into the macula, the central portion of the retina responsible for detailed central vision and color perception*
- **In Wet AMD, untreated blood vessel growth and associated leakage typically lead to retinal distortion, scarring, irreversible destruction of the macula and loss of vision.**



Source: *SEC Filings*

Wet AMD Background (continued)

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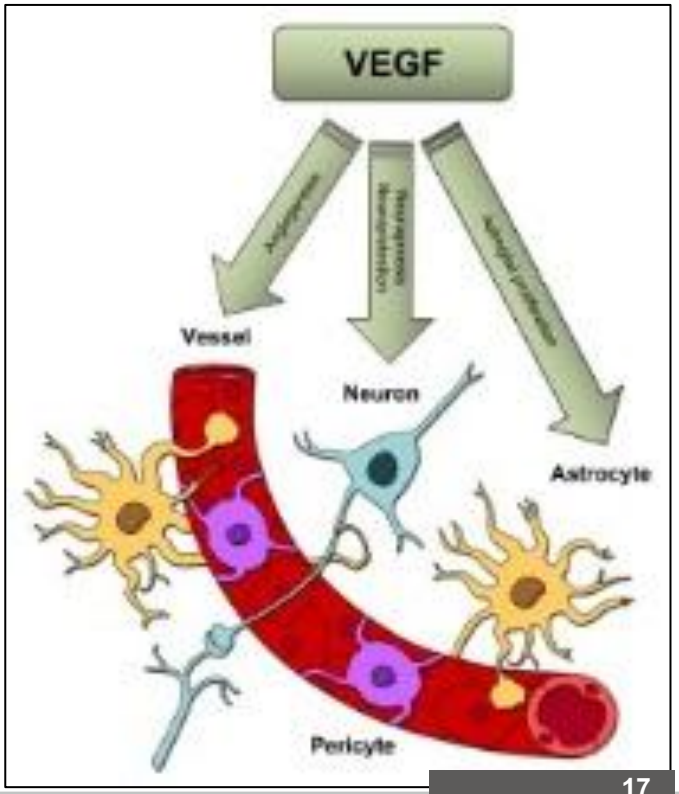
- According to a study on the burden of AMD published in 2006 in the peer reviewed journal *Current Opinion in Ophthalmology*, approximately 1.25MM people in the US suffer from Wet AMD with 200,000 new diagnoses each year
- The AMD alliance international estimates that the direct healthcare system costs of visual impairment worldwide due to dry and wet AMD is ~$255B
- There are no FDA approved treatments for dry AMD, which in some patients can render its progression into wet AMD inevitable
- **While wet AMD solely represents 10-15% of all cases of AMD, it is responsible for ~90% of the severe vision loss associated with the disease**

Vascular Endothelial Growth Factor: An Established Target in Wet AMD

- Abnormal new blood vessels in wet AMD are predominantly made up of two cell types: endothelial cells and pericytes
- The proliferation of endothelial cells is driven by the binding of Vascular Endothelial Growth Factor (VEGF), which provides survival signals and is also one of the most potent inducers of blood vessel permeability.

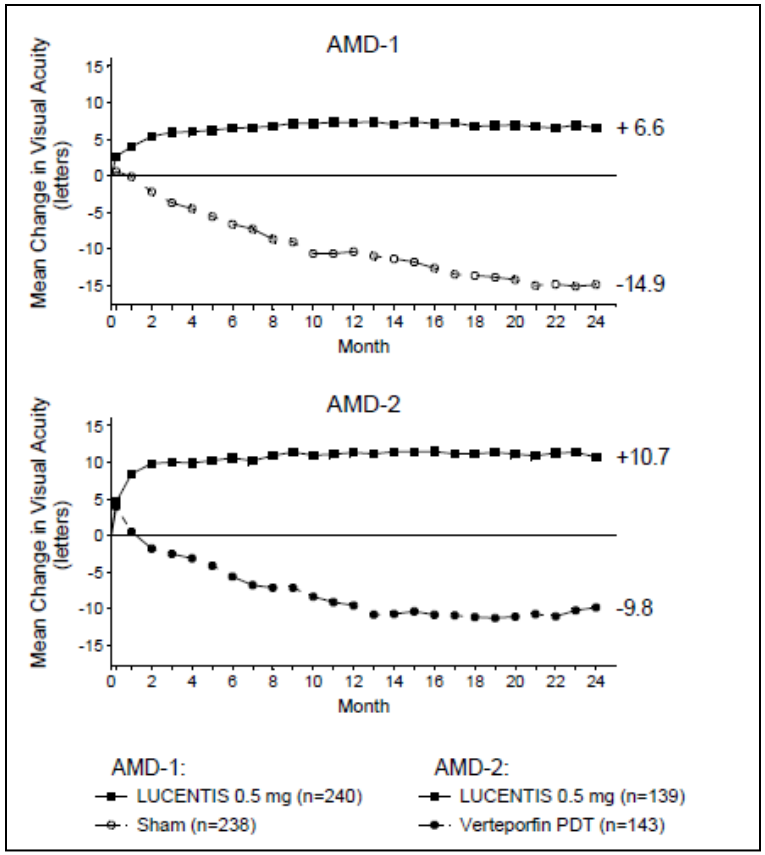
- VEGF is a naturally occurring glycoprotein in the body that regulates angiogenesis in various cell types and organs (demonstrated in the picture on the right), has in the past been a viable target in some cancers, and is believed to be one of the primary drivers of the pathological effects of neovascularization in AMD
- VEGF contributes mainly to the initiation stage of CNV by promoting not only angiogenesis but **vasculogenesis, the latter of which enables the formation of pores in vascular endothelial cells, incurring leakage and differentiating "wet" AMD from its dry precursor**

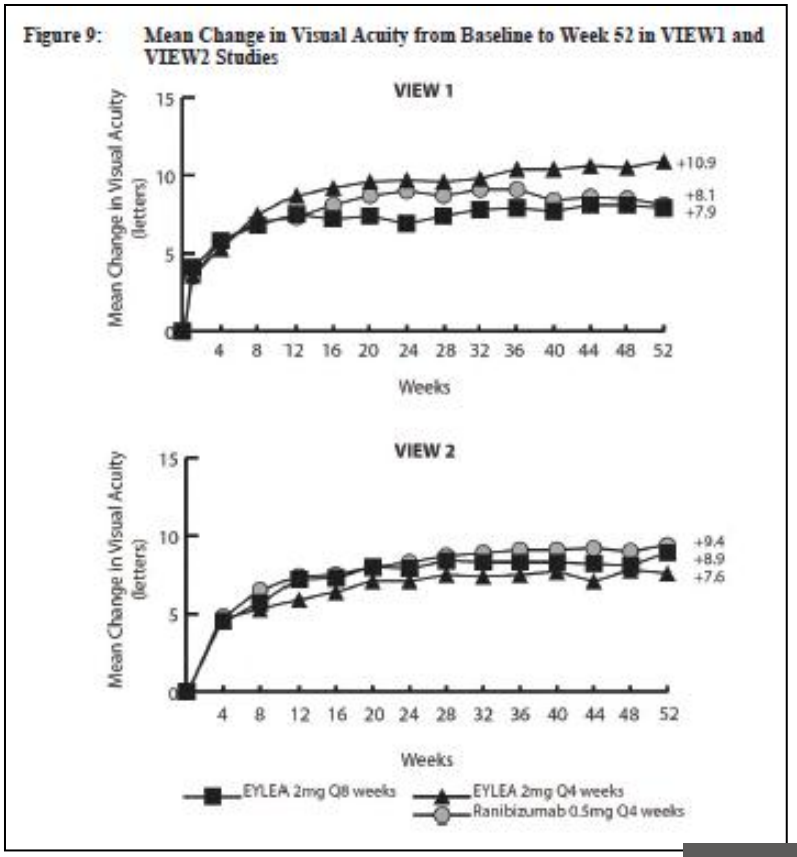


Source: Current Neurovascular Research

Vascular Endothelial Growth Factor: An Established Target in Wet AMD (continued)

- In attempt to inhibit choroidal neovascularization, Roche's Lucentis, REGN's Eylea, and Avastin (used off-label) bind VEGF and prevent it from hitting its target and incurring CNV
- **Shown below (Lucentis data on the left and Eylea non-inferiority to Lucentis data on the right), in multiple clinical trials, anti-VEGF agents have shown a significant ability to improve and stabilize vision in wet AMD patients**





Source: FDA Prescribing Information

Anti-VEGF Market Dynamics

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- The FDA has approved the anti-VEGF drugs Lucentis (ranibizumab) and Eylea (aflibercept) which in 2012 generated combined worldwide sales of $4.8B
- Lucentis is marketed by Roche in the US and NVS (OP) in Europe while Eylea is marketed by REGN in the US and Bayer AG in ROW markets
- Additionally, although solely approved by the FDA as a cancer therapy, the anti-VEGF drug Avastin (bevacizumab) is used off-label to treat wet AMD and is available through compounding pharmacies at a significantly lower price per dose than either Lucentis or Eylea
- Based on OPHT's research, company reports, and 2012/2013 sales figures, we estimate that Lucentis/Eylea/Avastin wet AMD market share is 30%/24%/47% in the US and 63%/13%/24% in the EU/ROW, and we expect Eylea's share to increase in each territory
- **Most importantly, because Fovista's ongoing Phase III program includes treatment groups that are administered Fovista with each of Lucentis, Eylea and Avastin, Fovista's label should ultimately allow use with any available anti-VEGF agents for the treatment of wet AMD**

Long-Term anti-VEGF Data Shows Need for New Therapies

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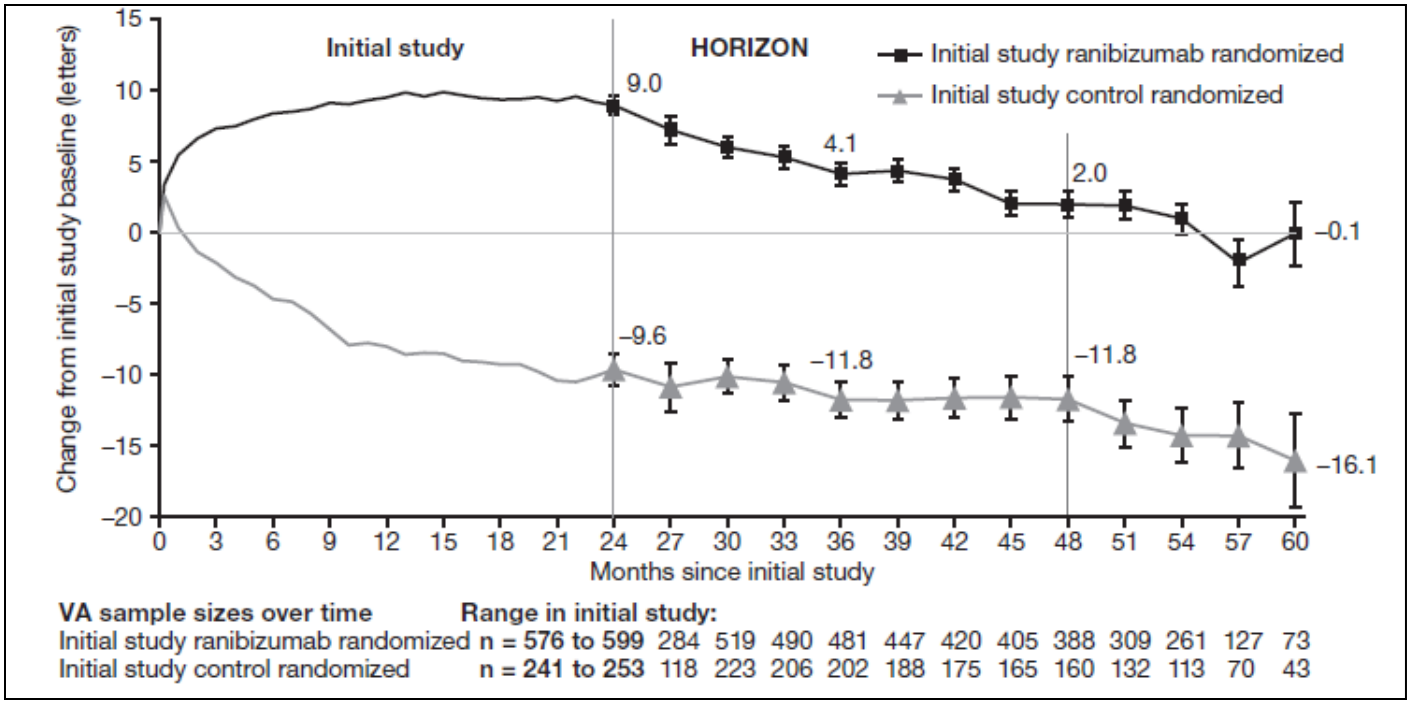
- **Longer-term research shows that the benefits of anti-VEGF therapy may not persist beyond a few years after the initiation of treatment**
- In the Lucentis HORIZON study (n=853), patients were treated for 2 years with monthly injections of Lucentis and subsequently treated on an as-needed basis.
- Shown in the chart below, **by the end of year 5, Lucentis treated patients' visual acuity had on average returned to baseline levels**. **We believe this suggests that many wet AMD patients would be good candidates for Fovista combo-therapy with an anti-VEGF agent.**



VA sample sizes over time	Range in initial study:												
Initial study ranibizumab randomized	n = 576 to 599	284	519	490	481	447	420	405	388	309	261	127	73
Initial study control randomized	n = 241 to 253	118	223	206	202	188	175	165	160	132	113	70	43

Seven year Lucentis outcomes data shows a similar trend, where 34% of patients showed a decline of 15 letters from baseline and 37% of patients had vision of 20/200 at the end of the study

Source: Journal of Ophthalmology

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Fovista Add-On Therapy Could Become a Staple of the Wet AMD Standard of care

OPHT's Fovista is a 32-mer DNA Aptamer

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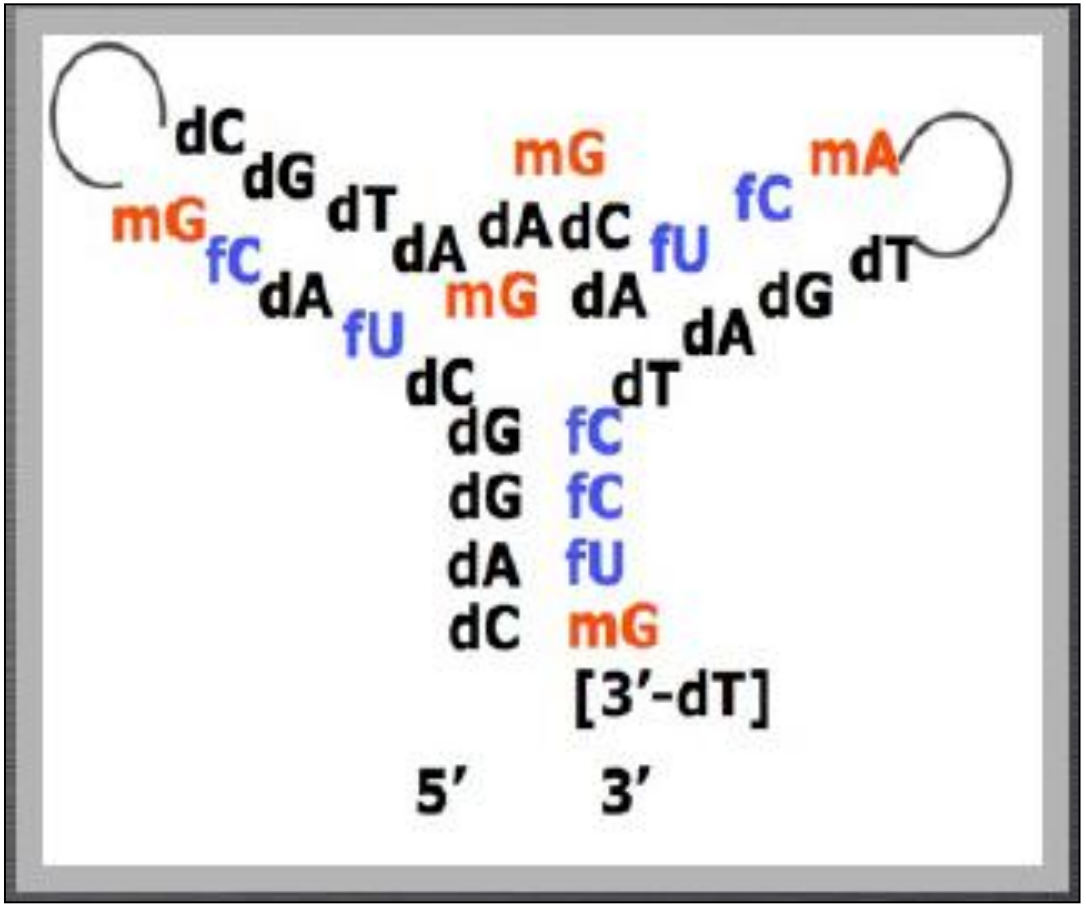
- Fovista is a 32-mer DNA Aptamer that binds to Platelet Derived Growth Factor
- OPHT has measured Fovista's inhibition of PDGF's ability to bind to both its receptors *(PDGFR-alpha/PDGFR-beta)* and found that Fovista strongly inhibits PDGF's binding capability with a potency equal to an antibody that has downstream effects.
- The anti-PDGF ingredient in Fovista is a chemically synthesized aptamer, comprised of a single strand of nucleic acid that adopts a 3-D structure and binds to an extracellular target



Source: Company Presentations

Fovista hits the wet-AMD pathway of pathogenesis from a different angle than anti-VEGFs

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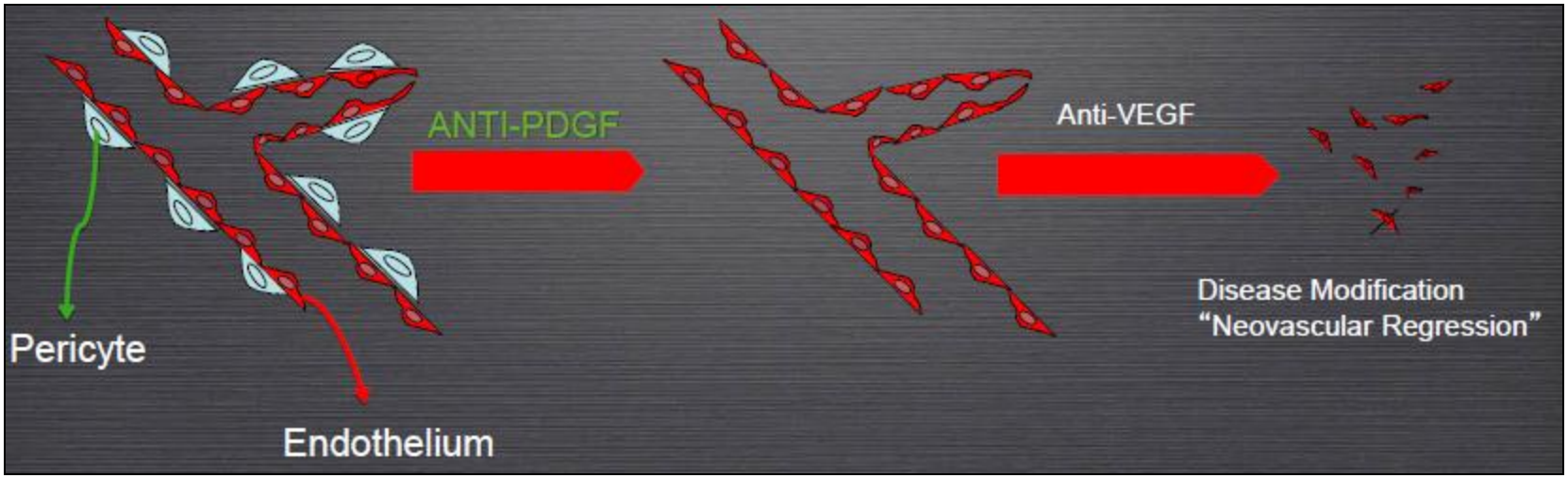
- Fovista exerts its biological effect by binding to PDGF, blocking PDGF's interaction to the pericyte cell surface receptor PDGF-beta.
- This results in stripping or the death of pericytes interrupting cell survival signals
- PDGF-B has been shown in multiple independent studies to be critical for pericyte survival and proliferation
- Pericyte coverage protects new blood vessels from anti-VEGF induced disruption
- PDGF necessary for pericyte cell survival and proliferation
- Results in a potentially more robust **anti-angiogenesis effect with a greater capacity for new vessel disruption and regression**



Source: Company Presentations

Phase I Trial Results: Fovista Safety Profile

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- **No Fovista dose limiting toxicity was found in OPHT's Phase I trial**
- Fovista with Lucentis was found to be generally well tolerated and no drug related adverse events were observed in Phase I
- Adverse events were primarily ocular adverse events related to the injection procedure, but none were related to Fovista or Lucentis and no patients discontinued
- No immunologic reactions observed in Fovista treatment arm

Visual Acuity: Primary Endpoint in Fovista Clinical Trials

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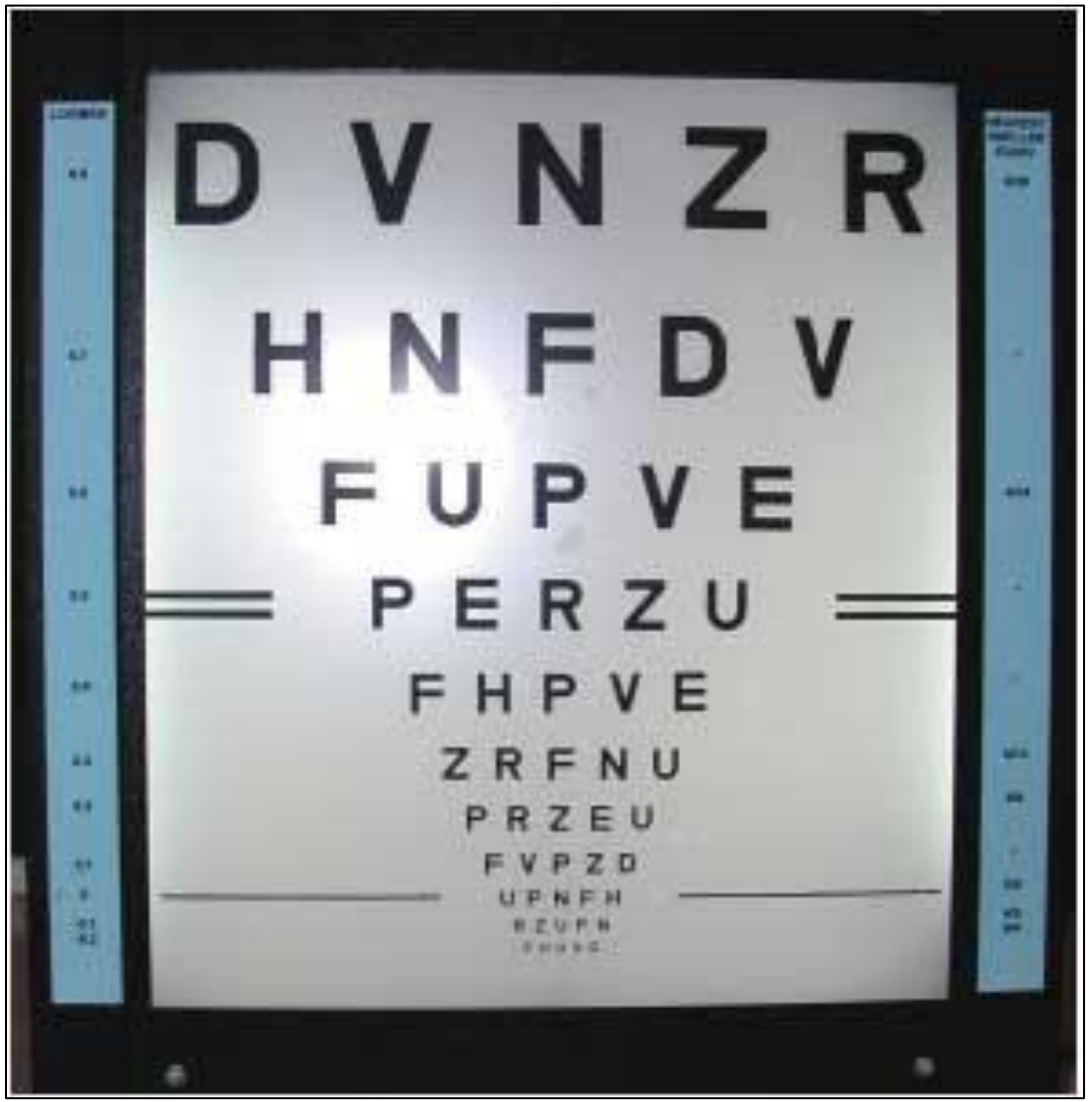
- Visual Acuity (or change in visual acuity) is a validated primary endpoint in ophthalmology clinical trials
- Visual acuity is measured as the number of letters, arranged in lines, that the patient can read on the Early Treatment Diabetic Retinopathy Scale (ETDRS) eye chart
- Each line on the ETDRS eye chart has five letters
- Normal visual acuity is commonly referred to as 20/20 vision, but to qualify to enter a Fovista clinical trial, a patient needs (and needed) to have visual acuity between 20/63 and 20/2000

Early Treatment Diabetic Retinopathy Scale – Found Everywhere, Likely at a Doctor Near You



Source: National Health Society UK

Fovista Phase II Structure and Background

LEERINK SWANN

- OPHT's Fovista Phase II was a double-blind, 449 patient study that took place at 69 centers in the US, South America, EU, Israel.
- Patients were randomly assigned to either 0.3mg Fovista+ 0.5mg Lucentis, 1.5mg Fovista + 0.5mg Lucentis or 0.5mg Lucentis w/sham Fovista injection (placebo)
- The primary efficacy endpoint was mean change in visual acuity from baseline at week 24
- Secondary endpoints included: proportion of patients in each treatment group gaining 15 or more ETDRS letters from baseline at 12 and 24 weeks; mean change in area of chorodial neovascularization from baseline at 24 weeks
- Statistics were evaluated using the Hotchberg procedure – multiple comparisons necessitated using p=0.025 as significance level
- Patients who received any prior treatment for AMD in the study eye (except oral supplements/vitamins or minerals) were excluded from participating in the study

Fovista Phase II: Baseline Demographics

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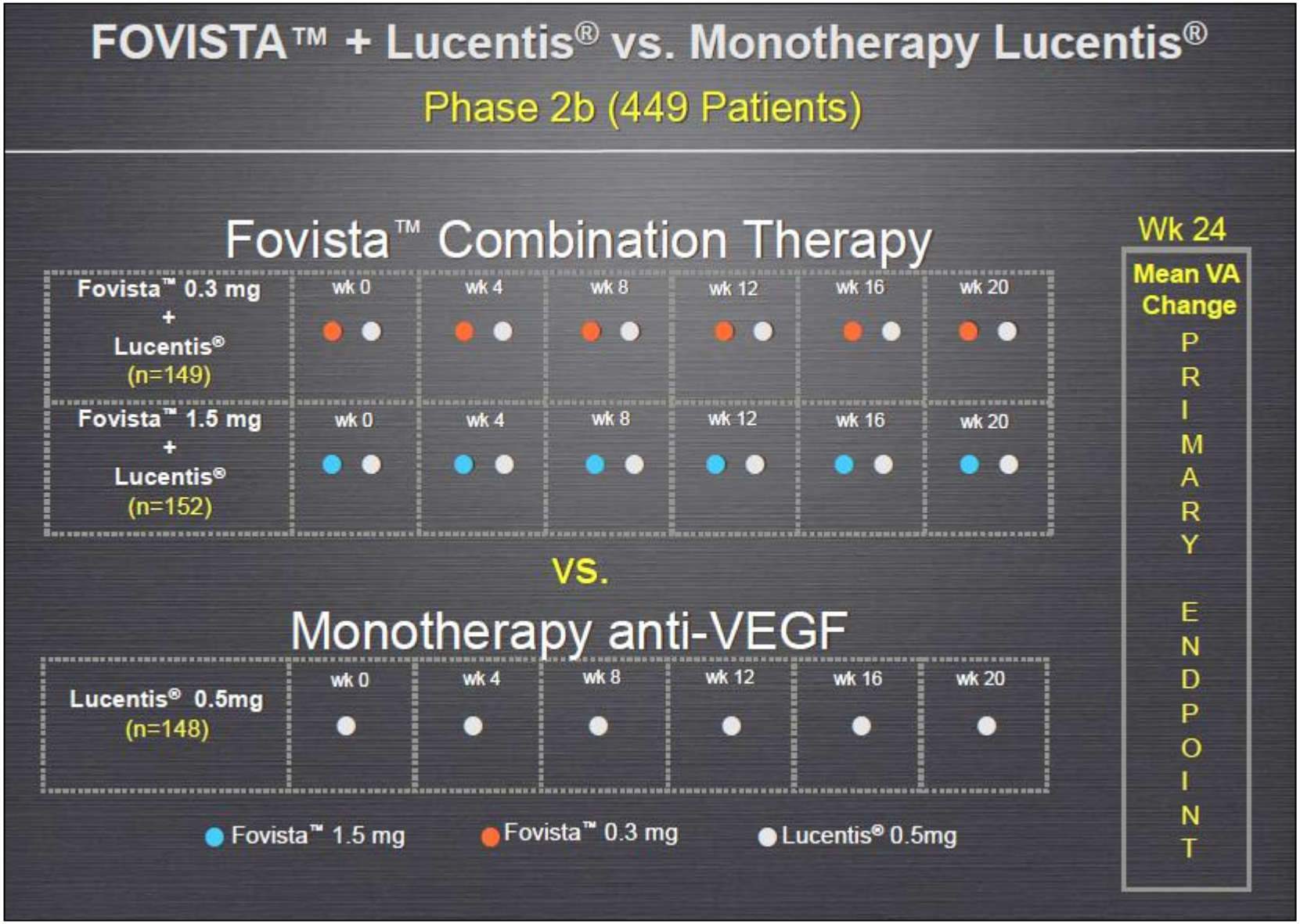
	Lucentis® N=148	0.3 mg FOVISTA™ (ANTI-PDGF) + LUCENTIS®	1.5 mg FOVISTA™ (ANTI-PDGF) + LUCENTIS® N=152
Female	93 (62.8%)	90 (60.4%)	92 (60.5%)
Caucasian	144 (97.3%)	145 (97.3%)	149 (98.0%)
Mean Age (years)	78	77.6	77.8
LESION SIZE * (DA)	1.8 *	1.9 *	1.5 *
Baseline VA (letters)	50.6	50.6	49.3
Active smoker	13 (8.8%)	21 (14.1%)	17 (11.3%)

Source: Company Presentations

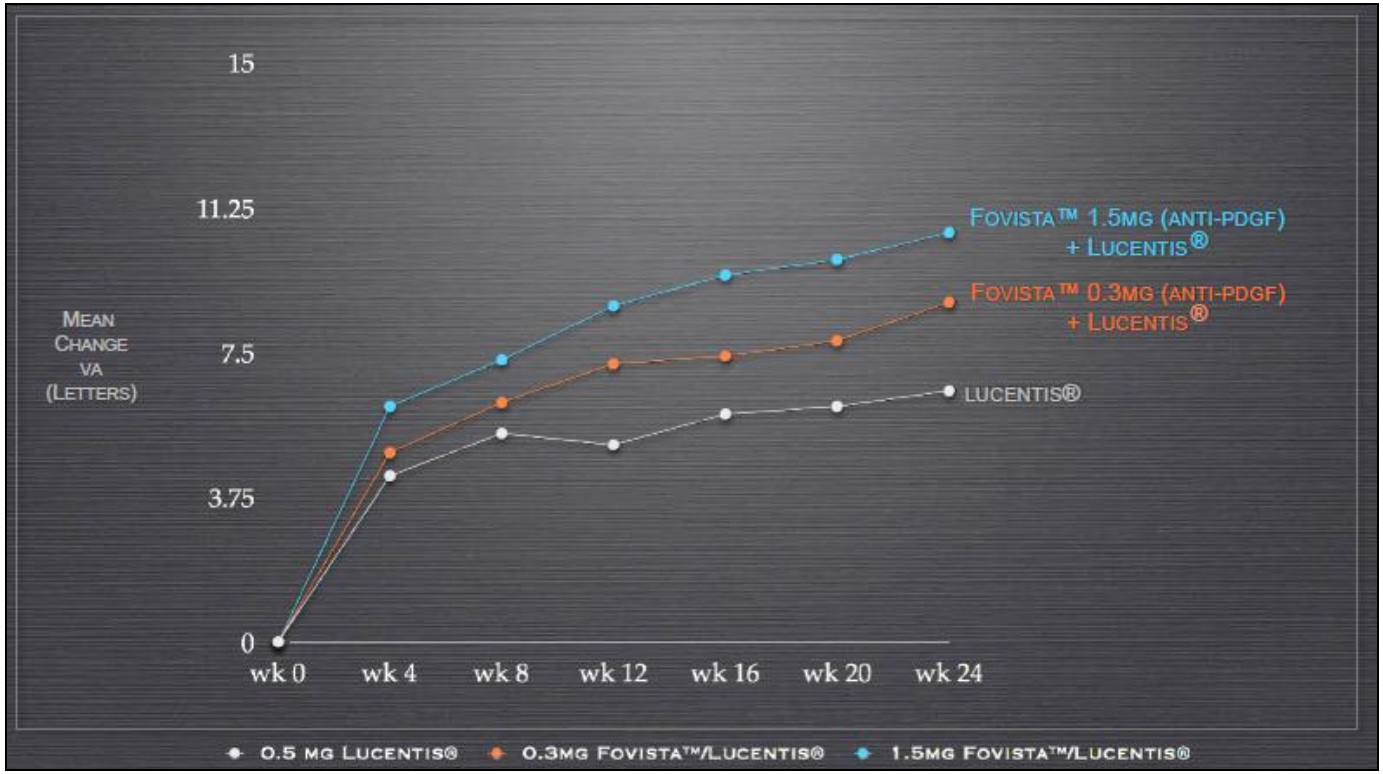
Fovista Phase II Trial Structure; 2 Doses Added on to Lucentis, Compared w/ Lucentis Monotherapy



Source: Company Presentations

Phase IIb Fovista Data – Trial Success, Primary Endpoint Met

- **At 24 weeks, patients receiving the combination of 1.5mg of Fovista and Lucentis gained a mean of 10.6 ETDRS letters compared to a mean improvement of 6.5 ETDRS letters for patients just receiving Lucentis monotherapy, representing a 62% comparative benefit from baseline (p=0.019)**
- A Fovista dose response was observed, as patients receiving 0.3mg Fovista + Lucentis gained 8.8 ETDRS letters from baseline which was not statistically significant but a represented 35% benefit nonetheless

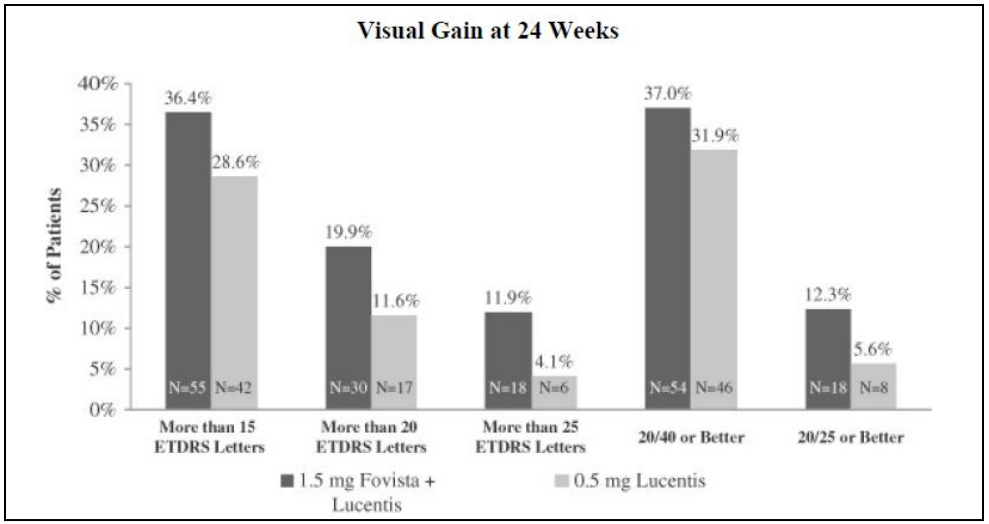


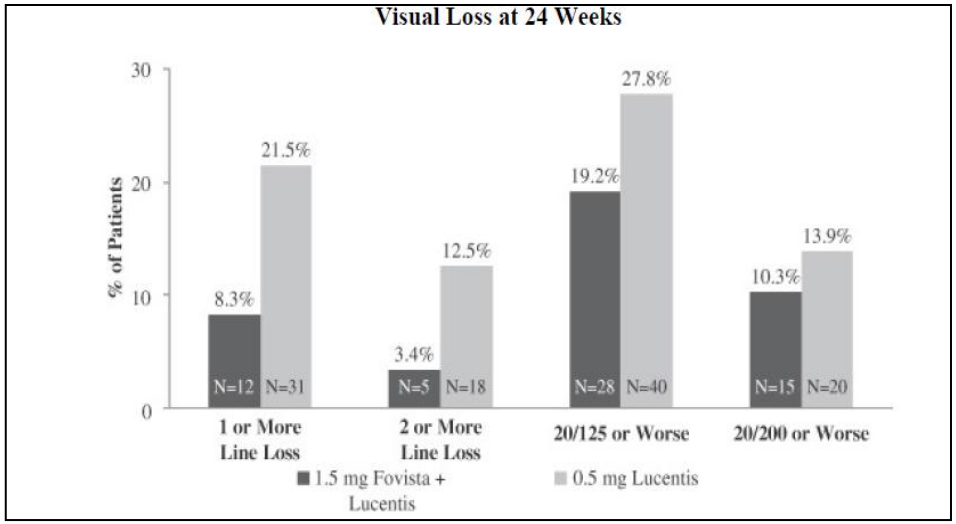
Source: Company Presentations

Additional Analyses Show Effect On Proportion of Patients with Visual Gain or Loss

LEERINK SWANN

- OPHT observed differences in the proportion of patients that showed improvement when measured by the number of lines of improvement in visual acuity from baseline, favoring the combination of 1.5mg of Fovista + Lucentis compared to Lucentis Monotherapy
- The graphs below show - for each of the 1.5mg of Fovista + Lucentis/Lucentis monotherapy groups - the percentage of patients in such treatment group who gained the specified number of lines in visual acuity and the percentage of patients whose final visual acuity improved to the specified level.
- **We believe these additional assessments show that pretty much any way you cut the data, Fovista add-on therapy was able to confer a robust clinical benefit to patients in the Phase IIb**





Source: SEC Filings

Additional Analyses Show Effect On Proportion of Patients Gaining 15 or More ETDRS Letters

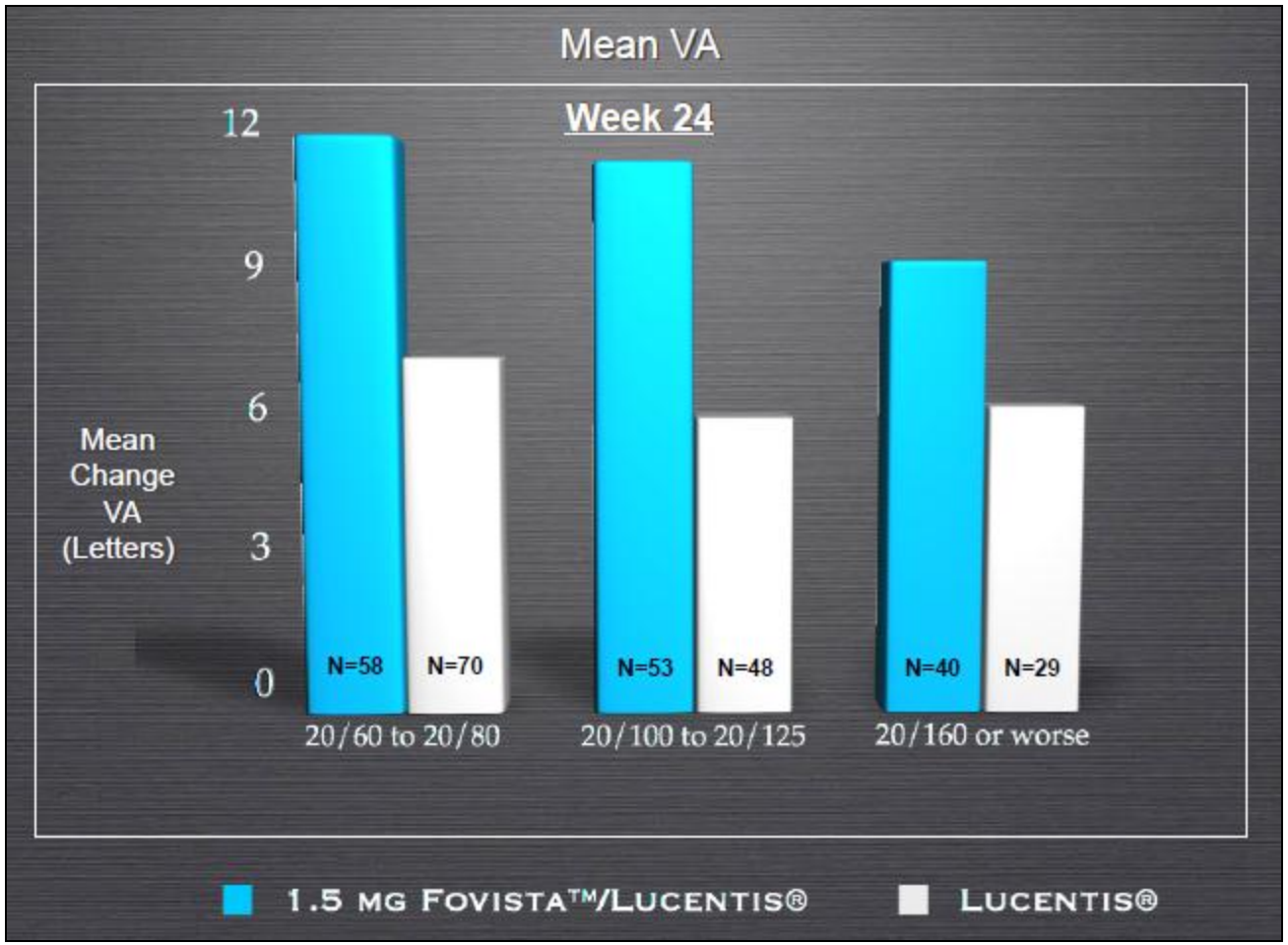
LEERINK SWANN

- **The analyses below were pre-specified and further validate Fovista's treatment benefit, we believe**
- A greater than 15 letter improvement is believed to represent a *significant visual benefit* in wet AMD

	Week 12	Week 24
Arm	# (%) Gaining ≥ 15 letters	# (%) Gaining ≥ 15 letters
0.3mg Fovista + Lucentis	31 (21.4%)	49 (33.3%)
1.5mg Fovista + Lucentis	48 (31.8%)	59 (39.1%)
Lucentis Monotherapy	33 (22.4%)	50 (34.0%)

Source: Company Presentations

Fovista's Benefit was Consistent Across Different Levels of Baseline Vision



Source: Company Presentations

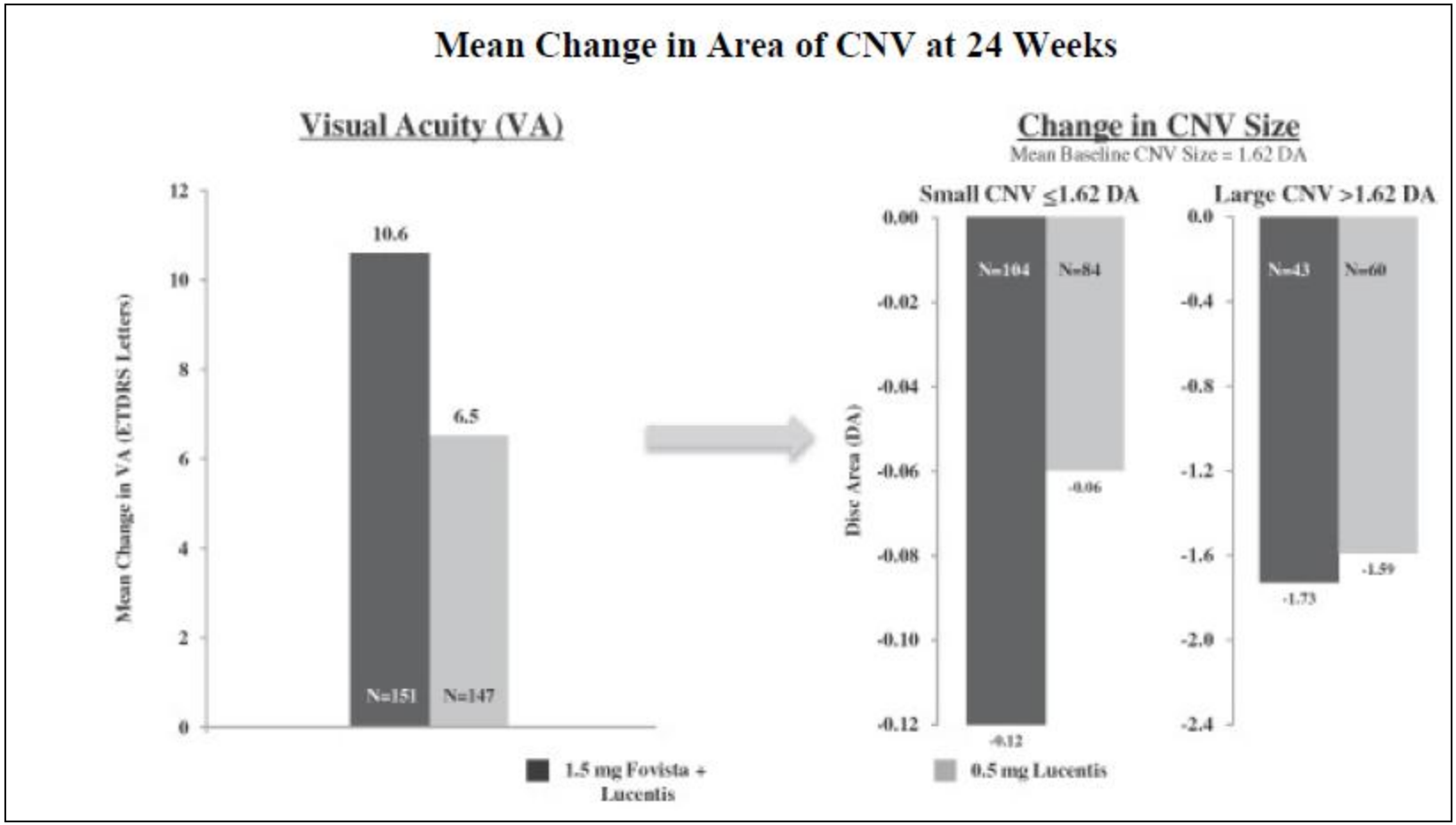
A robust benefit in patients 20/160 or worse supports Fovista use in anti-VEGF monotherapy non-responders. The fact that results were not driven by particular subgroups also provides confidence that positive results are likely to be replicated in Phase III.

VA=visual acuity

Anatomical Changes Seen in Phase IIb Participants Corroborate Fovista's Mechanism of Action

LEERINK SWANN

- Anatomical changes seen in choroidal neovascularization (CNV) in the Phase IIb participants validate the biological relevance of Fovista's activity, we believe



Source: SEC Filings

Phase IIb Safety Data Show Little Difference Between Fovista Add-On and Lucentis Monotherapy

LEERINK SWANN

Ocular Serious Adverse Events	Monotherapy Lucentis® N = 148	0.3mg Fovista™ + Lucentis® N = 149	1.5mg Fovista™ + Lucentis® N = 152
Eye disorders	**1 (0.7%)**	**1 (0.7%)**	**1 (0.7%)**
Corneal erosion	0 (0.0%)	0 (0.0%)	1 (0.7%)
Uveitis	0 (0.0%)	1 (0.7%)	0 (0.0%)
Visual acuity reduced	1 (0.7%)	0 (0.0%)	0 (0.0%)

Source: Company Presentations

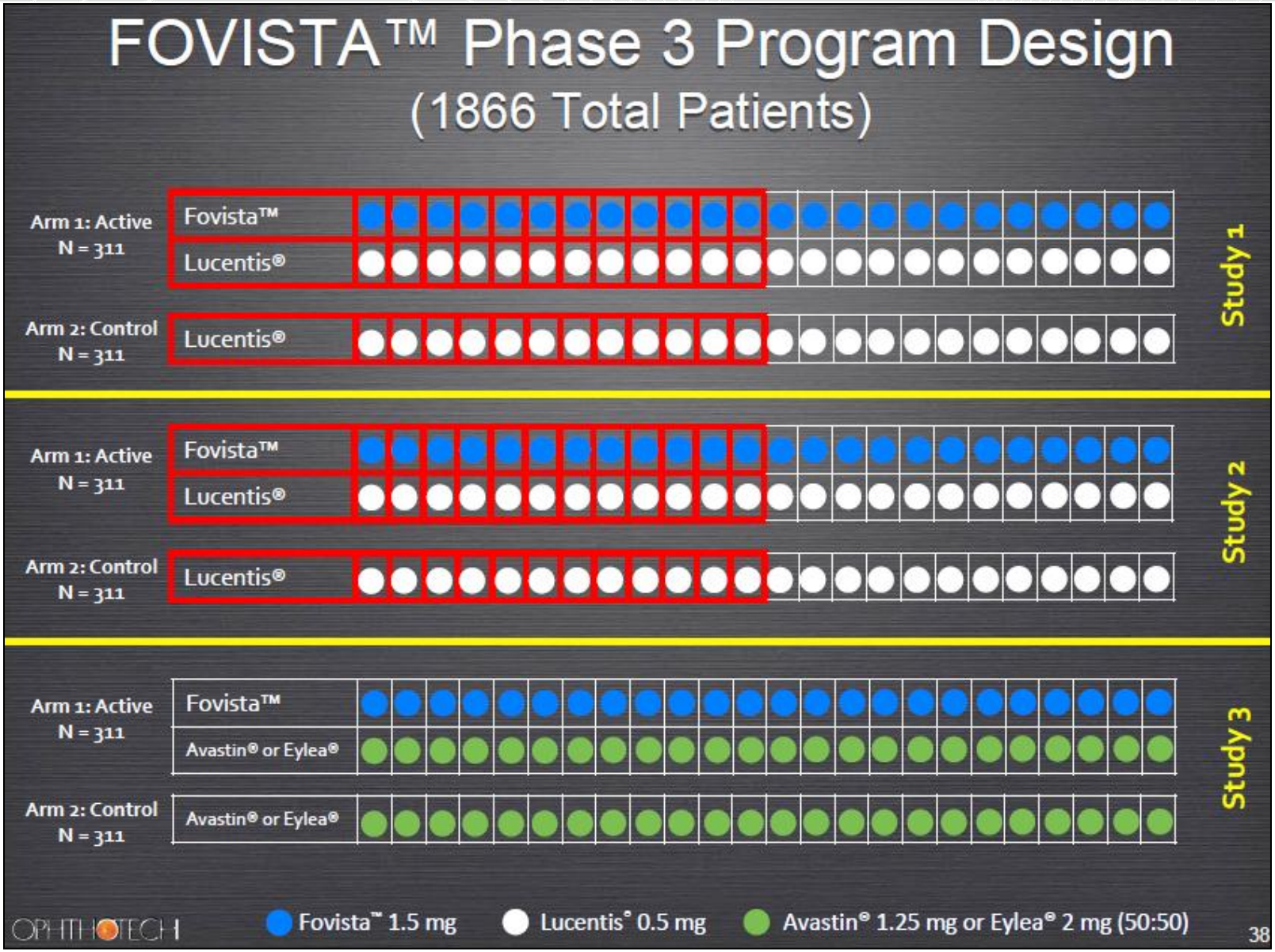
Phase IIb Safety Data Show Little Difference Between Fovista Add-On and Lucentis Monotherapy (cont'd)

LEERINK SWANN

	Monotherapy Lucentis® N = 148	0.3mg Fovista™ + Lucentis® N = 149	1.5mg Fovista™ + Lucentis® N = 152
Patients with ≥ 1 Systemic SAE	**11 (7.4%)**	**13 (8.7%)**	**9 (5.9%)**
MedDRA system organ class*			
Cardiac disorders	2 (1.4%)	2 (1.3%)	2 (1.3%)
Gastrointestinal disorders	1 (0.7%)	2 (1.3%)	3 (2.0%)
Infections	1 (0.7%)	2 (1.3%)	0 (0.0%)
Musculoskeletal disorders	1 (0.7%)	0 (0.0%)	2 (1.3%)
Neoplasms	3 (2.0%)	3 (2.0%)	1 (0.7%)
Nervous system disorders	3 (2.0%)	1 (0.7%)	0 (0.0%)
Respiratory disorders	0 (0.0%)	3 (2.0%)	2 (1.3%)
Any APTC event†	**3 (2.0%)**	**1 (0.7%)**	**0 (0.0%)**
Non-fatal myocardial infarction	0 (0.0%)	0 (0.0%)	0 (0.0%)
Non-fatal stroke	2 (1.4%)	1 (0.7%)	0 (0.0%)
Vascular death	1 (0.7%)	0 (0.0%)	0 (0.0%)

Source: Company Presentations

Fovista Phase III Could Enable a Label With Any anti-VEGF



Source: Company Presentations

Fovista Phase III: Same Exclusion Criteria, Primary Endpoint Evaluated at 48 Weeks

LEERINK SWANN

Phase III: Key Inclusion Criteria

- Subfoveal choroidal neovascularization (CNV) due to AMD with some classic component (i.e., predominantly classic or minimally classic)
- Best corrected visual acuity in the study eye: 20/63 - 20/200
- Total area of the lesion must be ≤ 5 disc areas (DA)
- Non-diabetic

Primary Endpoint is the Mean Change in Visual Acuity from Baseline at Week 48, Measured with the validated ETDRS

Source: Company Presentations

LEERINK SWANN

Fovista Revenue Model and Wet AMD Market Dynamics

Cost Differences Influence Avastin/Lucentis/Eylea Use at Different Centers in Different Types of Patients

LEERINK SWANN

- While Lucentis and Eylea each cost almost $2,000 per treatment, Avastin for wet AMD is made at compounding pharmacies and is thus generally costs around $100 per injection.
- Thus, many Academic centers with a smaller budget and fewer nursing resources available for pursuing reimbursement primarily utilize Avastin, while many private practices primarily treat patients with Lucentis or Eylea
- At Academic centers, many patients are started on Avastin treatment and move to Lucentis or Eylea only if they continue to lose vision or are not dried up
- On the margin, these cost dynamics have led many “healthier” wet AMD patients to primarily be treated with Avastin and sicker wet AMD patients to be treated with Lucentis or Eylea

The Wet AMD Market Has Been Evolving Towards More Eylea Use

LEERINK SWANN

- While none of Avastin, Lucentis, and Eylea has demonstrated superiority to its peers in a clinical study, most MEDACorp KOLs believe that Eylea is the most efficacious treatment for wet AMD, followed by Lucentis and then Avastin, since Eylea has been shown to enable longer intervals between treatment.
- The approval of Eylea has led it to gain ~24% of the anti-VEGF market share in 2013 by our estimate, which we expect to increase to ~36% by 2021.
- We expect additional Eylea use to be driven by both 1) the drug's superior efficacy and 2) the increasing prevalence of the older, wet AMD population, and expect that most patients who begin to plateau or decline on Avastin will be switched to Eylea before Lucentis.

MEDACorp KOL Feedback Has Led us to Project More Fovista Use in Lucentis & Eylea Patients

LEERINK SWANN

- Based on our conversations with specialists, we estimate that ~15% and ~20% of Avastin and Lucentis/Eylea patients are good candidates for Fovista add-on therapy, and penetrate these patient cohorts in our revenue model.
- We believe this may be a conservative assumption since if Fovista demonstrates strong superiority to each of Eylea, Lucentis and Avastin monotherapy arms in Phase III, Fovista may be used frequently in patients that are relatively well-controlled.
- Within the 15% of Avastin-treated and 20% of Lucentis- or Eylea-treated patients that we assume are suited for Fovista add-on therapy, we project US peak market penetrations of 28%/39%/35% Avastin/Lucentis/Eylea in 2027.
- Our probability weighted analysis assumes a 25% probability that Fovista's market share continues to grow until 2027 (patent protection expiry) and a 75% probability that Fovista's share stays flat after 2021 with the entrance of additional competition from REGN who aims to develop a co-formulated anti-VEGF/anti-PDGF antibody fragment product.

US Revenue Model Assumptions

Competitive Scenarios	Probability
1 - PDGF/VEGF Competition Enters 2021	75%
2 - No PDGF/VEGF Competition	25%

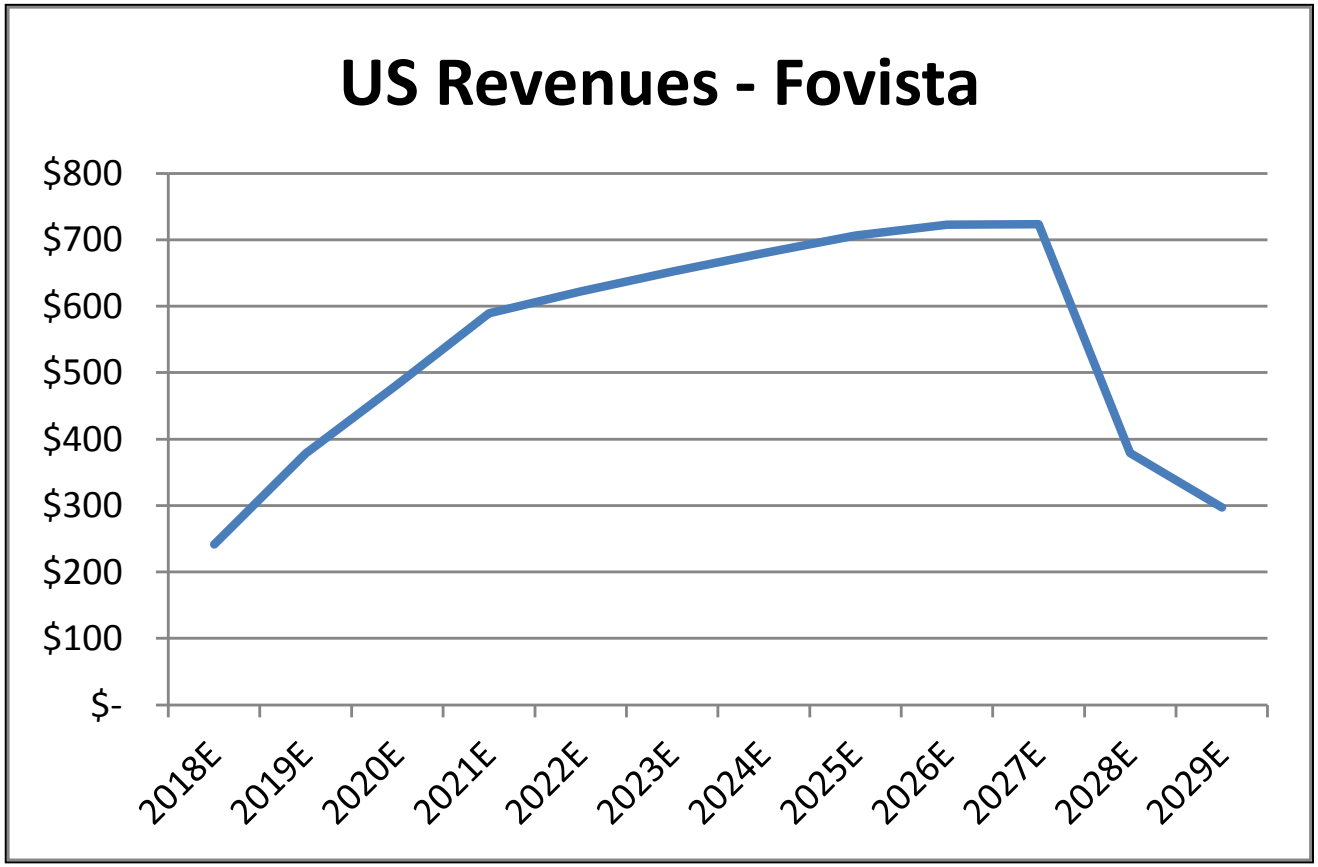
Fovista Assumptions	
Fovista/Lucentis Combo Therapy Approval Probability	80%
Fovista/Eylea Combo Therapy Approval Probability	65%
Fovista/Avastin Combo Therapy Approval Probability	65%
Fovista Cost Per Injection	$1,500

Source:

Similar to other wet AMD treatments, we assume that Fovista is dosed in a "treat-and-extend" manner, with an average of 8.5 injections per patient per year in 2018 that decreases to 6.6 in 2024 and 5.7 in 2027. We assume a slightly higher probability of success for Fovista/Lucentis approval since robust Phase II data has already been generated for this combination.

Source: Leerink Swann Research

Fovista Sales in the US: Rapid Growth, ~$720MM at Peak



Our peak US Fovista sales estimate is based off of p(w) penetrations of 39%/35%/28% in Lucentis/Eylea/Avastin patients and only ~5.2% of the anti-VEGF market overall, assuming that current growth rates in wet AMD patients persist.

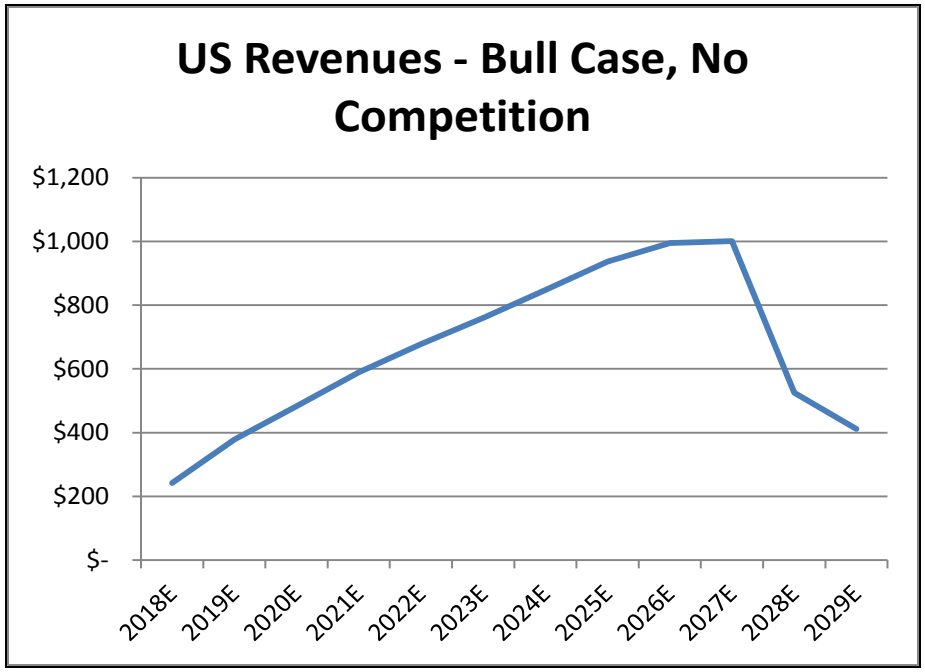
We believe our estimates could be conservative, since new products for wet AMD have historically launched very robustly. Eylea sales are projected to exceed $1B in its 2nd full year on the market.

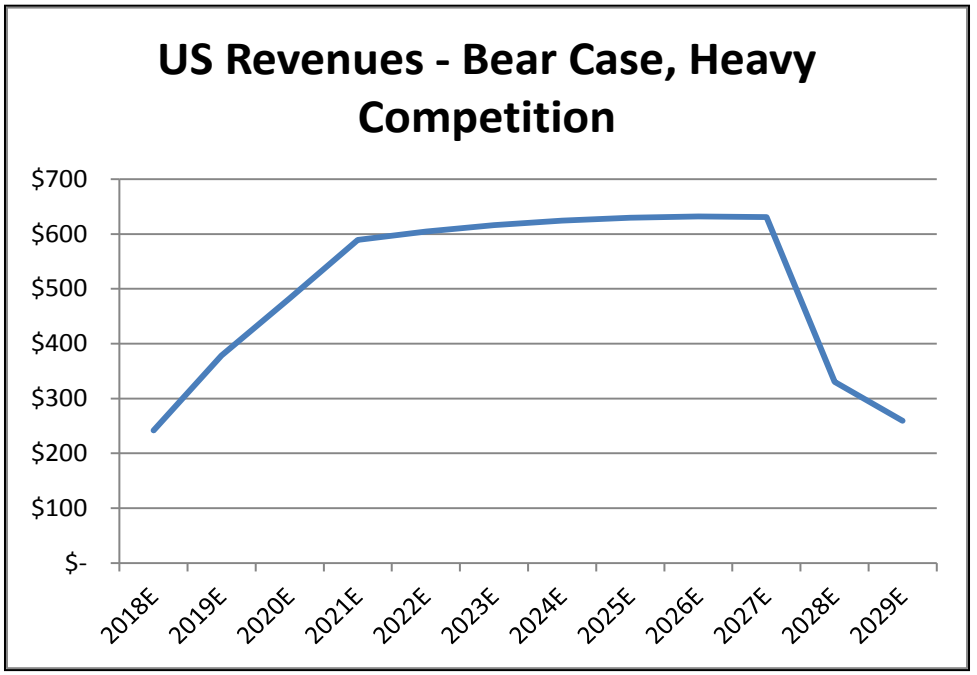
Source: Leerink Swann Research, Graph Revenues in ($MM)

Fovista US Scenario Analysis: Little Additional Competition Presents Significant Upside

LEERINK SWANN

While we assume a 75% probability of the emergence of competition in 2021, in a case where Fovista continues to gain market share until patent expiry in 2021, we project ~$1.1B in peak US sales and estimate an OPHT per share value of ~$44.





Competitive Scenarios	OPHT Valuation
1 - PDGF/VEGF Competition Enters 2021	$34
2 - No PDGF/VEGF Competition	$44

Source: Leerink Swann Research, Graph Revenues in ($MM)

EU/Japan Revenue Model Assumptions

LEERINK SWANN

Competitive Scenarios	
1 - PDGF/VEGF Competition Enters 2021	75%
2 - No PDGF/VEGF Competition	25%

Fovista Assumptions	
Fovista/Lucentis Combo Therapy Approval Probability	80%
Fovista/Eylea Combo Therapy Approval Probability	65%
Fovista/Avastin Combo Therapy Approval Probability	65%
Fovista Cost Per Injection	$1,200
Royalty Rate on Fovista Sales	25%

We assume that OPHT strikes a partnership deal to commercialize Fovista in ex-US markets

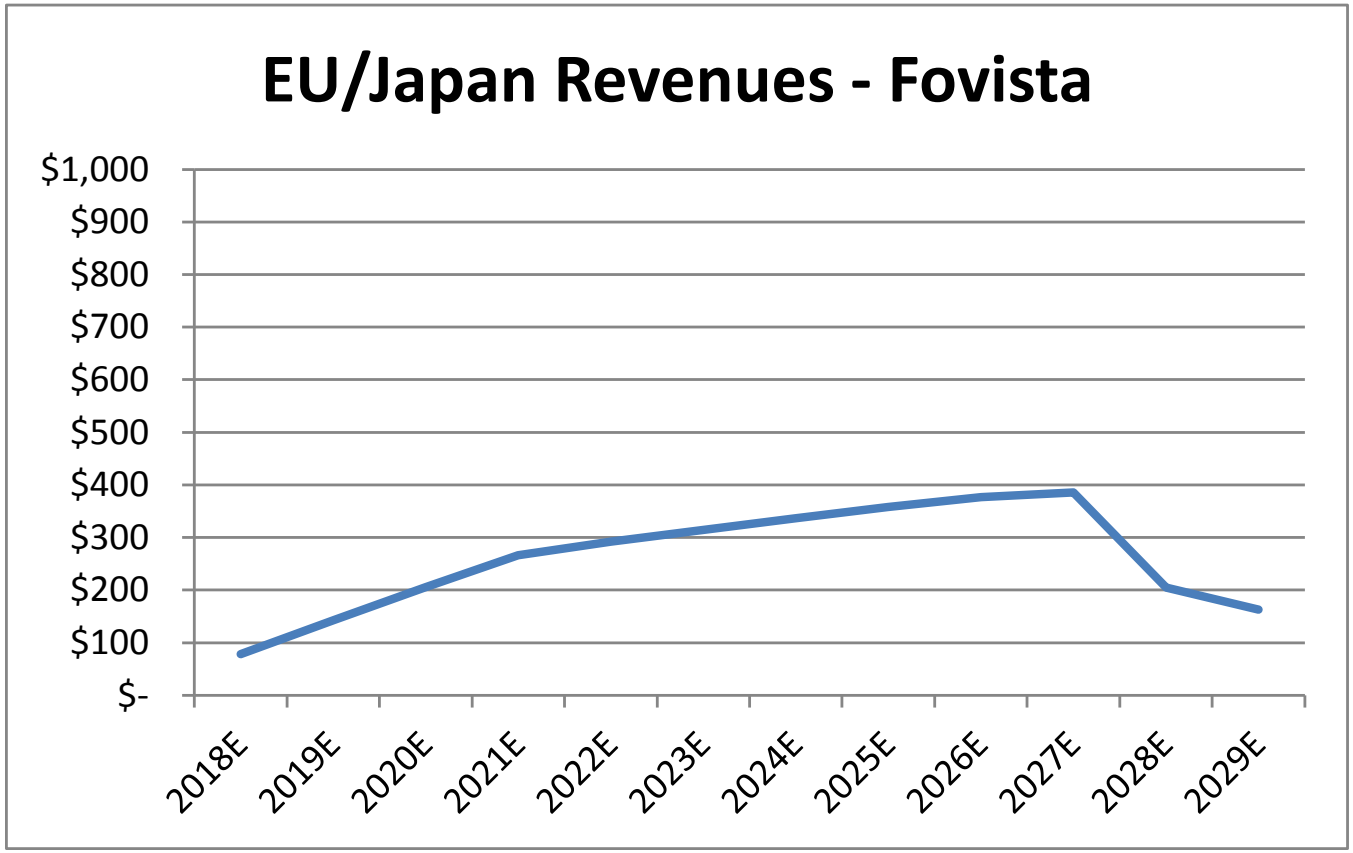
Similar to other wet AMD treatments, we assume that Fovista is dosed in a "treat-and-extend" manner, with an average of 8.5 injections per patient per year in 2018 that decreases to 6.6 in 2024 and 5.7 in 2027.

Source: Leerink Swann Research

Fovista Sales in the EU/Japan: Slower, Linear Growth, ~$400MM at peak

LEERINK SWANN

Our ~$400MM peak probability-weighted sales estimate for Fovista in Europe/Japan implies ~$100MM in peak royalties, assuming a partnership offers a 25% royalty rate. Any milestones that could be offered to OPHT in such a collaboration remain upside to our estimates.



Similar to in the US, our peak Fovista ROW sales estimate is based off of p(w) penetrations of 35%/30%/24% in Lucentis/Eylea/Avastin patients and only ~6% of the anti-VEGF market overall, assuming that current growth rates in wet AMD patients persist.

Source: Leerink Swann Research, Graph Revenues in ($MM)

LEERINK SWANN

Fovista Label Expansion and ARC1905 Present Upside to Our Estimates

Fovista Label Expansion: Wet AMD anti-VEGF Non-responders

LEERINK SWANN

- A subpopulation of wet AMD patients treated with anti-VEGF monotherapy experience some form of visual decline or anti-VEGF resistance
- OPHT believes that an exploratory trial in wet AMD patients that do not respond to the standard of care would require just ~50 patients
- OPHT notes that based on other company clinical trials for Lucentis/Avastin/Eylea, after one year of treatment with an anti-VEGF drug approximately 18-22% of newly diagnosed patients lose additional vision
- **A significant proportion of these patients could be anti-VEGF resistant because of pericyte coverage, and thus could benefit significantly from a combination of Fovista and another anti-VEGF agent**

Fovista Label Expansion: Proliferative Vitreoretinopathy

LEERINK SWANN

- Proliferative Vitreoretinopathy (PVR) is a complication that occurs in 5-10% of cases of retinal detachment. PVR is characterized by various degrees of scarring in the retina. There are 5,000 to 10,000 new cases of PVR in the US each year.
- In its moderate to severe form, PVR may become recurrent with a subsequent poor visual outcome. Surgery is often utilized to treat PVR, but the recurrent form is often untreatable.
- **Local concentrations of PDGF have been shown to be elevated in patients suffering from PVR, and in animal models of PVR Fovista strongly inhibited scarring of the retina.**
- Thus, OPHT believes that Fovista could augment the PVR standard-of-care and may benefit patients if utilized in combination with surgical intervention
- OPHT believes an exploratory trial would only involve up to 25 patients with PVR

Fovista Label Expansion to *Von Hippel-Lindau Disease?*

LEERINK SWANN

- Von Hippel-Lindeau disease (VHL) is an inherited condition characterized by multiple benign and malignant tumors and cysts in the eye and other organs. VHL is rare w/ approximately 5k patients in the US and deficiency of the protein “pVHL” is believed to cause VHL.
- In the eye, tumors consisting of blood cells called retinal capillary hemangiomas are the most common and earliest manifestation of VHL
- These tumors cause significant retinal leakage and may lead to significant vision loss
- While smaller lesions located away from the central regions of the retina can be treated by laser or freezing, other large and poorly situated lesions are untreatable
- Smaller trials with anti-VEGF monotherapy over 6-months have not demonstrated any improvement in patients with retinal capillary hemangiomas, but PDGF levels have been shown to be elevated in cells with a deficiency of pVHL so its possible that treatment with Fovista and an anti-VEGF could provide a benefit. An exploratory trial would involve up to 20 patients
- **OPHT notes that additional Fovista trials could be run simultaneously with the ongoing Phase III program or as subsequent Phase IIIb/IV studies. If initiated in 2014, OPHT expects Fovista label expansion data could be available by YE15**

ARC1905 Could Dampen the Pathological Inflammation Afflicting Wet AMD patients

LEERINK SWANN

- Administered via intravitreal injection, ARC1905 is a chemically synthesized, pegylated aptamer that inhibits complement factor C5 and is being evaluated for wet AMD in combo with anti-VEGF agents
- While other clinical trials of complement inhibitors in dry AMD have not been successful, OPHT believes that (based on results from its own Phase I/II evaluating ARC1905 and Lucentis for dry and wet AMD) that wet AMD patients may stand to benefit from ARC1905 treatment
- Complement C5 is a central component of the complement cascade that is believed to be involved in the development of AMD, as local inflammation and activation of the complement cascade play a role in drusen formation in the eye
- Inhibiting complement C5 prevents the formation of its key terminal fragments C5a and C5b-9, while sparing immunoprotective functions
- **OPHT plans to develop ARC1905 in a sub population of patients with wet AMD who do not respond adequately to anti-VEGF monotherapy and are defined as resistant on the basis of complement mediated inflammation**

ARC1905 Phase I/II Data is Encouraging, We Believe

LEERINK SWANN

- OPHT conducted a 60-patient Phase I/II ascending dose, parallel group open label study of ARC1905 with Lucentis in wet AMD patients
- ARC1905 was well tolerated with no dose limiting toxicity
- In patients who had never been treated with an anti-VEGF, multiple combo doses of ARC1905 and Lucentis (0/3mg/1.0mg/2.0mg) led to improvements of 13.6 letters, 11.7 letters and 15.3 letters respectively on the ETDRS
- OPHT is evaluating a clinical trial of ARC1905 in combo with VEGF drugs for treatment of wet AMD patients who have experienced anti-VEGF treatment failure and are defined as resistant on the basis of complement mediated inflammation
- OPHT believes an additional trial would involve up to 50 patients. In another ARC1905 trial OPHT seeks to include patients with a variant of wet AMD called “polypoidal choroidal vasculopathy” or PCV. There is a high prevalence of PCV in Asia and therapeutic resistance of PCV to anti-VEGF agents is sub-optimal, and OPHT suspects that complement mediated inflammation could play a role in poor outcomes in this subset of patients.

LEERINK SWANN

Financial Model Assumptions

We believe OPHT is Well-Resourced with an Estimated ~$240MM in Cash by YE13

LEERINK SWANN

- After its very successful primary offering, the sale of series C preferred stock and additional warrant issuance, we believe that OPHT is well resourced with cash to support operations into 2016, assuming a drawdown of the 3rd Novo payment in 2014
- We project R&D expense of $16.7MM, $66.0MM and $79.2MM in 2013, 2014 and 2015 as OPHT funds its ongoing pivotal Fovista study.
- For SG&A, we model a spike in to $39.3MM (from $19.4MM in 2016) when OPHT hires a Fovista sales force which is expected to consist of 72 reps including: 50 sales reps, 8 sales managers, 4 training/administration personnel, 4 reimbursement personnel, and 6 med science liaisons
- We assume that OPHT pays a 6% royalty on Fovista sales to Novo Nordisk, who has signed a $125 financing agreement with OPHT that offers 3 $41.7MM tranche payments, 2 of which have been drawn down
- OPHT also owes a low-single digit royalty on Fovista sales to Eyetech (we assume 3%)

We Project Commercialization *and* Cash Flow Breakeven in 2018

LEERINK SWANN

- We estimate Fovista approval in 2017 and model the first Fovista sales in 2018
- As evidenced by Eylea which generated >$750MM in sales in its first year on the market, we believe that the wet AMD market adopts new products very rapidly – we model Fovista 2018-2020 sales of ~$240MM, ~$375MM and $480MM
- Our peak domestic sales estimate of ~$720MM assumes ~120K patients on drug in 2027

Ophthotech P&L ($MM except EPS)	2012	1H13	3Q13E	4Q13E	2013E	1Q14E	2Q14E	3Q14E	4Q14E	2014E	2015E	2016E	2017E	2018E
Fovista Product Sales by OPHT (p/w)	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**241.8**
Royalties on EU Fovista Sales	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**19.7**
Royalties to Novo	-	-	-	-	-	-	-	-	-	-	-	-	-	19.2
Royalties to OSI (eyetech)	-	-	-	-	-	-	-	-	-	-	-	-	-	9.6
COGS	-				-	-	-	-	-	-	-	-	-	29.0
R&D	6.8	6.7	4.0	6.0	16.7	15.0	16.0	17.0	18.0	66.0	79.2	71.3	71.3	64.2
SG&A	6.9	5.0	3.5	3.0	11.5	3.5	4.0	4.0	4.5	16.0	17.6	19.4	39.3	48.4
Operating Expenses	13.7	11.7	7.5	9.0	28.2	18.5	20.0	21.0	22.5	82.0	96.8	90.6	110.6	170.3
Operating Income	**(13.7)**	**(11.7)**	**(7.5)**	**(9.0)**	**(28.2)**	**(18.5)**	**(20.0)**	**(21.0)**	**(22.5)**	**(82.0)**	**(96.8)**	**(90.6)**	**(110.6)**	**91.1**
Interest income, expense (net)	(0.5)	(1.5)	-	-	(1.5)	-	-	-	-	-	-	-	-	-
Other income, expense (net)	(0.3)	(1.5)	-	-	(1.5)	-	-	-	-	-	-	-	-	-
EBT	(14.5)	(14.6)	(7.5)	(9.0)	(31.1)	(18.5)	(20.0)	(21.0)	(22.5)	(82.0)	(96.8)	(90.6)	(110.6)	91.1
Tax Expense (Benefit)	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net Income	**(14.5)**	**(14.6)**	**(7.5)**	**(9.0)**	**(31.1)**	**(18.5)**	**(20.0)**	**(21.0)**	**(22.5)**	**(82.0)**	**(96.8)**	**(90.6)**	**(110.6)**	**91.1**
Diluted EPS	(2.52)	(0.65)	(0.25)	(0.30)	(1.20)	(0.61)	(0.66)	(0.68)	(0.73)	(2.68)	(2.72)	(2.48)	(2.94)	2.16
Basic Shares Outstanding	8.6	22.5	30.1	30.1	27.6	30.3	30.5	30.7	30.9	30.6	35.6	36.6	37.6	38.6
Diluted Shares Outstanding	8.6	22.5	33.6	33.6	29.9	33.8	34.0	34.2	34.4	34.1	39.1	40.1	41.1	42.1

Balance Sheet and Cash Flow Statement ($MM)														
Cash & Cash Equivalents	**4.3**	**39.9**	**261.8**	**253.5**	**253.5**	**236.5**	**218.1**	**198.8**	**219.8**	**219.8**	**131.7**	**174.3**	**73.6**	**174.7**
Debt	**11.0**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**

Change in Cash	**(2.1)**	**28.0**	**222.0**	**(8.3)**	**241.7**	**(17.0)**	**(18.4)**	**(19.3)**	**21.0**	**(33.7)**	**(88.1)**	**42.6**	**(100.7)**	**101.1**
Cash Flow From Operations	**(13.1)**	**(13.7)**	**(6.9)**	**(8.3)**	**(28.9)**	**(17.0)**	**(18.4)**	**(19.3)**	**(20.7)**	**(75.4)**	**(89.1)**	**(83.4)**	**(101.7)**	**100.1**
Net Income	(14.5)	(14.6)	(7.5)	(9.0)	(31.1)	(18.5)	(20.0)	(21.0)	(22.5)	(82.0)	(96.8)	(90.6)	(110.6)	91.1
Stock Option Expense	0.6	0.9	0.6	0.7	2.3	1.5	1.6	1.7	1.8	6.6	7.7	7.3	8.8	9.0
Other	0.8	-	-	-	-	-	-	-	-	-	-	-	-	-
Cash Flow From Investing	**-**	**-**	**-**	**-**	-	**-**	**-**	**-**	**-**	**-**	**1.0**	**1.0**	**1.0**	**1.0**
Sale (Purchase) of Marketable Securities	-	-	-	-	-	-	-	-	-	-	-	-	-	-
CapEx	-	-	-	-	-	-	-	-	-	-	1.0	1.0	1.0	1.0
Other	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Cash Flow From Financing	**11.0**	**41.7**	**228.9**	**-**	**258.5**	**-**	**-**	**-**	**41.7**	**41.7**	**-**	**125.0**		
Equity Issuance (Buyback/Costs)	0.0		**187.2**	-	187.2	-	-	-	-	-	-	125.0		
Debt Issuance (Buyback/Costs)	11.0	-	-	-	(12.0)	-	-	-	-	-	-	-		
Other		41.7	41.7	-	83.4	-	-	-	41.7	41.7		-		

Source: SEC Filings and Leerink Swann Estimates

OPTH DCF Analysis	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	TV
Free Cash Flow to Equity	(28.9)	(75.4)	(89.1)	(83.4)	(101.7)	100.1	199.4	273.7	332.2	265.4	280.1	294.2	307.4	315.7	316.0	142.4	159.7	
Discount Periods	0	0.25	1.25	2.25	3.25	4.25	5.25	6.25	7.25	8.25	9.25	10.25	11.25	12.25	13.25	14.25	15.25	
Discounted FCFE	(7.2)	(73.3)	(77.3)	(64.6)	(70.4)	61.8	110.0	134.8	146.1	104.2	98.2	92.1	85.9	78.8	70.4	28.3	28.4	236.4

NPV	**$ 982.37**
Net Cash YE13	**$ 253.53**
OPHT Per Shr Val	**$ 36.81**
Implied Mkt. Cap	**$ 1,235.9**

WACC	12%
TG	0%
Diluted Shrs. Outstanding	33.57

Source: Leerink Swann Estimates

		Cost of Capital				
		10%	**11%**	**12%**	**13%**	**14%**
Terminal Growth Rate	**-1%**	$ 44.89	$ 40.19	$ 36.21	$ 32.80	$ 29.85
	0%	$ 46.00	$ 41.00	**$ 36.81**	$ 33.26	$ 30.20
	1%	$ 47.36	$ 41.98	$ 37.53	$ 33.79	$ 30.60
	3%	$ 51.24	$ 44.67	$ 39.44	$ 35.18	$ 31.63
	5%	$ 58.23	$ 49.15	$ 42.45	$ 37.26	$ 33.12

Wet AMD Revenue Model - US	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Total Wet AMD Patients	1,875,000	2,075,000	2,275,000	2,475,000	2,675,000	2,875,000	3,075,000	3,275,000
New Wet AMD Patients	200,000	200,000	200,000	200,000	200,000	200,000	200,000	200,000
VEGF Therapy Penetration New Ptnt Penetration	49%	50%	50%	50%	50%	50%	50%	50%
New VEGF Patients	98,000.00	100,000	100,000	100,000	100,000	100,000	100,000	100,000
Total Wet AMD Patients on VEGF therapy	670,442	770,442	870,442	970,442	1,070,442	1,170,442	1,270,442	1,370,442
Lucentis Wet AMD Treaters Penetration	30%	28%	26%	24%	23%	22%	21%	20%
Patients on Lucentis	197,780	215,724	226,315	232,906	246,202	257,497	266,793	274,088
% not well controlled (suited for Fovista add-on)	-	-	-	-	20%	20%	20%	20%
# Fovista Candidates	-	-	-	-	49,240	51,499	53,359	54,818
% add-on Fovista	0%	0%	0%	0%	0%	15%	22%	28%
# Patients on Lucentis + Fovista	-	-	-	-	-	7,725	11,739	15,349
Fovista Cost Per Injection	-	-	-	-	$1,500	$1,500	$1,500	$1,500
Fovista Injections Per Year	-	-	-	-	0.0	8.5	8.2	7.8
Revenue to OPHT (Non risk adjusted)	-	-	-	-	$0.0	$98.5	$144.4	$179.6
Approval Probability	-	-	-	-	80%	80%	80%	80%
Probability Weighted Revenue to OPTH	-	-	-	-	**$0.0**	**$78.8**	**$115.5**	**$143.7**
Eylea Wet AMD Treaters Penetration	24%	26%	28%	31%	33%	34%	35%	36%
Patients on Eylea	157,554	200,315	243,724	300,837	353,246	397,950	444,655	493,359
% not well controlled (suited for Fovista add-on)	-	-	-	-	20%	20%	20%	20%
# Fovista Candidates	-	-	-	-	70,649	79,590	88,931	98,672
% add-on Fovista	0%	0%	0%	0%	0%	15%	20%	25%
# Patients on Eylea + Fovista	-	-	-	-	-	11,939	17,786	24,668
Fovista Cost Per Injection	-	-	-	-	$1,500	$1,500	$1,500	$1,500
Fovista Injections Per Year	-	-	-	-	0.0	8.5	8.2	7.8
Revenue to OPHT (Non risk adjusted)	-	-	-	-	0	152.2	218.8	288.6
Approval Probability	-	-	-	-	65%	65%	65%	65%
Probability Weighted Revenue to OPTH	-	-	-	-	**0**	**98.9**	**142.2**	**187.6**
Avastin Wet AMD Treaters Penetration	47%	46%	46%	45%	44%	44%	44%	44%
Patients on Avastin	315,108	354,403	400,403	436,699	470,994	514,994	558,994	602,994
% not well controlled (suited for Fovista add-on)	-	-	-	-	15%	15%	15%	15%
# Fovista Candidates	-	-	-	-	70,649	77,249	83,849	90,449
% add-on Fovista	0%	0%	0%	0%	0%	10%	18%	22%
# Patients on Avastin + Fovista	-	-	-	-	-	7,725	15,093	19,899
Fovista Cost Per Injection	-	-	-	-	$1,500	$1,500	$1,500	$1,500
Fovista Injections Per Year	-	-	-	-	0.0	8.5	8.2	7.8
Revenue to OPHT (Non risk adjusted)	-	-	-	-	0	98.5	185.6	232.8
Approval Probability	-	-	-	-	65%	65%	65%	65%
Probability Weighted Revenue to OPTH	-	-	-	-	**0**	**64.0**	**120.7**	**151.3**
Fovista Patients on Drug	-	-	-	-	-	27,388	44,618	59,916
Fovista Overall Penetration of US VEGF-treated Patients	-	-	-	-	-	2.3%	3.5%	4.4%
Gross Fovista Revenues	-	-	-	-	-	349	549	701
Risk-Adjusted Fovista Sales to P&L	**-**	**-**	**-**	**-**	**-**	**242**	**378**	**483**

Competitive Scenarios	**Probability**
1 - PDGF/VEGF Competition Enters 2021	75%
2 - No PDGF/VEGF Competition	25%

Fovista Assumptions	
Fovista/Lucentis Combo Therapy Approval Probability	80%
Fovista/Eylea Combo Therapy Approval Probability	65%
Fovista/Avastin Combo Therapy Approval Probability	65%
Fovista Cost Per Injection	$1,500

Fovista Injections Per Year					0	8.5	8.2	7.8

Source: SEC Filings, Company Reports and Leerink Swann Estimates

Wet AMD Revenue Model - EU and Japan	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Total EU/Japan Wet AMD Patients (MM)	2,000,000	2,200,000	2,400,000	2,600,000	2,800,000	3,000,000	3,200,000	3,400,000
New Wet AMD Patients	200,000	200,000	200,000	200,000	200,000	200,000	200,000	200,000
VEGF Therapy Penetration New Ptnt Penetration		20%	22%	26%	30%	35%	40%	40%
New VEGF Patients		40,000	44,000	52,000	60,000	70,000	80,000	80,000
Total Wet AMD Patients on VEGF therapy	285,249	325,249	369,249	421,249	481,249	551,249	631,249	711,249
Lucentis Wet AMD Treaters Penetration	63%	60%	58%	54%	50%	48%	46%	44%
Patients on Lucentis	180,000	195,149	214,164	227,474	240,625	264,600	290,375	312,950
% not well controlled (suited for Fovista add-on)		-	-	-	20%	20%	20%	20%
# Fovista Candidates	-	-	-	-	48,125	52,920	58,075	62,590
% add-on Fovista	0%	0%	0%	0%	0%	10%	18%	25%
# Patients on Lucentis + Fovista	-	-	-	-	-	5,292	10,453	15,647
Fovista Cost Per Injection	-	-	-	-	$1,200	$1,200	$1,200	$1,200
Fovista Injections Per Year	-	-	-	-	0	8.5	8.2	7.8
Revenue to Partner (Non risk adjusted)	-	-	-	-	0	54.0	102.9	146.5
Approval Probability	-	-	-	-	80%	80%	80%	80%
Probability Weighted Revenue to EU Partner	-	-	-	-	**0**	**43.2**	**82.3**	**117.2**
Eylea Wet AMD Treaters Penetration	13%	18%	22%	28%	34%	38%	41%	43%
Patients on Eylea	37,710	58,545	81,235	117,950	163,625	209,475	255,656	305,837
% not well controlled (suited for Fovista add-on)	-	-	-	-	20%	20%	20%	20%
# Fovista Candidates	-	-	-	-	32,725	41,895	51,131	61,167
% add-on Fovista	0%	0%	0%	0%	0%	10%	15%	20%
# Patients on Eylea + Fovista	-	-	-	-	-	4,189	7,670	12,233
Fovista Cost Per Injection	-	-	-	-	$1,200	$1,200	$1,200	$1,200
Fovista Injections Per Year	-	-	-	-	0	8.5	8.2	7.8
Revenue to Partner (Non risk adjusted)	-	-	-	-	0	42.7	75.5	114.5
Approval Probability	-	-	-	-	65%	65%	65%	65%
Probability Weighted Revenue to EU Partner	-	-	-	-	**0**	**27.8**	**49.1**	**74.4**
Avastin Wet AMD Treaters Penetration	24%	22%	20%	18%	16%	14%	14%	13%
Patients on Avastin	68,460	71,555	73,850	75,825	77,000	77,175	85,219	92,462
% not well controlled (suited for Fovista add-on)	-	-	-	-	15%	15%	15%	15%
# Fovista Candidates	-	-	-	-	11,550	11,576	12,783	13,869
% add-on Fovista	0%	0%	0%	0%	0%	10%	13%	17%
# Patients on Avastin + Fovista	-	-	-	-	-	1,158	1,662	2,358
Fovista Cost Per Injection	-	-	-	-	$1,200	$1,200	$1,200	$1,200
Fovista Injections Per Year	-	-	-	-	0	8.5	8.2	7.8
Revenue to Partner (Non risk adjusted)	-	-	-	-	0	11.8	16.4	22.1
Approval Probability	-	-	-	-	65%	65%	65%	65%
Probability Weighted Revenue to EU Partner	-	-	-	-	**0.0**	**7.7**	**10.6**	**14.3**
Fovista Overall Penetration of EU VEGF-treated Patients	0%	0%	0%	0%	0%	2%	3%	4%
Gross Fovista Revenues to Partner	-	-	-	-	-	109	195	283
Risk-Adjusted Fovista Revenues to Partner	-	-	-	-	-	79	142	206
Royalty Rate on Fovista Sales	*25%*	25%	25%	25%	25%	25%	25%	25%
Risk-Adjusted Fovista Royalties to P&L	**-**	**-**	**-**	**-**	**-**	**20**	**35**	**51**

Competitive Scenarios	
1 - PDGF/VEGF Competition Enters 2021	75%
2 - No PDGF/VEGF Competition	25%

Fovista Assumptions	
Fovista/Lucentis Combo Therapy Approval Probability	80%
Fovista/Eylea Combo Therapy Approval Probability	65%
Fovista/Avastin Combo Therapy Approval Probability	65%
Fovista Cost Per Injection	$1,200
Royalty Rate on Fovista Sales	25%

Fovista Injections Per Year					0	8.5	8.2	7.8

Source: SEC Filings, Company Reports and Leerink Swann Estimates

OPHT Milestones		
Product	**Event**	**Timing**
Fovista	Phase III Initiation	2H13
Fovista	Additional Indication Trials	2014
Fovista	Additional Indication Data	2015
Fovista	Pivotal Phase III Data	Mid-2016
Fovista	NDA/MAA Filings	2H16
Fovista	FDA/EMA Approval	2H17

Source: SEC Filings and Leerink Swann Estimates

Disclosures Appendix

Analyst Certification

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

Distribution of Ratings/Investment Banking Services (IB) as of 09/30/13				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	111	64.90	27	24.00
HOLD [MP]	60	35.10	0	0.00
SELL [UP]	0	0.00	0	0.00

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Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

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For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than $2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over $2 billion.

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