OHR Pharmaceutical Inc.

Aegis Healthcare Conference, Las Vegas, NV

September 28, 2013

Safe Harbor Statement

This presentation contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. These forward looking statements are made only as the date thereof, and Ohr undertakes no obligation to update or revise the forward looking statement whether as a result of new information, future events or otherwise. Our actual results may differ materially and adversely from those expressed in any forward-looking statements as a result of various factors and uncertainties, including the future success of our scientific studies, our ability to successfully develop products, rapid technological change in our markets, changes in demand for our future products, legislative, regulatory and competitive developments, the financial resources available to us, and general economic conditions. For example, there can be no assurance that Ohr will be able to sustain operations for expected periods. Shareholders and prospective investors are cautioned that no assurance of the efficacy of pharmaceutical products can be claimed or assured until final testing; and no assurance or warranty can be made that the FDA will approve final testing or marketing of any pharmaceutical product. Ohr's most recent Annual Report and subsequent Quarterly Reports discuss some of the important risk factors that may affect our business, results of operations and financial condition. We disclaim any intent to revise or update publicly any forward-looking statements for any reason.

Company Overview

- Founded in late 2008, public (NasdaqCM: OHRP)
- Executed on strategy to acquire two late stage clinical programs in 2009 that address large unmet medical needs: *wet-AMD & cancer cachexia*
  - *Wealth of preclinical and clinical data*
  - *Clear competitive path forward*
  - *Risk mitigation*
- Experienced management team headquartered in New York, NY
- Strong intellectual property protection
- Tight expense controls
- Unknown story with several upcoming catalysts

Management Team

- Dr. Irach Taraporewala, CEO
  - Over 30 years experience in drug development and regulatory affairs
  - Former Vice President, Regulatory Affairs & Clinical Research, Mystic Pharmaceuticals
  - Former Senior Consultant at PAREXEL Drug Development Consulting, advising pharmaceutical and biotechnology company clients on regulatory strategy and product manufacturing
  - Well versed in pharmaceutical technology evaluation due diligence and intellectual property matters
- Sam Backenroth, VP of Business Development, CFO
  - Former Investment Banker with The Benchmark Co.
  - Completed numerous biotech transactions for micro-cap biotechnology companies
  - Strategic advisor to multiple micro-cap public and private biotechnology companies
- Dr. Shalom Hirschman, Chief Scientific Advisor
  - 30+ years as Director of Infectious Diseases and Vice Chairman of Mount Sinai School of Medicine
  - Former CEO & President of Advanced Viral Corp.
  - Founder and Board member of Xtramedics (Quantrx)
  - Founder of Touro College

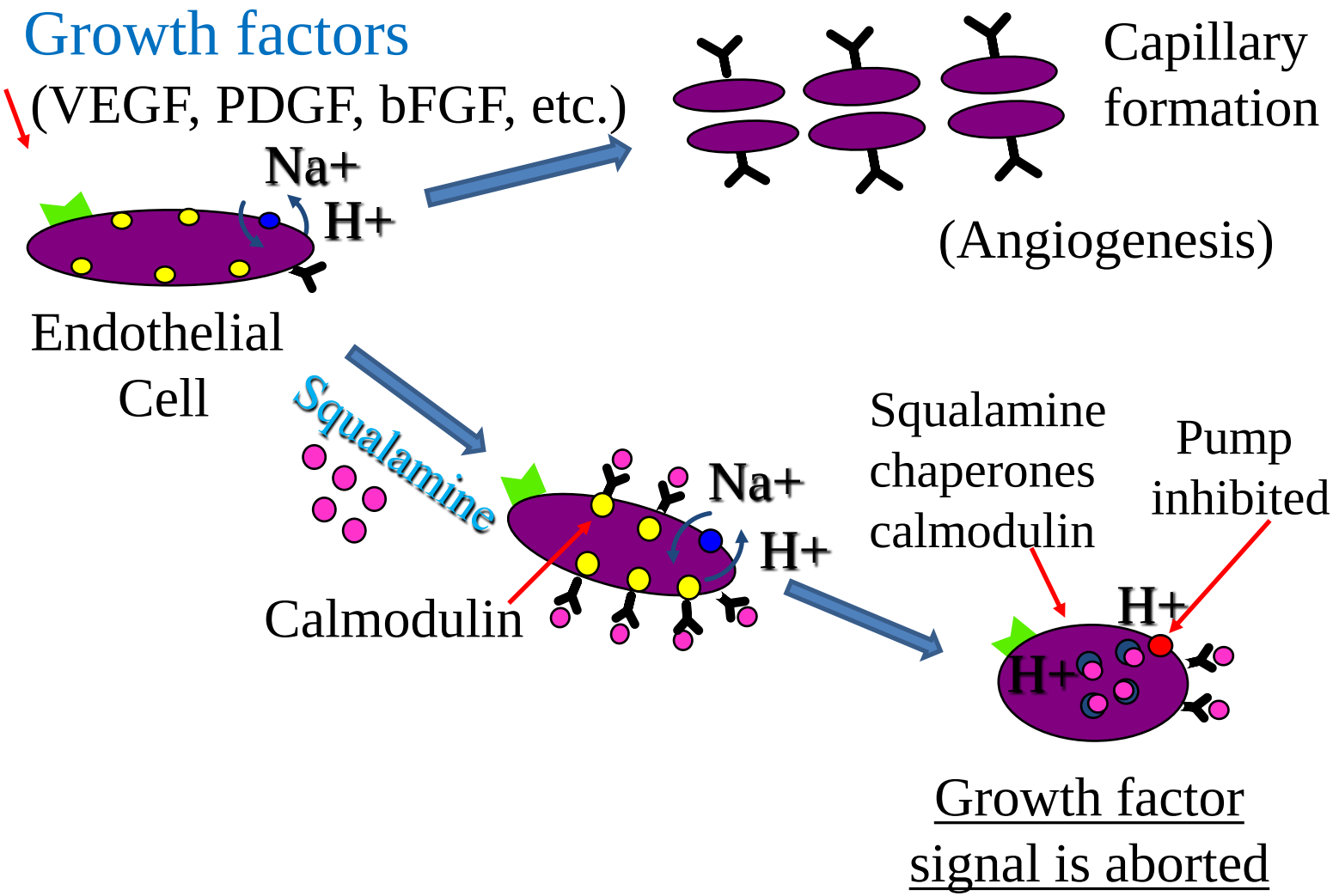
Drug Pipeline

	Preclinical	Phase 1	Phase 2	Phase 3
SQUALAMINE				
Eye-drop formulation WET AGE RELATED MACULAR DEGENERATION	FDA Fast Track →			
IV formulation RESISTANT OVARIAN CANCER	Orphan Drug Designation →			
OHR / AVR 118				
CANCER CACHEXIA	→			
TRODUSQUEMINE				
UNDISCLOSED INDICATION	→			

Squalamine

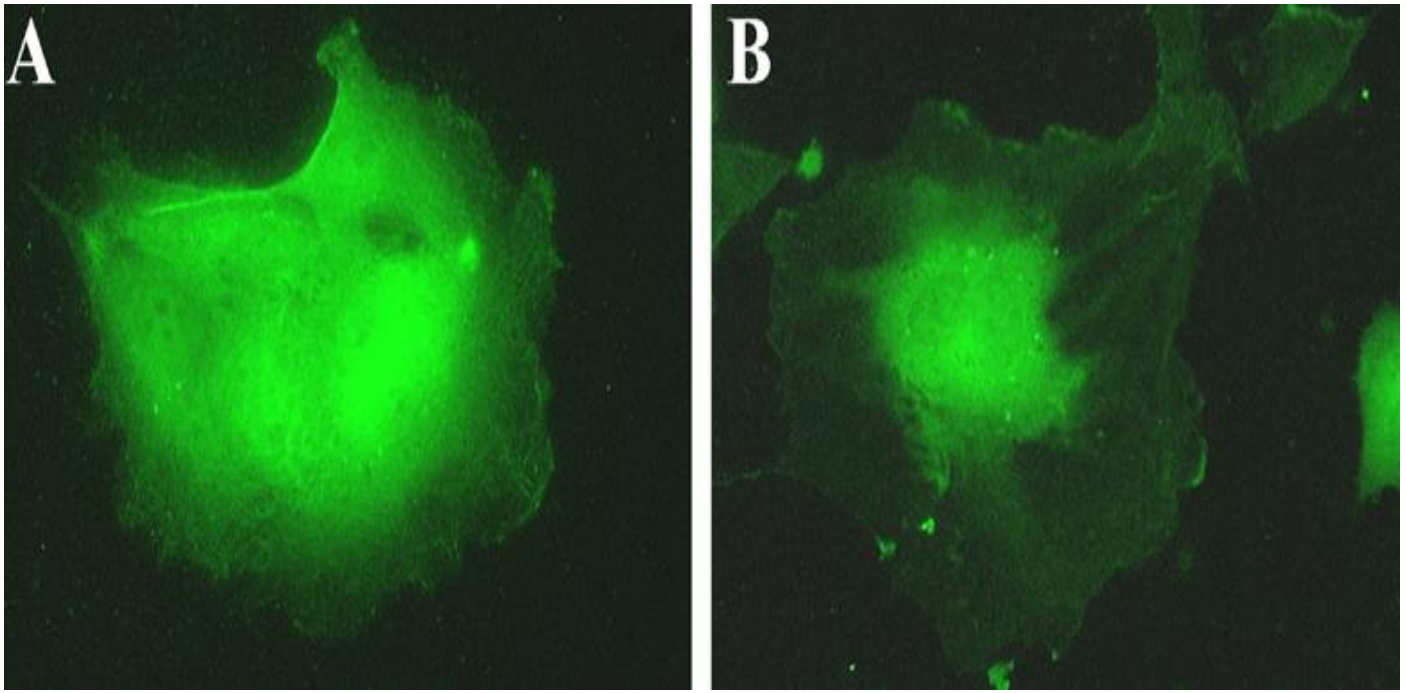
- First-in-class small molecule anti-angiogenic drug with a novel intracellular mechanism of action.
- Inhibitor of multiple angiogenic growth factors
  - VEGF, PDGF, and bFGF
- Ohr Pharmaceutical has developed a **proprietary eye drop formulation** using FDA approved excipients
  - Biodistribution studies show ability of the drug to reach the back of the eye at concentrations that can inhibit neovascularization.
  - Practical delivery method that is superior to IV administration. More convenient and less painful than intravitreal injections.
  - Favorable safety profile
- Development pathway
  - Eye drops for Wet-AMD and neovascular eye diseases
  - Granted Fast Track Designation by US FDA for Wet-AMD
  - Phase 2 in Wet-AMD ongoing (n= 120), interim data expected 2Q-2014

Anti-Angiogenic Mechanism



Squalamine Chaperones Calmodulin

After Entry into Activated Endothelial Cells, Squalamine and Calmodulin Bind, and the Complex is Transported to a Perinuclear Membrane Compartment



Control | Squalamine treated

FITC (green fluorescence) labeled anti-calmodulin antibody

Squalamine Ophthalmic Snapshot

- Phase II development proceeding with eye drop administration
- Previously studied in over 450 patients using an intravenous formulation
  - ~250 patients with Wet-AMD
  - ~200 oncology patients (solid tumors, ovarian, lung, and prostate cancers)
- Intravenous clinical data in Wet-AMD
  - Demonstrated biological effect
  - Gains in visual acuity
  - Strong maintenance of vision
  - Effect in advanced, low vision wet-AMD (“fellow eye”)
- IV formulation entered phase III trials for wet-AMD under fast track status and a Special Protocol Assessment (US FDA)
  - Discontinued due to enrollment difficulty of chronic IV infusion and suboptimal dosing/pharmacokinetics of systemic administration

Eye Drop Solves IV Drawbacks

IV Drawbacks

- Suboptimal dosing- Pharmacokinetic analysis confirms that prior IV dosing was suboptimal especially when going from a weekly to monthly "maintenance" dosing period
- Patient compliance- 40 minute weekly infusion very burdensome on elderly patient population
- Commercial challenges- ophthalmologist offices not equipped to give large scale prolonged infusions
- Infusion site reactions- Due to rapid infusion rates

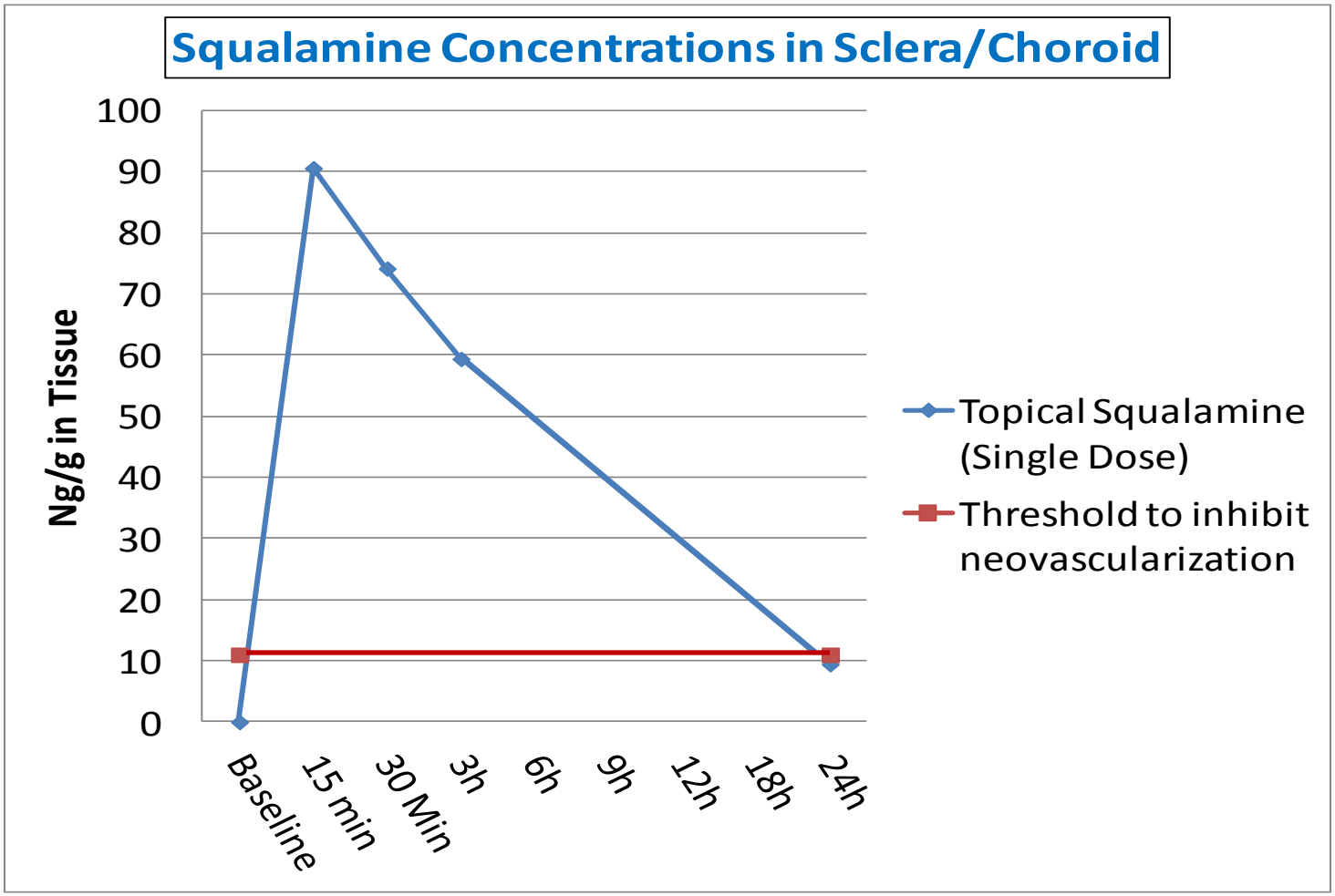
Eye Drop Advantages

- Sustained Therapeutic Levels- *In vivo* studies confirm tissue concentrations well in excess of the antiangiogenic level and can consistently stay above threshold levels
- Self administered eye drop
- No Ophthalmologist infrastructure build out to accommodate large scale IV infusions
- Negligible systemic uptake and topical dosing is orders of magnitude lower than previous IV MTD

Squalamine Eye Drop Formulation

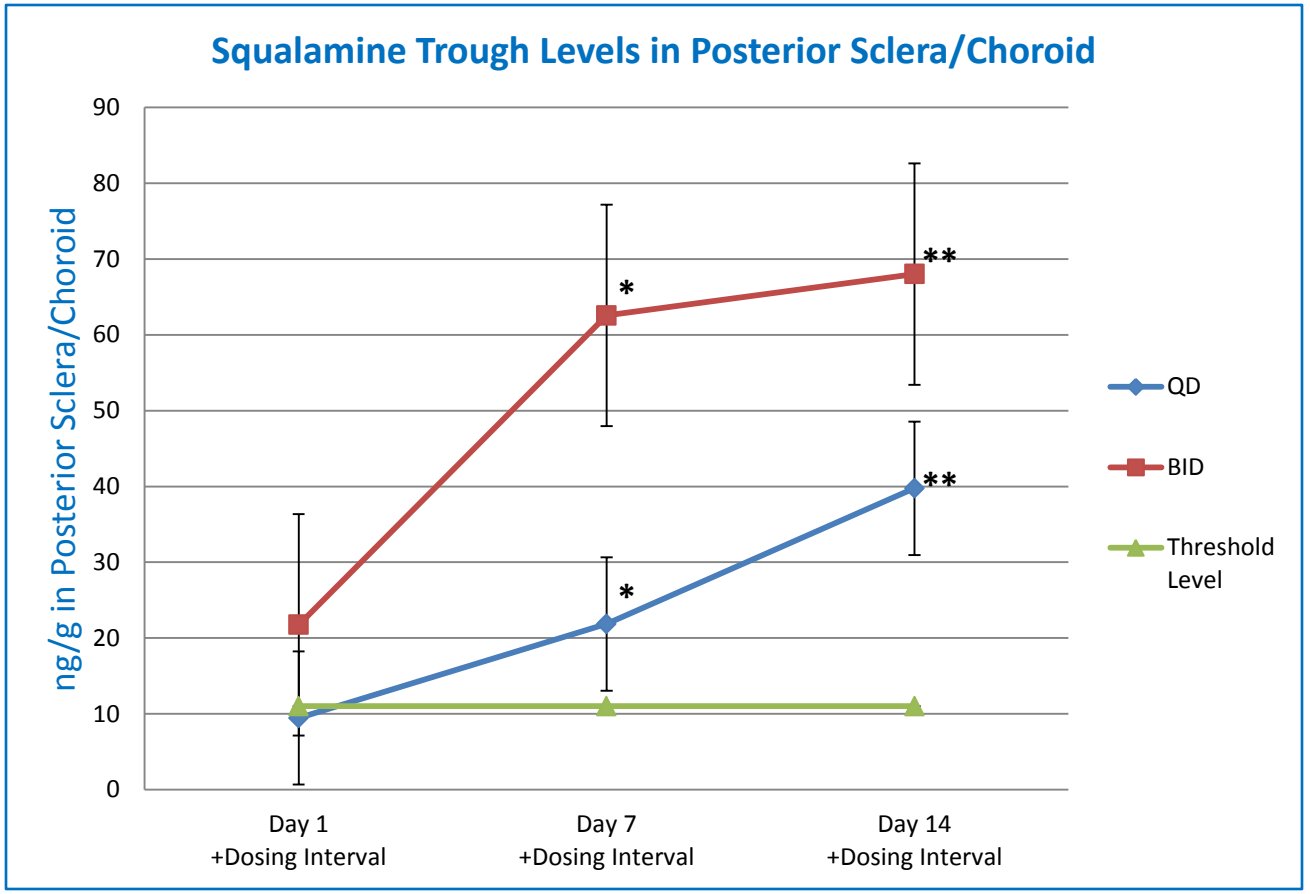
- Proprietary reformulation using FDA approved excipients
- *In-vivo* studies in Dutch belted rabbits
  - 28 day ocular tolerance and toxicity
    - Demonstrated safety and tolerability to ocular tissues
    - No macroscopic or histopathology changes
  - Biodistribution study- single dose
    - Peak concentrations 8x the threshold level to inhibit choroidal neovascularization
  - Biodistribution study- QD & BID up to 14 days
    - Results presented at ARVO & Macula Society- 2012
  - 6 month BID ocular tolerance and toxicity
    - No adverse findings

Eye Drop Single Administration



Threshold level refers to tissue concentrations above which Squalamine is known to inhibit neovascularization

Eye Drop
Multi Dose Trough Levels



Dosing Interval= QD 24 Hours, BID 12 Hours
*=p-value <.01 **=p-value <.001 (values vs day 1+Dosing Interval)

Trough levels represent lowest tissue concentrations prior to next dosing (QD 24h, BID 12h)

Presented at ARVO and Macula Society 2012. Full poster can be found at http://ohrpharmaceutical.com/ARVO%20poster%20FINAL.pdf

Biodistribution Studies Conclusions

- Studies Demonstrated:
  - Rapid uptake to the posterior sclera/choroid ocular tissues with slow tissue clearance
  - Sustained Squalamine concentrations well above threshold anti-angiogenic levels, which persist throughout the period in between doses
  - Safety to ocular tissues with no signs of ocular adverse clinical findings
  - Negligible systemic uptake which minimizes the potential for systemic adverse events

Risk Mitigation

IV Clinical Data Demonstrated Activity

- Visual Acuity Gains
- Maintenance of VA
- Biological Effect

….Even while being dosed suboptimally….

- Rapid systemic clearance
- Lack of sustained concentrations

Data demonstrates the eye drop achieves

- Sustained inhibitory concentrations
- Efficient delivery to back of the eye tissues

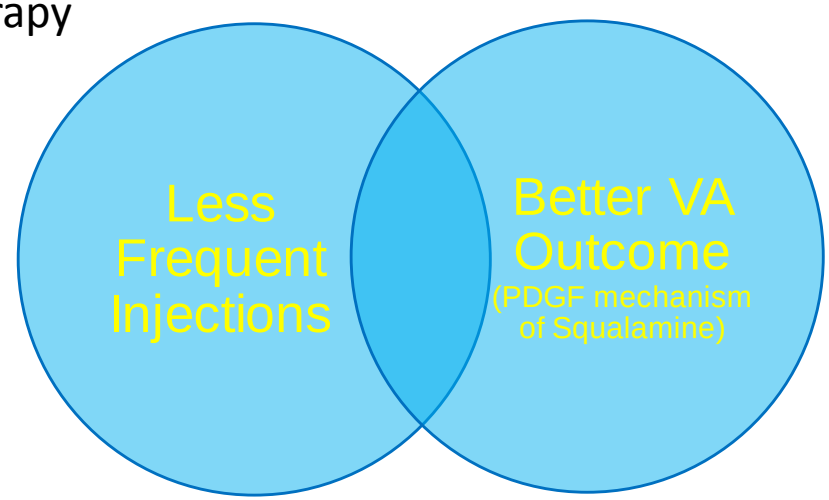
Competitive Advantages

Potential advantages over intravitreal injections ("IVT") for Wet-AMD

- ***Superior delivery method***
  - Current approved therapies are delivered via intravitreal injection directly into the eye every four to eight weeks.
- ***Inhibition of Multiple Angiogenic Growth Factors***
  - Clinical evidence has shown that inhibiting VEGF ***and*** PDGF provides improvement over Lucentis VA gain response rates.
- ***Activity in advanced AMD cases***
  - Many Wet-AMD patients have a more advanced, low vision wet AMD eye ("fellow eye"). Squalamine clinical data has shown significant VA improvement in these fellow eyes using the IV formulation.
- ***Safety profile***
  - Squalamine had minimal systemic or ocular drug-related adverse events when tested using the IV formulation at much higher doses.
- ***Cost effective manufacture***

Topical Path Forward

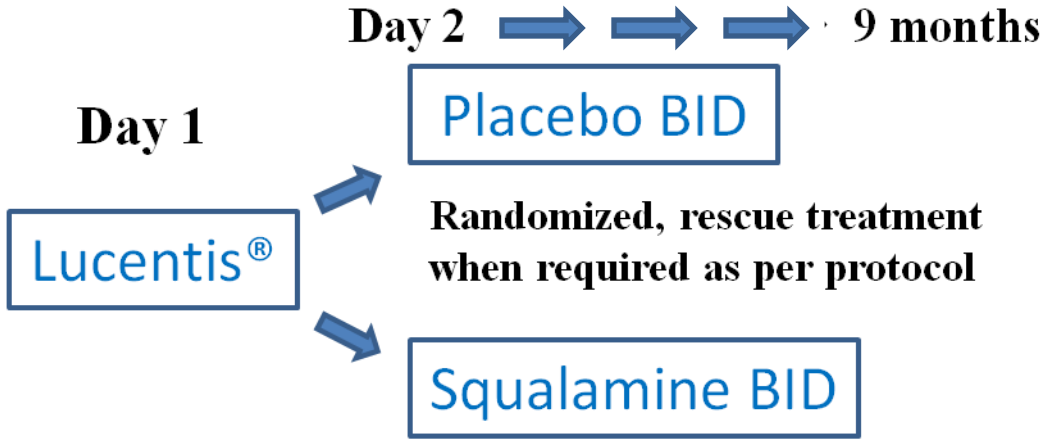
- Phase II trial designed by KOL's in the wet-AMD space
- Trial focuses on newly diagnosed wet-AMD patients
  - Randomized, double masked, placebo controlled study (n=120) at 20+ US Sites
- Trial design includes anti-VEGF treatment (Lucentis) as needed
  - Helps facilitate enrollment while providing clear indication of efficacy
- Design provides for multiple outcome scenarios to guide the path forward in future registration studies
  - Monotherapy
  - Adjunct therapy



- **Clinical Phase II trial began enrolling in late 2012 for wet-AMD**

Phase II Trial Design

- Newly diagnosed wet-AMD patients
- Duration: 9 month treatment period with interim analysis (50% completed)



- Rescue criteria based on objective parameters
- Efficacy Endpoints (10 endpoint hierarchical analysis)
  - 1°: Mean number of Lucentis injections
  - 2°: Mean time to Lucentis retreatment
  - 2°: VA gains, maintenance, and safety
- Primary endpoint is powered (90%) to detect a 1.5 injection difference between the arms
- 60 patients per arm (120 total)
- Interim Data anticipated in Q2 2014

Ophthalmic Advisory Board

Key Opinion Leaders (KOL) in retinal disorders

- David Boyer MD
  Retina-Vitreous Associates Medical Group (Los Angeles, CA)
- Thomas Ciulla MD
  Midwest Eye Institute (Indianapolis, IN)
- Michael Elman MD
  Elman Retina Group (Baltimore, MD)
- Jeffrey Heier MD
  Ophthalmic Consultants of Boston (Boston, MA)
- Daniel Roth MD
  Retina Vitreous Center (New Brunswick, NJ)
- Lawrence Singerman MD
  Retina Associates of Cleveland (Cleveland, OH)
- Jason Slakter MD
  Vitreous Retina Macula Consultants of NY (New York, NY)
- John Wroblewski MD
  Cumberland Valley Retina Consultants (Hagerstown, MD)

Competitive Landscape

	Squalamine	Lucentis®	Eylea®	Fovista®	Pazopanib	DARPin's
Developer	Ohr Pharmaceutical	Genentech/ Roche	Regeneron	Ophthotech	Glaxo	Molecular Partners
Mechanism	Intracellular	Extracellular	Extracellular	Extracellular	Intracellular	Extracellular
Target	VEGF, PDGF, bFGF	VEGF	VEGF	PDGF	Tyrosine Kinases	VEGF, VEGF &PDGF
Delivery	Eye Drops	Intravitreal	Intravitreal	Intravitreal	Eye Drops	Intravitreal
Dev. Stage	Phase II	FDA Approved	FDA Approved	Entering Phase III	Phase II	Phase II & Preclinical
Molecule size	Small Molecule	Large molecule	Large molecule	Large molecule	Small molecule	Large Molecule
Cost/Dose	_	$2,000	$1,850	_	_	_
Revenue/ Partnership	_	**$4b** ('12 global)	**$838mm** ('12 U.S.)	_	_	**$1.4b deal with Allergan**

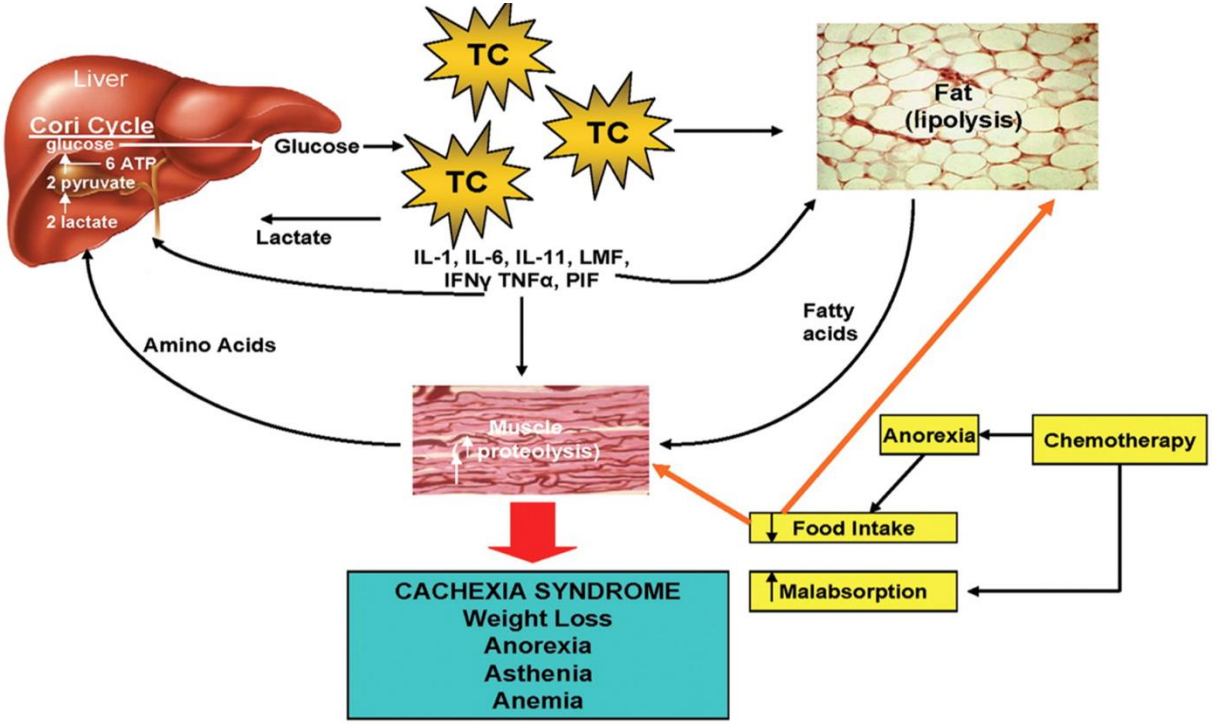
Squalamine Markets

Initial Indication	Future Indications	
Wet Macular Degeneration	Retinopathy & Macular Edema	Dry AMD Prophylaxis
1,750,000 Patients (U.S.)	1,200,000 Patients (U.S.)	13,000,000 Patients (U.S.)
200,000 New Cases (Annual, U.S.)	130,000 New Cases (Annual, U.S.)	300-700K New Cases (Annual, U.S.)

Current Market Leader:
Intravitreal Lucentis®
(~$4b Annual Revenue(WW)

OHR/AVR118 in Cancer Cachexia

- Broad spectrum, peptide immuno-modulator
- Modulates immune response by regulating multiple cytokines and chemokines involved in the disease process



From Loberg, R. D. et al.
CA Cancer J Clin 2007;57:225-241.

Cancer Cachexia Indication

- Wasting disorder often seen in late stage cancer patients characterized by anorexia, loss of muscle mass, fatigue, weakness, and poor quality of life
- Cachexia is exacerbated by the cellular stress of chemotherapy and radiation
- Research has shown that the etiology of cancer cachexia is likely attributable to a cascade of pro-inflammatory cytokine release (cytokine storm)
- 20-30% of cancer patients will succumb to cachexia

Phase IIa Clinical Data

- 18 Patients, various tumor types, primarily stage IV (84%)
- Results Demonstrated
  - Stabilization of weight, lean body mass, and body fat
  - Appetite increased (p=.001)
  - Total PG-SGA scores improved significantly (p =.025)
  - Enhanced quality of life
- Results seen even though 8/18 patients took concomitant chemotherapy or radiation
- 11/18 patients continued therapy after completion of the protocol for up to 153 days
- OHR/AVR118 was well tolerated with no serious side effects reported
- Detailed data expected to be presented at annual cachexia conference in Q4 2013

Corporate Strategy

Transition to core ophthalmology focus:

- Non-invasive delivery for back of the eye diseases including combination products
- Front of the eye diseases

In-license promising compounds to build pipeline

Out license or monetize non ophthalmology assets:

- OHR/AVR118 in cancer cachexia
- Trodusquemine and several analogs

Financial Highlights

Ticker	**NasdaqCM: OHRP**
Recent Share Price *(9-26-13)*	$8.09
Market Capitalization *(9-26-13)*	~$155mm
Average Daily Volume (30 day)	~60k shares
Cash on Hand *(6-30-13)*	~$6.05mm
Cash Burn Per Quarter	~$500-750K
Shares outstanding *(8-13-13)*	~19.7mm
Preferred Shares* *(6-30-13)*	~500k
Fully Diluted *(6-30-13)*	~26.9mm

*Convertible 3:1 into common stock at the holders option, no coupon

Cash on hand to fund operations into 2015

Analyst Coverage

- Jonathan Aschoff, Brean Capital

Investment Highlights

- 2 compounds in late stage development to address large unmet medical needs: *wet-AMD & cancer Cachexia*
- Strong intellectual property protection
- Experienced Management Team
- **Significant milestone events in 2013- 1H 2014**
  - ✓ *Results of phase IIa cancer cachexia trial*
  - ✓ *Listing on NASDAQ (mid 2013)*
  - ✓ *50% enrollment in ongoing wet-AMD eye drop trial (mid 2013)*
  - ○ *Publication of final trial results on resistant ovarian cancer orphan indication (median PFS, overall survival) (YE 2013)*
  - ○ *Presentation of OHR/AVR118 phase II results at society for cachexia and wasting disorders conference (Dec. 2013)*
  - ○ *Completion of enrollment in wet-AMD trial (Q1 2014)*
  - ○ *Interim data from wet-AMD eye drop trial (Q2 2014)*