



VISTA PARTNERS

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Price in USD (as of date of report): \$7.31

Corporate Overview



Ohr Pharmaceutical, Inc. ("the Company") based in New York, New York, is a research and development company with a primary focus in ophthalmology. The company's lead product, Squalamine, is currently being studied as an eye drop formulation in several company sponsored and investigator sponsored Phase II clinical trials for various back-of-the-eye diseases, including the wet form of age-related macular degeneration, retinal vein occlusion, diabetic macular edema, and proliferative diabetic retinopathy. In addition, Ohr has a sustained release micro fabricated micro-particle ocular drug delivery platform with several preclinical drug product candidates in development for glaucoma, steroid-induced glaucoma, ocular allergies, and protein drug delivery. The lead sustained release program in glaucoma is proceeding under a collaboration with a large global pharmaceutical company.

Website: www.ohrpharmaceutical.com

Stock Data

Industry: Biotechnology
Market Cap: \$181.3M**
Cash & STI (mrq): \$20.2M*
*(~3.5M Cash (mrq) + \$16.7M from April Offering)

Price Target: **\$31.00**
Shares Outstanding: 24.8M**
52 Week Range: \$4.20 – \$20.00
** Includes shares from mrq + shares issued to SKS

Highlights

- Released Phase II interim data for the Company's lead compound, Squalamine Eye Drops ("SED"), which showed increases in visual acuity
- Squalamine eye drops awarded Fast Track designation by the U.S. FDA for the potential treatment of the wet form of macular degeneration ("Wet-AMD")
- Raised approximately \$16.7M from institutional and accredited investors via a registered direct offering in April '14
- Acquired privately held SKS Ocular in May '14 which had a proprietary, patent protected, sustained release technology platform under development & a pipeline of pre-clinical sustained release drug product candidates
- Recently announced a joint venture with leading global cancer research center, Cold Spring Harbor Laboratory, to develop Trodusquemine and related analogs

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Business Description

Unless otherwise indicated, we use “OHR” and “the Company” in this report to refer to the business of [Ohr Pharmaceutical, Inc.](#) Ohr Pharmaceutical, Inc. (“the Company”) based in New York, New York, is a specialty research and development pharmaceutical company with a primary focus in ophthalmology. The Company's lead product, Squalamine, is currently being studied as an eye drop formulation in several company sponsored and investigator sponsored Phase II clinical trials for various back-of-the-eye diseases, including the wet form of age related macular degeneration, retinal vein occlusion, diabetic macular edema, and proliferative diabetic retinopathy. The Company's development pipeline also includes Trodusquemine (MSI-1436) which was recently validated as a therapeutic candidate for HER2-positive breast cancer, and is being developed by DepYmed, a joint venture with Cold Spring Harbor Laboratory and Ohr.

**Outlook, Conclusion &
Valuation****Squalamine Eye Drops**

OHR is advancing its clinical program in the exudative form of age-related macular degeneration (“wet-AMD”) with a novel topical eye drop formulation currently in a Phase II trial. Using its proprietary technology, OHR reformulated Squalamine for ophthalmic indications from an intravenous infusion (“IV”) to a topical eye drop. The topical Squalamine eye drop formulation is designed for enhanced uptake to the back of the eye and decreased potential for side effects. The previous IV formulation (acquired from Genaera Liquidating Trust) had been awarded fast track status and a Special Protocol Assessment (“SPA”) by the U.S. Food and Drug Administration for a Phase III registration study in patients with Wet-AMD. OHR's Squalamine Eye Drops were awarded Fast Track designation by the U.S. Food and Drug Administration (FDA) in May 2012 for its potential treatment of the wet form of macular degeneration (Wet-AMD).

The Squalamine Eye Drop program has the potential to create a monumental shift in the way patients are treated for Wet-AMD & Dry-AMD. It could provide tremendous benefit to patients who currently take chronic intravitreal injections of Roche/Genentech's Lucentis® or Regeneron's Eylea® directly into the eye.

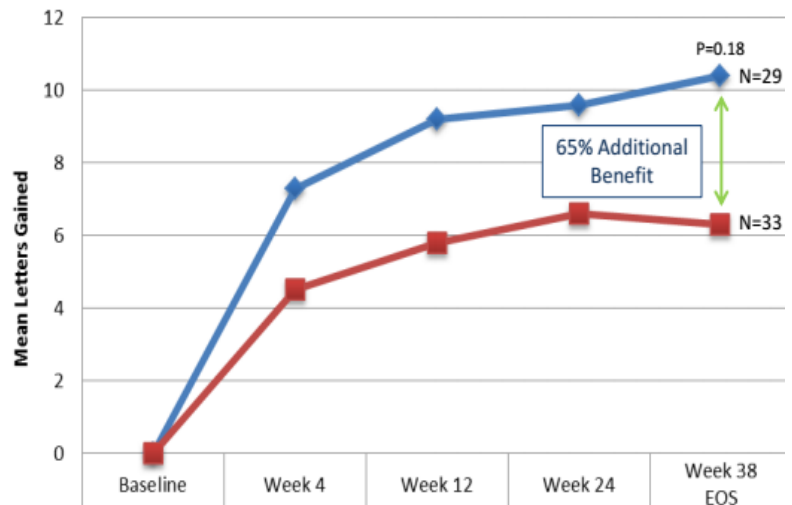
OHR's ongoing clinical trial (Study OHR-002) is a randomized, double-masked, placebo-controlled Phase II study to evaluate the efficacy and safety of Squalamine eye drops used in combination with Lucentis PRN for the treatment of wet-AMD. The trial has enrolled 142 newly diagnosed, treatment naive patients, of which the first 62 completed the treatment period at the time of the interim analysis. The inclusion criteria allowed for patients with visual acuity levels similar to previous Lucentis trials, varying lesion sizes up to 12 disc areas in size, and any lesion composition, including classic and occult only types of wet AMD. The trial also included diabetics with no concomitant diabetic retinopathy. Patients received an initial intravitreal injection of Lucentis at study entry, and then underwent a 1:1 randomization to receive a daily dose of either Squalamine

eye drops or placebo eye drops administered twice daily for nine months. Patients had monthly follow-up clinic visits, where they were evaluated and retreated as needed with Lucentis if pre-specified clinical criteria were met. The prespecified primary endpoint was the mean number of Lucentis injections and secondary endpoints included visual acuity as well as diagnostic imaging outcomes. The planned interim analysis was performed when more than 50 percent of the targeted study population finished their final study visit.

OHR completed enrolling patients in the ongoing Phase II trial, studying Squalamine for its potential indication in Wet-AMD, at the end of May 2014. The protocol includes an interim analysis, the data for which was released on the 24th of June, 2014, upon the completion of the treatment period in 50% of the patients (50%=60 patients). More information on the clinical trial can be found at clinicaltrials.gov.

The interim analysis showed that Squalamine eye drops were well tolerated and had a comparable safety profile to placebo eye drops. The data demonstrated a positive benefit in visual function across multiple clinically relevant endpoints **48.3% of Squalamine patients had BCVA (best-corrected visual acuity) gains of ≥ 15 letters (or 3 lines), versus 21.2% of placebo patients ($p=0.025$); patients receiving Squalamine were also more than twice as likely to gain ≥ 4 and ≥ 5 lines of vision compared with placebo patients (≥ 4 lines $p=0.022$, ≥ 5 lines $p=0.059$).** These outcomes in visual acuity (“VA”) were encouraging. Furthermore visual acuity improvements were seen as early as four weeks and **the relative difference in visual acuity between the two treatment arms continued to increase throughout the study**. OHR believes that Squalamine’s ability to improve VA in back of the eye disease may be due to its inhibition of multiple angiogenic growth factors. OHR-002’s interim results have given clinical validation to non-invasive topical eye drops treatment potential for back of the eye disease in general, as well as to OHR’s exclusive formulation of technologies. The positive VA results also provide a possible trajectory for a Phase 3 trial design, as clinical benefit is better established than injection frequency benefit, and according to OHR, vision endpoints are what the FDA has historically required for approval in wet-AMD. Consequently the Company will discuss trial registration with the FDA in the coming months prior to Phase II completion.

In addition, mean change in visual acuity (“VA”) shown on the chart on the next page at the end of study visit for the interim analysis group of +10.4 letters with Squalamine eye drops plus Lucentis PRN as opposed to +6.3 letters in the placebo eye drops plus Lucentis PRN arm, a 65% additional relative benefit ($p=0.18$).



Additionally the primary endpoint for the interim results the difference in the number of Lucentis injections, squalamine patients had on average 6.2 injections while placebo patients had on average 6.4. The Company believes that **showing VA improvements is far more meaningful and clinically relevant to a wet AMD patient than reducing the number of injections**, especially when the frequency of injections is already low (only 6-7 injections over the 38 week trial). The trial was designed as an exploratory study to evaluate the clinical utility of the molecule, and although the company chose injection frequency as the primary endpoint due to a greater likelihood of success, the secondary endpoint of visual acuity benefit is believed by OHR to be a far better outcome from a regulatory and patient perspective. OHR also believes that this result indicates Squalamine could be useful as a wet AMD prophylactic in dry AMD patients as well. There is a drug in development from Ophthotech (NASDAQ: OPHT; mkt cap \$1.42B) named Fovista®, which is an intravitreal anti-PDGF therapy that is currently in Phase III testing. Fovista® and Squalamine showed fairly similar gains in letters although the n's in the Ophthotech study were greater. The full analysis of the Squalamine interim data will be presented at an ophthalmology conference in 2H14 (most likely AAO, but potentially ASRS), and final data from all Phase II Wet AMD patients in 1Q15. We look forward to reviewing the full data set.

Squalamine Eye Drops also have the potential to be used in other retinal disorders which involve neovascularization and affect millions of patients. These disorders include diabetic macular edema ("DME"), proliferative diabetic retinopathy ("PDR") and retinal vein occlusions ("RVO").

To investigate these possible indications multiple studies have been undertaken, the following are some of the highlights from these studies:

- A Phase II investigator sponsored clinical trial, OHR-005, testing Squalamine Eye Drops in patients with DME announced on May 9th, 2014. The OHR-005 clinical trial is designed to evaluate the effect of Squalamine Eye Drops in patients with DME. The investigators in the trial are Dr. Daniel Roth of the Retina Vitreous Center/NJ Retina in New Brunswick, NJ, Dr. Lawrence J. Singerman of Retina Associates of Cleveland, and Dr. David S. Boyer of Retina-Vitreous Associates Medical Group, in Beverly Hills, CA.
- In August of 2013 the Company announced the initiation of an investigator-sponsored trial (OHR-004 clinical trial) to evaluate the efficacy and safety of Squalamine Eye Drops for the treatment of macular edema secondary to RVO. The trial is being conducted by Dr. John Wroblewski at Cumberland Valley Retina Consultants in Maryland and has completed enrollment of 20 treatment naïve patients. Dr. Wroblewski is a leading retinal specialist who also joined Ohr Pharmaceutical's scientific advisory board. Data from this study is expected to be presented at a scientific conference in 2H 2014.
- On February 20th, 2014 Ohr announced that they had presented a case report on the first patient treated, in the ongoing investigator sponsored trial (OHR-003), with Squalamine eye drops administered to patients with PDR and described the results as promising with regression of retinal neovascularization demonstrated in this monotherapy treatment with Squalamine. The case report was presented by the lead investigator, Michael J. Elman, M.D., Director of the Elman Retina Group from Baltimore, Maryland.

Trodsuquimine

In March of 2014 **the Company entered into a joint venture with, leading global cancer research center, Cold Spring Harbor Laboratory (CSHL)** and established DepYmed Inc., a new joint venture to develop trodsuquimine and related analogs. DepYmed Inc. recently validated Trodsuquimine as a therapeutic candidate for HER2-positive breast cancer. The joint venture is a private entity, initially with equal ownership by Ohr and Cold Spring Harbor Laboratory. The two partners will seek funding and contribute to the research and development of trodsuquimine and also newly patented analogs. The goal is to take the program into the clinic and to demonstrate proof of concept. Various options to fund later stage clinical trials will be explored. The continued preclinical research will be conducted under the guidance of Dr. Nicholas Tonks at CSHL, and a phase I trial is expected to be initiated by YE 2014 at North Shore LIJ Hospital for the treatment of breast cancer

Conclusion**Squalamine Eye Drops**

About 200,000 new cases of wet AMD are diagnosed each year in North America. As a result of the aging baby boomer population, the [National Eye Institute](#) estimates that the prevalence of advanced AMD will grow to nearly 3 million by the end of the next decade. Age-related Macular Degeneration (AMD) is the leading cause of irreversible vision loss in people over 65 in the United States. It is a slow, progressive, and painless condition that affects the macula, the small central part of the retina that allows you to see fine detail clearly. Many people develop AMD as part of the aging process. This disease can occur in two different forms known as either Dry-AMD or Wet-AMD.

The dry form of AMD accounts for 90% of all people with AMD. Wet-AMD affects 10% of people with AMD and is a much greater threat to vision. With the wet form of the disease, rapidly growing abnormal blood vessels develop under the central area of the retina. These vessels begin leaking fluid and blood that can cause severe loss of central vision. Currently, intravitreal injections of anti-VEGF therapeutics (Lucentis®, Eylea®, and Avastin®), which slows the growth of abnormal blood vessels that leads to Wet-AMD, is the standard of care treatment however some physicians use lasers to treating these leaky vessels. This treatment is limited to a small number of patients because the heat generated by the high power laser treatment can damage the retina and harm vision itself.

Despite new medical and surgical interventions, AMD remains an important cause of loss of vision worldwide. According to the [American Medical Association](#) (“AMA”) in the U.S., **advanced AMD is present in more than 1.75M individuals**, and owing to longer life span of Americans, the number of people with AMD is expected to increase to almost 3M by 2020. Anti-VEGF therapies such as: ranibizumab (Lucentis®), bevacizumab (Avastin®) and aflibercept (Eylea®) intravitreal injections have become the market leaders in treating AMD. Sales of Lucentis (ranibizumab) were approximately [\\$4.2B](#) in 2013. Lucentis was approved for age-related macular degeneration in the U.S. in June 2006 and the EU in January 2007. Regeneron’s EYLEA® sales for 2013 were [~\\$1.85B](#).

The anti-VEGF market has led to dramatic advances in the treatment of the disease, not only stabilizing vision, but also leading to significant visual gains. According to the AMA, **the Wet-AMD market represents a multi-billion opportunity worldwide**, with great potential for expansion with new treatment alternatives and combination therapies.

Squalamine eye drops may offer several potential advantages over just intravitreal injections (Lucentis® and Eylea®) as the current standards-of-care. Comparing Squalamine eye drops combine with intravitreal to intravitreal injections (IVT) alone:

- IVT’s requires monthly to bimonthly injections directly into the eye; Squalamine eye drops can be conveniently self-administered by the patient on a daily basis.

- IVT's have the propensity for side effects and potential inherent complications of an intravitreal injection, whereas Squalamine has shown a good safety profile even when administered systemically in significantly higher doses.
- Broad-spectrum inhibition of multiple angiogenic growth factors in addition to VEGF including PDGF, which has the potential to improve response rates and beneficial visual acuity outcomes.

Squalamine exhibited efficacy in more advanced cases of Wet-AMD in previous clinical trials. Squalamine is a small molecule anti-angiogenic with a novel intracellular mechanism of action that counteracts not only Vascular Endothelial Growth Factor ("VEGF") but also other angiogenic growth factors such as Platelet Derived Growth Factor ("PDGF"). Recent clinical evidence has shown PDGF to be an additional key target for the treatment of Wet-AMD. As mentioned previously, the positive VA results also provide a possible trajectory for a Phase 3 trial design, as clinical benefit is better established than injection frequency benefit, and according to OHR, vision endpoints are what the FDA has historically required for approval in wet-AMD. Consequently the Company will discuss trial registration with the FDA in the coming months prior to Phase II completion.

Recent Achievements

- At the end of May the Company completed an agreement with privately held SKS Ocular LLC and its affiliate SKS Ocular 1 LLC ("SKS Ocular") to acquire SKS Ocular's ophthalmology assets. The transaction will provide Ohr Pharmaceutical with a proprietary, patent protected, sustained release technology platform under development as well as a pipeline of pre-clinical sustained release drug product candidates that address unmet medical needs in glaucoma, retinal disease and other ophthalmic indications. The lead development program is currently being pursued under a research collaboration with a large global pharmaceutical company. As part of the agreement, Ohr will also gain a strong research and development team and a state of the art research laboratory in San Diego, CA. In connection with this transaction, three of the cofounders of SKS Ocular are being appointed to senior management and advisory roles at Ohr. Jason Slakter, MD has been appointed Chief Medical Officer and is expected to join the Board of Directors. Dr. Glenn L. Stoller has been appointed Chief Scientific Officer. Dr. Peter K. Kaiser will serve as Senior Vice President of product development. To view their bios, [click here](#).
- On April 8th, 2014, announced that it has entered into subscription agreements with institutional and accredited investors for the sale of its common stock in a registered direct offering. The Company is selling 1.8 million shares of common stock at a price of \$10.00 per share, **for gross proceeds of approximately \$18 million. The financing provides Ohr with the necessary resources to continue their clinical development programs, and seek out additional innovative ophthalmic candidates. Ohr completed enrollment in the OHR-002 wet-AMD**

study and initiated two randomized, controlled investigator sponsored trials to evaluate Squalamine eye drops for the treatment of diabetic macular edema early in the current quarter. Final data on the OHR-002 study is expected to be available in the first quarter of calendar 2015.

- On March 3rd of 2014 Ohr announced a joint venture with, leading global cancer research center, Cold Spring Harbor Laboratory (CSHL) for the establishment of DepYmed Inc., a new joint venture to develop trodusquemine and related analogs.
- On February 20th, 2014 Ohr announced that they had presented a case report on the first patient treated, in the ongoing investigator sponsored trial (OHR-003), with Squalamine eye drops in proliferative diabetic retinopathy ("PDR") and described the results as promising. The case report was presented by the lead investigator, Michael J. Elman, M.D., Director of the Elman Retina Group from Baltimore, Maryland.
- In November, 2013 the Company announced the granting of a Canadian patent for Squalamine. Issued as patent # CA 2606077, entitled "Polymorphic and Amorphous Salt Forms of Squalamine Dilactate".
- On December 11th, 2013 the Company announced that full results from its phase II trial to evaluate the effects of OHR/AVR118 in advanced cancer patients with cachexia. According to the Company the data showing the results which were achieved in these trials with OHR/AVR118 were very promising,
- In August of 2013 the Company announced the initiation of an investigator-sponsored trial (OHR-004 clinical trial) to evaluate the efficacy and safety of Squalamine Eye Drops for the treatment of macular edema secondary to retinal vein occlusion (RVO).

Valuation

We increased our split adjusted 12-month price target from \$14 to \$31 based on the promising interim data and SKS acquisition. Our price target is based on our risk-adjusted net present value calculation, which we conservatively estimate to be \$780,000,000, a little more than half of Ophthotech's (Nasdaq: OPHT) market cap of [\\$1.43B](#). We understand there is significant execution risk for OHR. With that said, we believe that the current valuation doesn't reflect the potential upside. We may look to revise our price target higher upon reviewing the entire Phase II data from the Squalamine Eye Drop Phase II trial expected in 1Q15.

We believe OHR could represent an attractive acquisition candidate based on the fact the Company is conducting late stage trials in large addressable markets. Large pharmaceutical companies are actively seeking to acquire smaller pharmaceuticals companies with late stage compounds and/or an approved product and OHR could possibly fit that bill. **On May 20, 2014, Novartis bought the ex-U.S. rights to Ophthotech's Fovista® for Up to \$1B.** On August 21, 2012 Allergan, Inc. (NYSE: AGN) and Molecular Partners AG announced that they have significantly expanded their

existing relationship by entering into two separate agreements to discover, develop, and commercialize proprietary therapeutic DARPIn® products for the treatment of serious ophthalmic diseases. Molecular Partners will receive combined upfront payments of \$62.5M under the two agreements and are eligible to receive additional success-based payments, including up to **\$1.4B** in aggregate development, regulatory and sales milestones, and tiered royalties up into the low double-digits for future product sales. We should note the DARPIn compound that targets both VEGF and PDGF angiogenic growth factors intended to treat Wet-AMD is currently in the pre-clinical stage.

We have listed some biotechnology deals below:

- Perrigo acquisition of Elan (\$8.6B)
- GlaxoSmithKline acquisition of Human Genome Sciences (\$2.8B)
- Gilead acquisition of Pharmasset (\$10B)
- Astellas acquisition of OSI (\$4B)
- Bristol Meyers acquisition of Inhibitex Inc. (\$2.5B)
- Amgen acquisition of BioVex (\$970M)
- Daichi Sankyo acquisition of Plexxikon (\$935M)
- Cubist acquisition of Adolor (\$415M)

Competition

If regulatory authorities approve the marketing and selling of any of OHR's product candidates, OHR's product candidates will face significant and long-term competition from pharmaceutical companies, pharmaceutical divisions of companies, and biotechnology, biopharmaceutical, and specialty pharmaceutical companies, among others. This competition likely will become more intense if any of OHR's products or competitor products achieves significant commercial success. Most of the competitors, particularly large pharmaceutical companies, have greater clinical, regulatory, manufacturing, marketing, distribution, compliance, and financial resources, as well as more experience than OHR. Additionally, many of these companies have commercial arrangements with other companies to supplement their internal capabilities.

Over the longer term, OHR's ability, independently or with a strategic or other partner, to successfully manufacture, market, distribute and sell any of OHR's or their approved products, expand their usage and bring new products to the marketplace will depend on many factors, including, but not limited to, the effectiveness and safety of those products, FDA and foreign regulatory agencies' approvals of new products and indications, the degree of patent protection afforded to particular products and the rates at which those products are reimbursed.



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Risks

Risks

As the majority of the Company's resources are focused on emerging products in the development stage, the Company expects to incur additional losses for the foreseeable future and will require additional financial resources. The continuation of the Company's research and development activities and the commercialization of its products are dependent upon the Company's ability to successfully complete its research and development programs, protect its intellectual property, obtain strategic partner support and finance its cash requirements on an ongoing basis.

The Company expects that its growth and future prospects will be largely dependent on the success of its clinical candidates. We encourage investors to view all risks listed in the Company's annual report on file with the Securities Exchange Commission which is available on the SEC website, www.sec.gov.

Legal Notes & Disclosures

This report has been prepared by Vista Partners LLC ("Vista") with the assistance OHR Pharmaceutical, Inc. ("the Company") based upon information provided by the Company. Vista has not independently verified such information, and in addition, Vista has been compensated by the Company for advisory services for a one year period. Vista and its officers may have an equity interest – both restricted and unrestricted in the securities mentioned herein. Statements in this report that are not historical facts are "forward-looking statements" that involve risks and uncertainties. Forward-looking statements can be identified by the use of words such as "opportunities," "trends," "potential," "estimates," "may," "will," "could," "should," "anticipates," "expects" or comparable terminology or by discussions of strategy. Such statements involve known and unknown risks, uncertainties and other factors that may cause actual results, performance or achievements to be materially different from the results, performance or achievements expressed or implied by such forward-looking statements. Additional risks, uncertainties and other factors are identified under the captions "Risk Factors" and "Special Note Regarding Forward-Looking Statements" in the Company's reports filed from time to time with the Securities and Exchange Commission, including its Annual Report for the current fiscal year. Vista and the Company disclaim any intention or obligation to update publicly or revise any forward-looking statements, whether as a result of new or additional information, future events or otherwise. The Company is solely responsible for the accuracy of that information. Information as to other companies has been prepared from publicly available information and has not been independently verified by the Company or Vista. For more complete information about the Company. The reader is directed to the Company's website, www.ohrpharmaceutical.com.

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