UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549
FORM 8-K

CURRENT REPORT

Pursuant to Section 13 OR 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 6, 2021

Ocuphire Pharma, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**001-34079**	**11-3516358**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

37000 Grand River Avenue, Suite 120 Farmington Hills, MI	**48335**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: **(248) 681-9815**

N/A

(Former name or former address, if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value	OCUP	Nasdaq Capital Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure.

On January 6, 2021, Ocuphire Pharma, Inc. (the "Company") posted an updated corporate presentation to its website at https://ir.ocuphire.com/presentations, which the Company may use from time to time in communications or conferences. A copy of the corporate presentation is attached as Exhibit 99.1 to this Current Report on Form 8-K (this "Report").

The information in this Report, including Exhibit 99.1 hereto, is furnished pursuant to Item 7.01 and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that Section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. The Company's submission of this Report shall not be deemed an admission as to the materiality of any information required to be disclosed solely to satisfy the requirements of Regulation FD.

This Report and Exhibit 99.1 hereto contain forward-looking statements within the meaning of the federal securities laws. These forward looking statements are based on current expectations and are not guarantees of future performance. Further, the forward-looking statements are subject to the limitations listed in Exhibit 99.1 and in the other reports of the Company filed with the Securities and Exchange Commission, including that actual events or results may differ materially from those in the forward-looking statements.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Exhibit Description
99.1	Corporate Presentation, dated January 2021.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

OCUPHIRE PHARMA, INC.

By: /s/ Mina Sooch
Mina Sooch
Chief Executive Officer

Date: January 6, 2021

Exhibit 99.1

Ocuphire PHARMA

Restore Vision & Clarity

Ocuphire Corporate Presentation

January 2021

Disclosures and Forward Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements concerning Ocuphire Pharma, Inc.'s ("Ocuphire" or the "Company") product candidates and potential. These forward-looking statements are based upon the Company's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, including, without limitation: (i) potential adverse reactions or changes to business relationships resulting from the announcement or completion of the merger; (ii) the success and timing of regulatory submissions and pre-clinical and clinical trials; (iii) regulatory requirements or developments; (iv) changes to clinical trial designs and regulatory pathways; (v) changes in capital resource requirements; (vi) risks related to the inability of the Company to obtain sufficient additional capital to continue to advance its product candidates and its preclinical programs; (vii) legislative, regulatory, political and economic developments, and (viii) the effects of COVID-19 on clinical programs and business operations. The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors detailed in documents that have been and may be filed by the Company from time to time with the SEC. All forward-looking statements contained in this presentation speak only as of the date on which they were made. The Company undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

The Company makes no representation or warranty, express or implied, as to the accuracy or completeness of the information contained in or incorporated by reference into this presentation. Nothing contained in or incorporated by reference into this presentation is, or shall be relied upon as, a promise or representation by the Company as to the past or future. The Company assumes no responsibility for the accuracy or completeness of any such information. This presentation may not be reproduced or provided to any other person (other than your advisor) without our prior written consent. By accepting delivery of this presentation, you agree to the foregoing and agree to return this presentation and any documents related thereto and any copies thereof to us or to destroy the same if you do not make an investment in any securities. The information contain within this presentation shall not, except as hereinafter provided, without the prior written consent of the Company, be disclosed by you or your representatives in any manner whatsoever, in whole or in part, and shall not be used by you or your representatives other than for the purpose of evaluating the transaction described herein. By accepting delivery of this presentation you further acknowledge and agree aware of the restrictions imposed by the United States securities laws on the purchase or sale of securities by any person who has received material, nonpublic information from the issuer of the securities or any affiliate thereof and on the communication of such information to any other person when it is reasonably foreseeable that such other person is likely to purchase or sell such securities in reliance on such information for so long as the information remains material and non-public. This presentation also contains estimates and other statistical data made by independent parties and by us relating to market shares and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. The trademarks included herein are the property of the owners thereof and are used for reference purposes only. Such use should not be construed as an endorsement of such products.

Ocuphire Opportunity

A Late-Stage Clinical Ophthalmic Biotech (Nasdaq Symbol: OCUP)

Late Clinical Stage Company Targeting Large, Unmet Ophthalmic Markets	• Nyxol eye drops target multiple chronic and acute front of the eye indications addressing large markets: Dim Light / Night Vision Disturbances (NVD), Reversal of Mydriasis (RM), & Presbyopia (P) • APX3330 tablets target chronic back of the eye indications: Diabetic Retinopathy (DR) and Diabetic Macular Edema (DME), a leading cause of blindness in diabetic patients
Significant Clinical Data and Regulatory Precedents	• Nyxol and APX3330 achieved promising clinical data over multiple Phase 1 and 2 trials ✓ Nyxol with > 150 patients treated across 7 trials ✓ APX3330 with > 340 patients treated across 11 trials • FDA End of Phase 2 meeting guidance for Nyxol (all indications) in May 2020
Significant IP Portfolio and Small Molecule CMC Advantages	• US and global issued patents thru 2034 obtained for both assets • Stable, small-molecule drugs ✓ Nyxol = single-use, preservative-free eye drop ✓ APX3330 = oral pill
Multiple Near-Term Data Catalysts with Capital Efficient Plan	• 4 late-stage trial readouts (2 Phase 3, 2 Phase 2) expected in 1Q through 4Q 2021 • $20+M financing provides sufficient cash to run capital-efficient ophthalmic-focused operations in 2021 • Analyst research coverage initiated by Cantor Fitzgerald and Encode Ideas • Nyxol NDA filing in one or more indications targeted for early 2023

Ocuphire PHARMA

Ocuphire Management Team

Decades of Biotech and Drug Development Experience

Mina Sooch, MBA
President & CEO and Founder

HARVARD BUSINESS SCHOOL
Gemphire Therapeutics
Apjohn Ventures
MONITOR
ProNAi

Charlie Hoffman, MBA
VP Corporate Development and Operations

Tuck School of Business at Dartmouth
Ocularis Pharma
Prudential
SynDevRx
Goldman Sachs

Amy Rabourn, CPA
VP Finance

MICHIGAN ROSS
Gemphire Therapeutics
Pfizer
NeuroBo PHARMACEUTICALS
pwc

Kostas Charizanis, PhD, MBA
Senior Director Market Strategy and R&D

MICHIGAN ROSS
RUBICON GENOMICS
Gemphire Therapeutics

Mitch Brigell, PhD
Head Clinical Development and Strategy

KANSAS STATE UNIVERSITY
aerpio
Pfizer
NOVARTIS

Daniela Oniciu, PhD
Head CMC and Global Clinical Supply

UF UNIVERSITY of FLORIDA
Gemphire Therapeutics
ESPERION
Cerenis™ THERAPEUTICS
Pfizer

Drey Coleman
Director Clinical Operations and Vendor Management

UCF
SIRION Therapeutics
OCULOS Integrated Insight.

Laura Gambino
Project Management

EASTERN MICHIGAN UNIVERSITY
aerpio
Pfizer
IQVIA

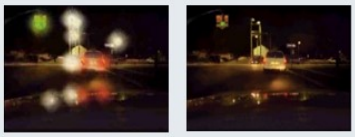
Ocuphire PHARMA

Large Unmet Opportunities for the Aging Eye

Developing Drugs to Treat Front & Back of the Eye Diseases

Night Vision Disturbances

U.S. Prevalence: ~16M adults



Reversal of Mydriasis

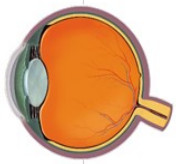
~100M pupil dilations per year in U.S.

Presbyopia

U.S. Prevalence: ~120M

$4-10B US Markets

Front | **Back**



Nyxol®

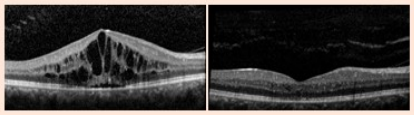
APX3330

Diabetic Retinopathy

U.S. Prevalence:~ 7M

Diabetic Macular Edema

U.S. Prevalence: ~750K



$4-10B US Markets

5 Source: GlobalData Market Research Report, 2020

Ocuphire PHARMA

Ocuphire Pipeline & Upcoming Milestones

Multiple Phase 3 & Phase 2 Clinical Data Catalysts Expected Throughout 2021

	Product Candidate	Indication	Development Stage				Anticipated Milestones
			Pre-clinical	Phase 1	Phase 2	Phase 3	
Ocuphire-Focused Development	0.75% Nyxol® Eye Drop	Dim Light or Night Vision Disturbances (NVD)					Initiated Phase 3 LYNX-1 trial 4Q2020; Data expected in 3Q21 (n=160)
	0.75% Nyxol® Eye Drop	Reversal of Mydriasis (RM)			Enrollment Complete		Initiated Phase 3 MIRA-2 trial 4Q2020; Data expected in 1Q21 (n=168)
	0.75% Nyxol® + Low-Dose 0.4% Pilocarpine Eye Drops	Presbyopia (P)					Initiate Phase 2 VEGA-1 trial 1Q2021; Data expected in 2Q21 (n=152)
	APX3330 Oral Pill	Diabetic Retinopathy (DR)/ Macular Edema (DME)					Initiate Phase 2 ZETA-1 trial 1Q2021; Data expected in 4Q21 (n=100)
Partnering-Focused Development	APX2009 Intravitreal	DME, Wet Age-Related Macular Degeneration (wAMD)					Next steps: IND enabling studies (with partner funding)
	Combo (0.75% Nyxol® + Latanoprost) Eye Drops	Glaucoma (16 to 24 mmHg)					Next steps: 2nd line add-on Phase 2 trial (with partner funding)

Note: 0.75% Nyxol (Phentolamine Ophthalmic Solution) is the same as 1% Nyxol (Phentolamine Mesylate Ophthalmic Solution)

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Extensive Development on Both Drug Candidates

Well-Controlled Phase 1 & Phase 2 Clinical Programs Set Stage for NDA Path

Nyxol

7
Phase 1 & Phase 2 Trials

> 150
Subjects Dosed

Exposure in Humans
28
Days

Patents to
2034+

Studied in ocular refractory diseases (NVD) & elderly glaucoma patients

505(b)(2) Development Pathway

APX3330

11
Phase 1 & Phase 2 Trials

>340
Subjects Dosed

Exposure in Humans
365
Days

Patents to
2034+

Studied in inflammation/hepatitis & cancer patients

NCE Development Pathway

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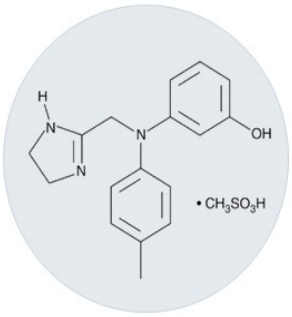
Nyxol®

- **NVD** Night Vision Disturbances
- **RM** Reversal of Mydriasis
- **P** Presbyopia



Phentolamine Mesylate

Nyxol History & MOA

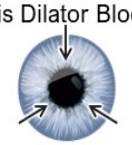
Rationale for Differentiated Product Profile & 505(b)(2) Path

- Nyxol's active ingredient, phentolamine mesylate (PM), is currently approved for 2 indications
  - Pheochromocytoma (60+ years ago, Regitine®) – intravenous injection
  - Reversal of oral anesthesia (10+ years ago, OraVerse®) – intramuscular injection
- PM has been reformulated as a topical eye drop (Nyxol)
- Nyxol is a first-in-class non-selective α1 and α2 blocker product candidate
  - MOA of relaxing the iris dilator muscle (α1)
  - Redness is an on-target α1 effect on sclera vessels (transient, mild)

Phentolamine Mesylate

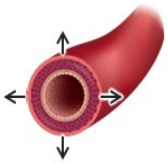
Reduces Pupil Size

α1: Iris Dilator Blockade



Dilates Blood Vessels (Vasodilation)

α1: Smooth Muscle Blockade



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Nyxol Product Candidate Profile

First-in-Class Alpha 1/2 Blocker Eye Drop for Refractory Indications

Nyxol: Phentolamine 0.75% Ophthalmic Solution
Preservative Free, EDTA Free, and Stable

Efficacy Data

Improving Vision

- ↓ Pupil Size (moderate miotic)
- ↑ Contrast Sensitivity (night)
- ↑ Near Visual Acuity (light/dark)
- ↑ Distance Visual Acuity

Safety Data

No Systemic Effects
No Changes in Blood Pressure
No Changes in Heart Rate

Tolerated Topical Effects
Mild / Transient / Reversible Eye Redness

IOP Unchanged or Decreased
↓ Intraocular Pressure (IOP) at Normal Baseline

Chronic daily dosing of Nyxol at bedtime demonstrated ***no significant daytime redness*** *and durability of effects for more than* ***24 hours***

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Night Vision Disturbances (NVD) – Chronic Opportunity

Imperfections in the Eye Affect Night Vision in Millions

The Problem

- Peripheral imperfections scatter light when pupils enlarge in dim light, causing halos, starbursts, and glare that impair vision
- The imperfections may be caused by LASIK surgery, IOL implants, certain types of cataracts (cortical), and natural reasons (especially with age)
- Symptoms cannot be properly corrected by any type of lens (reading glasses, contact lenses) or surgical procedures

> *"I'm no longer comfortable driving at night, especially with my son in the car. I have a hard time playing beach volleyball in the evenings due to the bright lights at the courts."*
>
> *Post-LASIK, aged 42*

No Currently Approved Therapies

Moderate-to-Severe NVDs	US Patients
Night Myopia	10.8M
Cortical Cataracts	4.1M
Post-LASIK	500k
Post-IOL Implant	300k
Total	**~16M**

Source: GlobalData Market Research Report, 2020

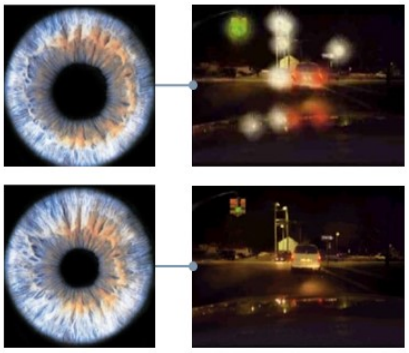
Night Vision Disturbances (NVD) – Chronic Opportunity

Peripheral Optical Imperfections Allowing Pupil Modulation as a Solution

Nyxol's Potential Differentiated Solution

- **Moderate Decrease in Pupil Size** for scattered light gets blocked by the iris
- **Clinical Effect** to potentially improve low contrast night vision as seen in trials
- **Tolerable** with a minimal side effect profile
- **Convenient and Durable** with chronic once-daily evening dose

> *"Once there is a drug and a category, that's when they start looking for the disease."*
>
> *Physician KOL*



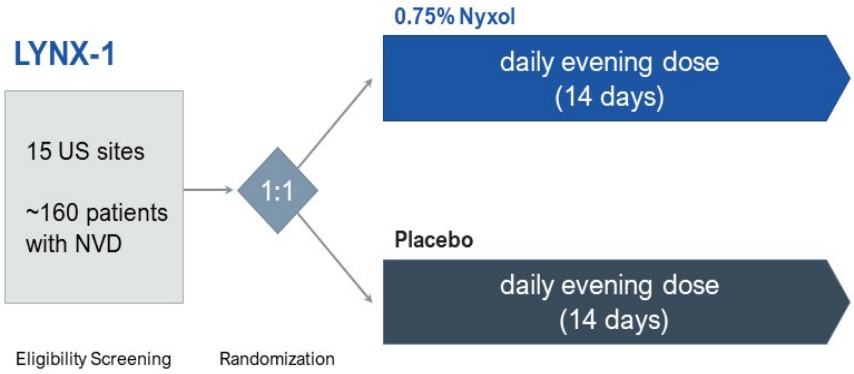
Before

After

Seeking Treatment Findings	
Patients willing to try a new eye drop treatment	67%
Patients avoiding driving at night	25%

NVD LYNX-1 Phase 3 Registration Design

Randomized, Double-Masked, Placebo-Controlled Two-Week Trial



Phase 3 Initiated in Late 4Q20

Top Line Expected 3Q21

Endpoints

Primary: % of subjects with ≥ 3 lines of improvement in mesopic low contrast best-corrected distance visual acuity (Day 8)

Secondary (Days 8 & 15):

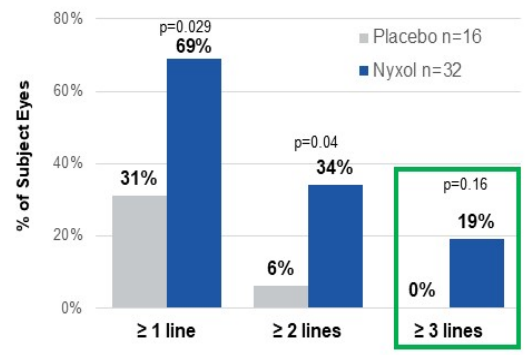
- Pupil diameter
- Visual acuity measures (distance and near)
- Safety and tolerability (redness)

Nyxol Demonstrated Clinical Effect in NVD

Key Endpoints Observed in Multiple Phase 2 Trials

NYX-SNV Phase 2 Trial

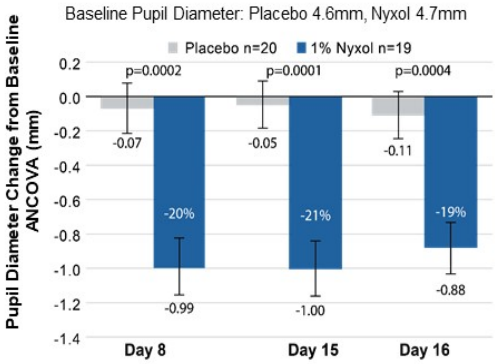
Improved Low Contrast Distance Visual Acuity*



Source NYX-SNV

ORION-1 Phase 2 Trial

Durable > 24 hour Pupil Modulation Effect



Source: NYXG-201

*NYX-SNV trial was small and not designed for a statistical 3-line improvement in low-contrast visual acuity; the 20% effect was used for powering and sizing of Phase 3 trial

Reversal of Mydriasis (RM) – Acute Treatment

Annual Exams and Specialty Visits Involve Dilation to Monitor Eye Health

The Problem

- At every annual eye exam and many specialty visits, pupils are pharmacologically dilated, impairing vision for 6-24 hours
- Dilated eyes:
  - heightened sensitivity to light
  - inability to focus
  - reading, working, and driving are difficult
  - halos and glare

> *"I have to stay indoors. They say it only lasts a few hours, but it lasts all day, and it is very annoying."*
>
> *RM Patient, Aged 51*

No Currently Approved Therapies

~100M eye exams / year in US

Source: GlobalData Market Research Report, 2020

Reversal of Mydriasis (RM) – Acute Treatment

Single Use Indication Leveraging a Precedent Approval Pathway

Nyxol's Potential Differentiated Solution

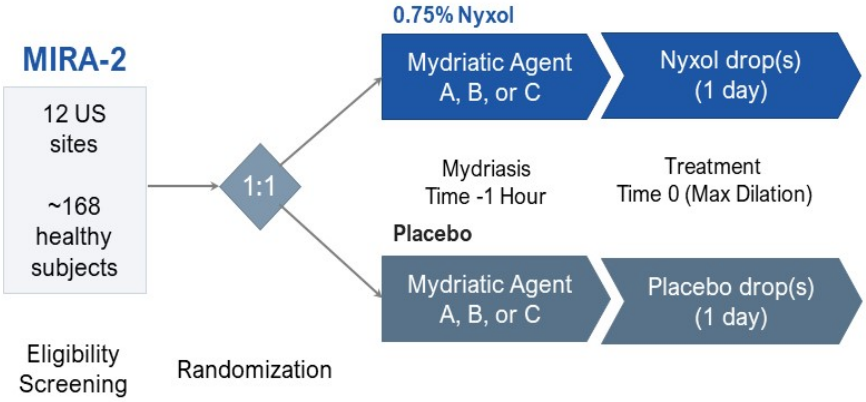
- **Regulatory Precedent** with RevEyes (an alpha 1 blocker), approved by the FDA in 1990 but shortly thereafter discontinued (not for safety or efficacy reasons)
- **Clinical Effect** to potentially reduce pupil size and reverse mydriasis by counteracting the drugs (alpha agonists and cholinergic blockers) used to dilate the pupil
- **Convenient** eye drop given at the office that may allow vision to return to normal sooner
- **Tolerable** with a minimal side effect profile (unlike cholinergic agonists such as pilocarpine)

Seeking Treatment Findings	
Patients likely to request reversal of dilation	45%
Eye care providers likely to use reversal drops	40%

Source: GlobalData Market Research Report, 2020

RM MIRA-2 Phase 3 Registration Design

Randomized, Double-Masked, Placebo-Controlled, Parallel, One-Day Trial



Started and Completed Enrollment in 4Q20 – 185 Subjects

Top Line Expected 1Q21

Endpoints

Primary: % of subjects returning to baseline (within 0.2 mm) pupil diameter at 90 min

Secondary:

- % of subjects returning to baseline at 30min, 1h, 2h, 3h, 4h, 6h, 24h
- Mean change in pupil diameter from mydriatic max at all timepoints
- Accommodation (tropicamide)
- Safety and tolerability (redness)

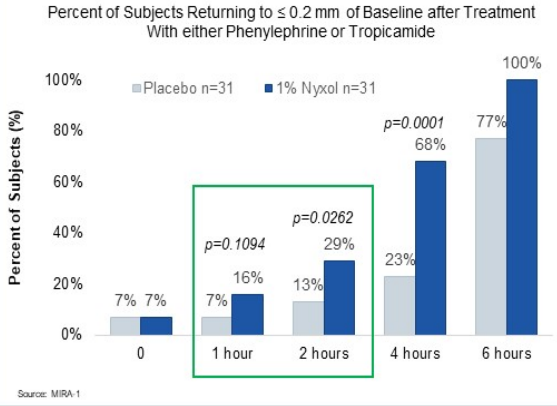
Mydriatic Agents: phenylephrine (alpha agonist), tropicamide (cholinergic blocker), Paremyd® (combination)

Nyxol Demonstrated Clinical Effect in RM

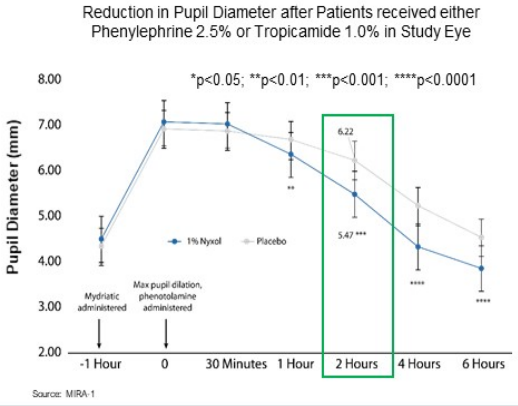
Key Endpoints Observed from MIRA-1 Phase 2b Trial

MIRA-1 Phase 2 Trial

Nyxol Reduced More Subjects to Pupil Diameter (PD) Baseline



Nyxol Reduced PD Faster Across Phenylephrine & Tropicamide Mydriatic Agents



MIRA-2 protocol enhancements: 1 ➔ 2 drops of Nyxol in study eye AND dark irides ➔ dark/light irides

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Presbyopia – Chronic Opportunity

Aging Population Drives Demand for Alternatives to Reading Glasses

The Problem

- Lens loses ability to change shape when viewing objects up close as we age
- Dependence on reading glasses for intermittent and prolonged use
- Growing need for therapies that improve, rather than hinder, quality of life

> *"Effectively everyone over 40 will have the problems with reading."*
> *Physician KOL*

No Currently Approved Drug Therapies

Seeking Treatment Findings	
Patients requesting alternative to reading glasses	40%
Patients would consider an eye drop alternative	69%

Source: GlobalData Market Research Report, 2020

Ocuphire PHARMA

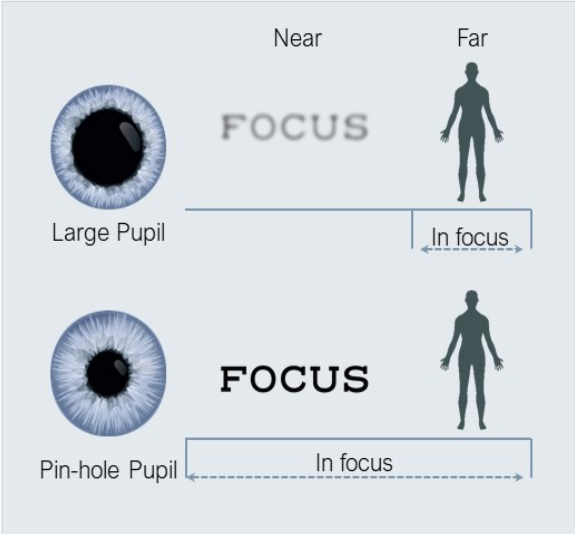
Presbyopia – Chronic Opportunity

Pupil Modulation Eye Drops May Replace Reading Glasses

Nyxol's Potential Differentiated Solution

- **"Pin-hole"** effect of Nyxol and low dose pilocarpine may improve near vision by increasing depth of focus as validated by other devices/therapies
- **More durable** combination of two miotics affecting different muscles (iris dilator and sphincter) involved in pupil size modulation
- **Tolerable** use with minimal side effects expected with chronic evening use of Nyxol

> *"This would just become part of my daily routine for my eyes to be able to see things up close. How convenient is that?"*
>
> *Presbyopic Patient, age 49*

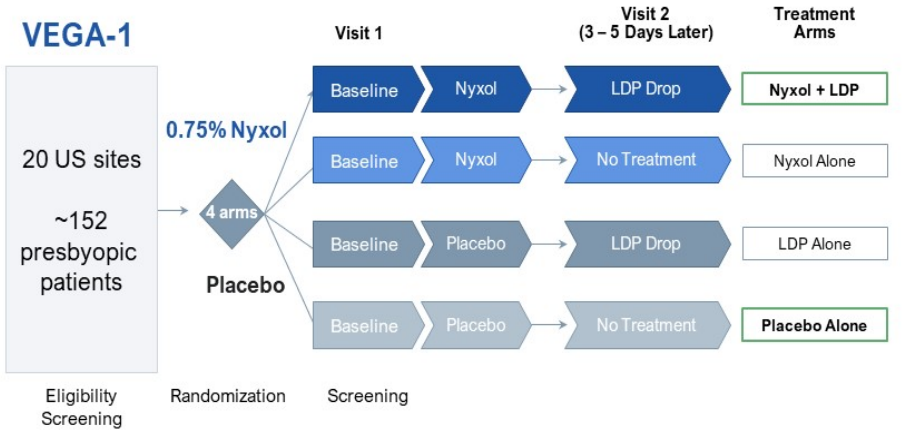


Retinaeyedoctor.com, GlobalData Market Research Report, 2020

Ocuphire Pharma

Presbyopia VEGA-1 Phase 2 Proposed Design

Planned Randomized, Double-Masked, Placebo-Controlled One-Week Trial



Phase 2 Start Targeted for 1Q21

Top Line Expected 2Q21

Endpoints

Primary: % of subjects with ≥ 3 lines of improvement in distance-corrected near visual acuity comparing Nyxol + LDP vs placebo alone at 1 hour

Secondary:

- % of subjects with ≥ 2 and ≥ 3 lines gained at time points from 30 min to 6 hours in photopic and mesopic lighting comparing Nyxol + LDP vs placebo, Nyxol alone, and LDP alone
- No loss of distance vision
- Pupil diameter at time points
- Safety and tolerability (redness)

LDP= low-dose pilocarpine (0.4%)

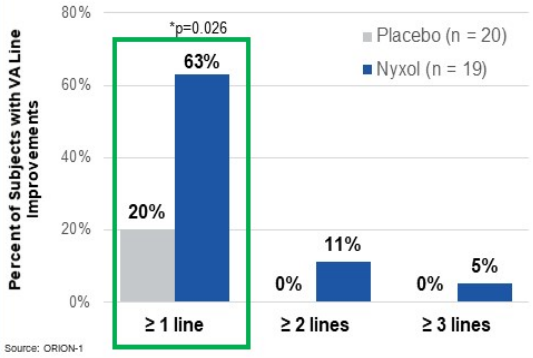
Nyxol Demonstrated Clinical Effect in Presbyopia

Key Endpoints Observed from Multiple Phase 2 Trials

ORION-1 Phase 2 Trial

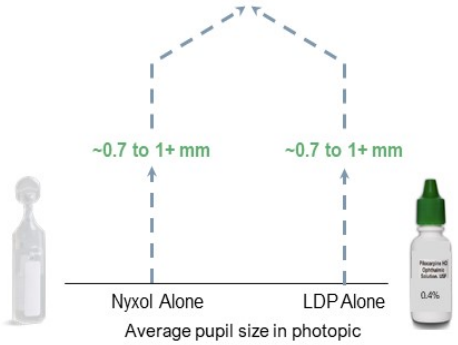
Improvements in DCNVA with Nyxol Alone With No Change in Distance Vision

Near VA Line Improvement at Day 15
(Full Analysis Set, Study Eye, Photopic)



Pinhole PD Size Illustrated with Addition of LDP to Nyxol

1.5 to 2+ mm PD reduction
Achieve the pin-hole (1.6 to 2 mm) effect



Average pupil size in photopic conditions is 3.5 to 4+ mm

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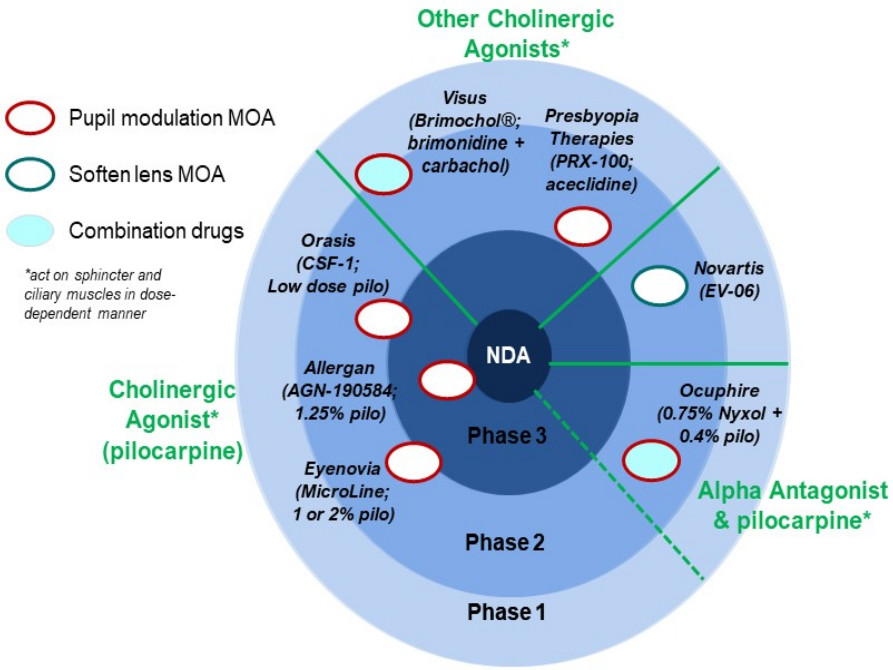
Presbyopia Eye Drops Competitive Landscape

Validation of Pupil Modulating Drops Achieving Pin-Hole Effect & Efficacy, Many with Pilocarpine



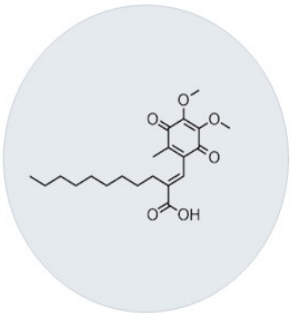
An ideal formulation for presbyopia treatment would meet the following criteria:

- Comfort and tolerability
- Fast onset
- Long duration
- Efficient pupil size modulation
- Strong safety profile
- Maintain good distance visual acuity

Corporate Websites, Grzybowski, A, Markeviciute A, Zemaitiene R. A Review of Pharmacological Presbyopia Treatment. 2020

APX3330

- **DR** Diabetic Retinopathy
- **DME** Diabetic Macular Edema
- **wAMD** Wet Age-Related Macular Degeneration

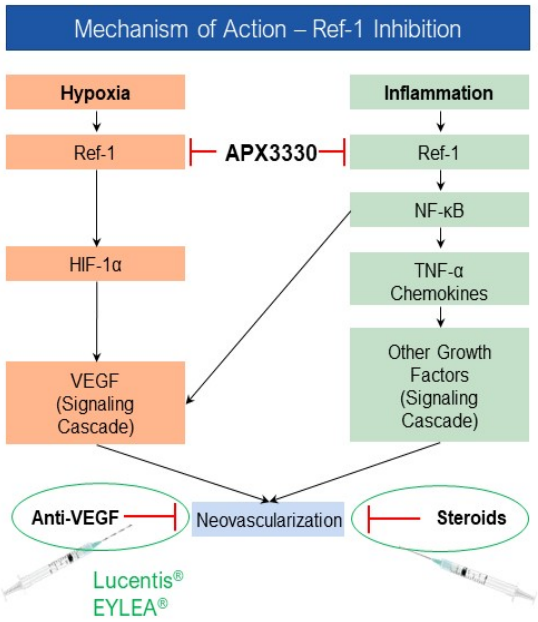


APX3330

APX3330 is a Ref-1 Inhibitor

Ref-1 Involved in Multiple Pathways that Contribute to Diabetic Retinopathy and DME

- APX3330 is a small molecule oral drug candidate and a first-in-class inhibitor of Ref-1
- Ref-1 (reduction-oxidation effector factor-1) is a novel target discovered and characterized by Dr. Mark R. Kelley at Indiana University School of Medicine
- APX3330 previously developed by Eisai for multiple hepatic inflammatory indications and later by Apexian for advanced solid tumors
  - Similar oncology origin as approved anti-VEGFs
- MOA uniquely decreases both abnormal angiogenesis and inflammation by blocking pathways downstream of Ref-1



Logsdon et al (2018), Li et al (2014).

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APX3330 Product Candidate Profile

First-in-Class Ref-1 Inhibitor Phase 2 Ready for Retina Diabetic Indications

APX3330: 600mg Oral Dose
(120mg or 300mg tablets)

Expected Efficacy Data

Improving Eye Health in Diabetics
- ↓ Inflammation
- ↓ Hypoxia Signaling
- ↓ Abnormal Angiogenesis

Enhance Compliance & Exposure
Oral pill may reduce the burden of frequent anti-VEGF injections

Safety Data

Few Systemic Adverse Effects
- Mild Gastrointestinal (diarrhea)
- Mild Skin Rash (Reversible)
- Lack of Significant Acute Neurologic, Cardiovascular, Liver, or Pulmonary toxicity

No Topical Effects
- No observed ocular AEs

Twice a day dosing of APX3330 being developed to provide steady state effectiveness with a tolerable chronic safety profile

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Diabetic Retinopathy & Macular Edema

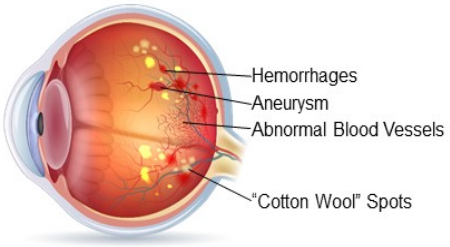
Non-Injectable Alternative Therapies are Needed For Earlier Stages of Disease

The Problem

- Diabetic retinopathy (DR) and diabetic macular edema (DME) are a leading cause of vision loss worldwide, especially in working age adults in developed countries
- Diabetes damages small blood vessels within the eye causing leakage, oxygen starvation, and abnormal vessel growth, which can obstruct vision
- DR patients are not commonly treated with approved injectable anti-VEGF drugs given earlier stage of retinal disease and many are asymptomatic
- DR progresses in steps and may result in vision loss if left untreated
- Current treatment for DME: 25% non-responders and 50% partial responders to anti-VEGF drugs

Injectable Anti-VEGF Approved Therapies Not Commonly Used for NPDR

Diabetic Eye



Diabetic Eye Opportunity	
DR	~7.7M Patients
DME	~750K Patients

Sources: Global Market Insights Report 2019-2025, Market Watch 2019 Report, Gene.com Retinal Diseases Fact Sheet

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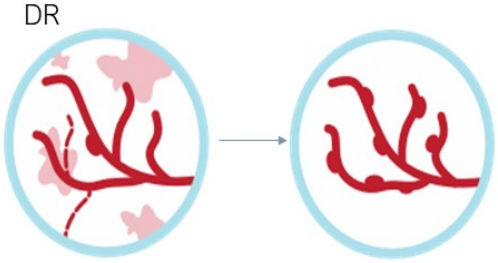
Diabetic Retinopathy & Macular Edema

APX3330 to Treat Patients Before Vision Loss Occurs

APX3330's Potential Differentiated Solution

- **Potential First Oral Therapy** to be used as an earlier intervention for the diabetic eye before vision symptoms appear or as add-on therapy to current anti-VEGF treatment
- **Proven Novel Mechanism** that may decrease both inflammation and VEGF activity
- **Convenient** option for patients to potentially alleviate the burden of injections and increase compliance
- **Tolerable** as seen in 11 completed Phase 1 and Phase 2 clinical trials

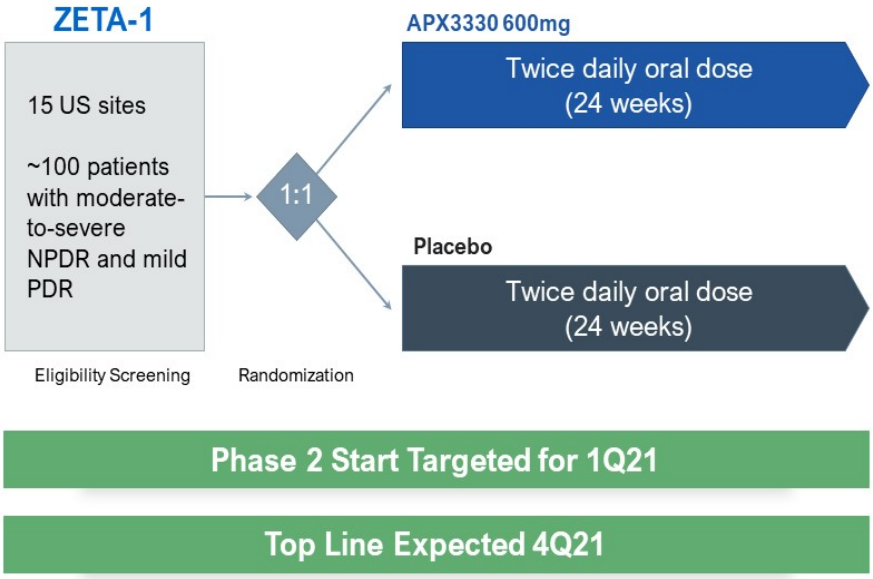
DR



DME

DR/DME ZETA-1 Phase 2 Proposed Design

Planned Randomized, Double-Masked, Placebo-Controlled 24-Week Trial



Endpoints

Primary: % of subjects with a ≥ 2 step improvement on the DRSS (Diabetic Retinopathy Severity Scale) score at week 24

Secondary:

- Central subfield thickness (CST)
- BCDVA (ETDRS)
- Safety and tolerability

Exploratory:

- Labs / PK

NPDR = non-proliferative diabetic retinopathy (which includes non centrally involved diabetic macular edema)
PDR = proliferative diabetic retinopathy (which includes non centrally involved diabetic macular edema)

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DR
DME

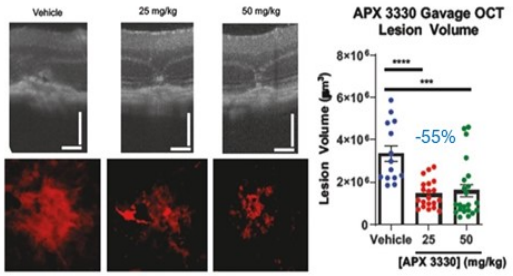
APX3330 Generally Well Tolerated with Clinical Signals

Observations from Pre-Clinical Studies and 11 Clinical Trials of APX3330

L-CNV Mouse Retina Model

APX3330 Reduces Neovascularization Similar to Eylea in Preclinical Models

Lesion Size and Corresponding Fluorescent Stains in L-CNV Models Treated with APX3330 at 25 mg/kg

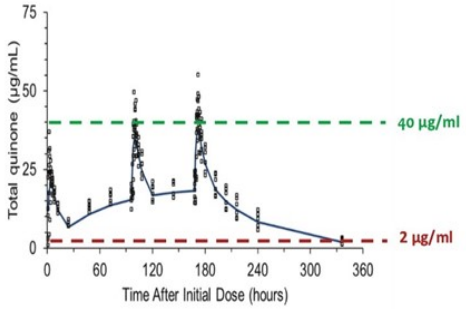


Source: Unpublished Data Dec 2019

Phase 1 Clinical Trials

Human Drug Exposure Multiple Times Higher than Mouse Efficacious Levels

Human Pharmacokinetics of APX3330 at 120 mg/day



Source: Unpublished Data Dec 2019

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Boards and Milestones

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Fortunate for the Insights of Leading KOLs & Drug Candidate Co-Founders

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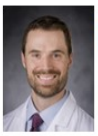
University of Minnesota

PHARMALOGIC

Gary Novak, PhD
UC Davis

Cleveland Clinic
Cole Eye Institute

Peter Kaiser, MD
Harvard Medical School



Duke Eye Center

Michael Allingham, MD, PhD
University of North Carolina

Retina-Vitreous Associates Medical Group

David Boyer, MD
Chicago Medical School

OPHTHALMIC CONSULTANTS OF BOSTON
EXCELLENCE IN EYE CARE

Jeffrey Heier, MD
Boston University

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Théa let's open our eyes
Shire
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2020 to 2022 Cadence of Milestones

Multiple Data Catalysts for Value-Building

2018/2019	1H 2020	2H 2020	1H 2021	2H 2021	2022
✓ NVD Podium Presentation at AAO 2018	✓ Completion of APX3330 License	✓ Announced Ocuphire Reverse Merger and PIPE Financing (Co-Led by Cantor and Canaccord)	✓ Enrollment of Phase 3 RM trial	❑ Enrollment of Phase 3 NVD trial	❑ Initiate Phase 3 Presbyopia Trials
✓ Initiate/Report Phase 2b Data for ORION-1	✓ ARVO 2020 Presentation for MIRA-1	✓ Completion of Transaction (Nasdaq: OCUP)	❑ Initiate Phase 2 Presbyopia Trial	❑ Report Phase 3 Data for NVD	❑ Report 2nd Phase 3 Data for RM & NVD
✓ Initiate/Report Phase 2b Data for MIRA-1	✓ ARVO 2020 Presentation for ORION-1	✓ Initiate Phase 3 RM Trial	❑ Initiate Phase 2 DR/DME Trial	❑ Enrollment of Phase 2 DR/DME trial	❑ Report Phase 3 Safety Data
✓ Expand Patent Estate	✓ FDA EOP2 Meeting May 2020	✓ Initiate Phase 3 NVD Trial	❑ Report Phase 3 Data for RM	❑ Report Phase 2 Data for DR/DME	❑ Report Phase 3 Data for Presbyopia
		✓ Complete Nyxol Market Research	❑ Enrollment of Phase 2 Presbyopia trial	❑ Report 6-month Rabbit Tox Study	❑ Initiate Phase 3 DR/DME Trial(s)
		✓ Journal Publications	❑ Report Phase 2 Data for Presbyopia	❑ Initiate 2nd Phase 3 Trials for RM & NVD	❑ Industry/Journal Publications
			❑ New Patent Claims	❑ Initiate Acute and Chronic Phase 3 Safety Trial	❑ Registration Batches for Nyxol Blow-Fill-Seal Eye Drops

Ongoing partnering discussions with leading ophthalmic companies (inc. European and Asian players)

Nyxol NDA filing for RM & NVD in early 2023

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Restore Vision & Clarity

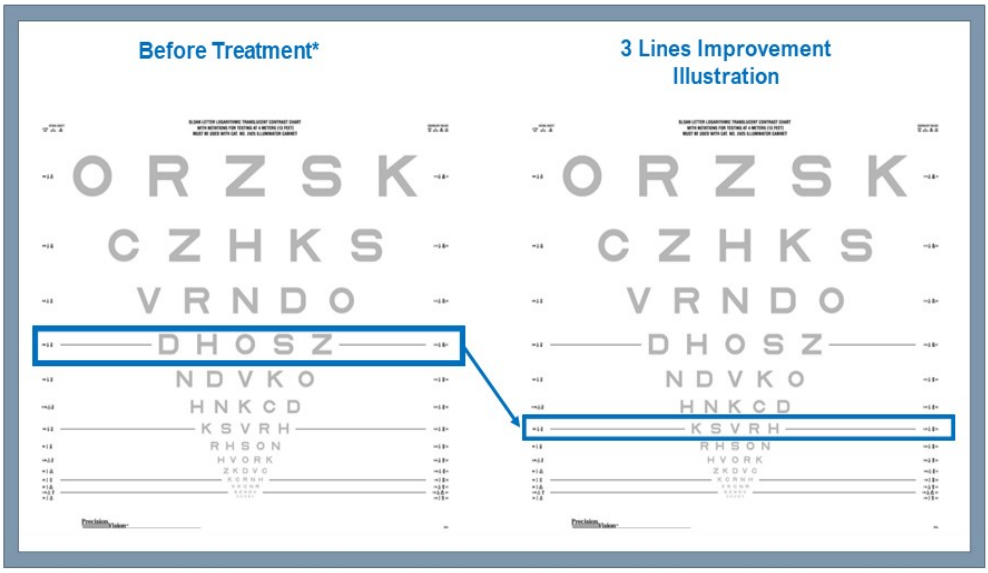
www.ocuphire.com
ir@ocuphire.com

NVD Endpoint: 5% Low Contrast Visual Acuity (LCVA) Chart

FDA Accepted Endpoint for Contrast Sensitivity Assessment

Primary Endpoint of Nyxol LYNX-1 Trial

Percent of subjects with ≥ 3 lines of improvement in mesopic low contrast best-corrected distance visual acuity (7 days)



** Inclusion Criteria includes subjects with baseline mesopic LCVA of 20/100 or worse*

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DR/DME Endpoint: Diabetic Retinopathy Severity Scale (DRSS)

FDA Accepted Endpoint for DR (EYLEA® in PANORAMA Pivotal Trial)

Primary Endpoint of APX3330 ZETA-1 Trial

Percent of patients with a ≥ 2 step improvement on the DRSS score at week 24

Patients included in the ZETA-1 Trial

DRSS Score	1 (10)	2 (20)	3 (35)	4 (43)	5, 6 (47, 53)	7 – 13 (60, 61, 65, 71, 75, 85,90)
Description	DR Absent	Micro-aneurysm only	Mild NPDR	Moderate NPDR	Moderately Severe NPDR	PDR – Mild, Moderate, and Severe
Retinal Image	 Healthy blood vessels with no bulges	 Small bulges in blood vessel walls as well as other signs in the retina	 More changes in the blood vessels in the retina and small spots of blood can become more visible	 More blood vessels in larger areas of the retina show changes	 Many of the blood vessels in the retina show visible changes	 Increased growth of new, damaged blood vessels

A 13-point Scale Outlining the Various Stages of Diabetic Retinopathy

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