Ocuphire Corporate Presentation
October 2022

Restore Vision & Clarity
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 the success and timing of planned regulatory filings, including planned NDA filings, pre-commercial activities, commercialization strategy and timelines, business strategy, product labels, cash runway, scalability, future clinical trials in reversal of mydriasis (RM), presbyopia (P), dim light/night vision disturbance (NVD) and diabetic retinopathy (DR)/diabetic macular edema (DME), including the potential for Nyxol to be a “best in class” presbyopia drop, and the potential market opportunity in RM/NVD/P/DR/DME. 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) the success, costs, and timing of regulatory submissions and pre-clinical and clinical trials, including enrollment and data readouts; (ii) regulatory requirements or developments; (iii) changes to clinical trial designs and regulatory pathways; (iv) changes in capital resource requirements; (v) risks related to the inability of Ocuphire to obtain sufficient additional capital to continue to advance its product candidates and its preclinical programs; (vi) legislative, regulatory, political and economic developments, (vii) changes in market opportunities, (viii) the effects of COVID-19 on clinical programs and business operations, (ix) the success and timing of commercialization of any of Ocuphire's product candidates, including the scalability of Ocuphire's product candidates and (x) the maintenance of Ocuphire's intellectual property rights. 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 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.
Upcoming Catalysts in 4Q22:

- Topline Results APX3330 ZETA-1 P2b trial for DR/DME
- NDA Filing for Nyxol for RM

Ocuphire Pharma
Nasdaq: OCUP

Founded in 2018, Acquired 2 Lead Assets for Front & Back of Eye Therapies with Novel MOAs & Patent Coverage to 2034+

- Nyxol eyedrops
  - *Reversal of Mydriasis* (“RM”) – eye dilation
  - *Presbyopia* – age-related blurry near vision
  - *Night Vision Disturbance* (“NVD”) – halos, glares, starbursts
- APX3330 oral tablets
  - *Diabetic retinopathy* (“DR”) – diabetes-related retinal (eye) disease

Four Large Markets (~$20B US total) w/Unmet Needs and Limited to No Competition

Successful Execution of 5 Trials in last 2 Years with 6 Positive Phase 3 & Phase 2 Data Read-outs for Nyxol in RM, Presbyopia, and NVD

- Potential 2023 commercialization opportunities in RM
- Near-term initiation planned for Presbyopia VEGA Phase 3 program with Nyxol alone and Nyxol with 0.4% Low Dose Pilocarpine as adjunctive therapy
## Ocuphure Overview

**Two Late-Stage Clinical Assets Addressing Unmet Needs in Multiple Large Markets**

### Refractive

<table>
<thead>
<tr>
<th>Nyxol</th>
<th>Novel α1/α2 Blocker 505(b)(2)</th>
<th>NDA-Filing Ready</th>
</tr>
</thead>
<tbody>
<tr>
<td>12</td>
<td>Completed Phase 1, Phase 2, and Phase 3 Trials</td>
<td>Exposure in Humans: 28 Days</td>
</tr>
<tr>
<td>&gt;650</td>
<td>Subjects Dosed</td>
<td></td>
</tr>
</tbody>
</table>

### Retina

<table>
<thead>
<tr>
<th>APX3330</th>
<th>Oral REF-1 Inhibitor New Chemical Entity</th>
<th>Phase 2b Data 4Q22</th>
</tr>
</thead>
<tbody>
<tr>
<td>11</td>
<td>Completed Phase 1 and Phase 2 Trials</td>
<td>Exposure in Humans: 365 Days</td>
</tr>
<tr>
<td>&gt;340</td>
<td>Subjects Dosed</td>
<td>Patent Coverage: 2034+</td>
</tr>
</tbody>
</table>

### Prevalence (US) & Development Milestone

<table>
<thead>
<tr>
<th>Condition</th>
<th>Prevalence</th>
<th>Milestone</th>
</tr>
</thead>
<tbody>
<tr>
<td>Reversal of Mydriasis</td>
<td>~100 M</td>
<td>2 Phase 3 Positive Data &amp; Ped P3</td>
</tr>
<tr>
<td>Presbyopia</td>
<td>~128 M</td>
<td>Phase 2 Positive Data Single &amp; Combo</td>
</tr>
<tr>
<td>Night Vision Disturbances</td>
<td>~36 M</td>
<td>1st Phase 3 Positive Data</td>
</tr>
<tr>
<td>Diabetic Retinopathy</td>
<td>~8 M</td>
<td>Phase 2b Last Patient Last Visit Completed Aug 22</td>
</tr>
<tr>
<td>Diabetic Macular Edema</td>
<td>~2.4 M</td>
<td></td>
</tr>
</tbody>
</table>

---

Source: Eisai and Apexian Data; GlobalData Market Research Report, 2020; Company Estimates for US Market Size; Ocuphure internal estimates
## Ocuphire Pipeline & Clinical Milestones

*Multiple Phase 3 & Phase 2 Clinical Data Readouts Anticipated this Year*

<table>
<thead>
<tr>
<th>Indication</th>
<th>Product Candidate</th>
<th>Pre-clinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Regulatory Approval</th>
<th>Anticipated Milestones</th>
</tr>
</thead>
<tbody>
<tr>
<td>Reversal of Mydriasis (RM)</td>
<td>Nyxo\textsuperscript{®} Eye Drop</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Positive MIRA-3 Phase 3 data in 1Q 2022 (n=368)</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Positive MIRA-4 Pediatric data in 2Q 2022 (n=23)</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>File Nyxol NDA for RM in 4Q 2022</td>
</tr>
<tr>
<td>Presbyopia (P)</td>
<td>Nyxo\textsuperscript{®} Eye Drop</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Positive VEGA-1 Nyxol alone data in 1Q 2022 (and in combination with LDP in mid-2021)</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>VEGA Phase 3 program planned to initiate in 2H 2022 for single agent and combination with LDP</td>
</tr>
<tr>
<td>Dim Light or Night Vision Disturbances (NVD)</td>
<td>Nyxo\textsuperscript{®} Eye Drop</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Positive LYNX-1 Phase 3 data in 2Q 2022 (n=145)</td>
</tr>
<tr>
<td>Diabetic Retinopathy (DR)/Macular Edema (DME)</td>
<td>APX3330 Oral Pill</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Phase 2b Fully Enrolled</td>
<td>ZETA-1 Phase 2b data expected in 4Q 2022 (n=103)</td>
</tr>
<tr>
<td>DME or Wet Age-Related Macular Degeneration (wAMD)</td>
<td>APX2009 (Intravitreal or Local Delivery)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Seeking partner funding for IND enabling studies and further development</td>
</tr>
</tbody>
</table>

*Note: 0.75% Nyxol (Phentolamine Ophthalmic Solution) is the same as 1% Nyxol (Phentolamine Mesylate Ophthalmic Solution)*
NYXOL®
EYE DROPS

THREE INDICATIONS

1. Reversal of Mydriasis
2. Presbyopia
3. Night Vision Disturbance
Nyxol’s Differentiated MOA as an Alpha-1 Blocker

No Engagement of Ciliary Muscle ➔ No Headaches and Lower Risk of Retinal Detachment

Phentolamine is the Active Ingredient in Nyxol: a non-selective $\alpha_1$ Antagonist

Phentolamine blocks $\alpha_1$ receptors on the Iris Dilator Muscle

- Decreases pupil size (moderately)
- without affecting the iris sphincter or ciliary muscles

- Allows for 3 indications: RM, Presbyopia and NVD

505(b)(2) Regulatory Pathway Supported by Phentolamine Approval for 2 Indications:

- Regitine® (Pheochromocytoma) – intravenous injection approved in 1952
- OraVerse® (Reversal of oral anesthesia) – intramuscular injection approved in 2008
# Nyxol Product Candidate Profile

*Novel, Differentiated Alpha-1 Blocker Eye Drop for Refractive Indications*

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

<table>
<thead>
<tr>
<th>Efficacy Data</th>
<th>Favorable Safety Profile</th>
<th>Durable</th>
</tr>
</thead>
</table>
| Nyxol Improves Vision by Decreasing Pupil (~1-1.5mm)  
↑ Near Vision  
↑ Distance Vision  
↑ Contrast Sensitivity (night) | No Systemic Effects  
No changes in Blood Pressure  
No changes in Heart Rate  
Well-Tolerated Topical Effects  
• Mild, transient, reversible Eye redness (11%)  
• Mild, instillation site discomfort (11%)  
IOP unchanged or decreased  
Minimal to No headaches | Effects Last ≥ 24 Hours  
Chronic daily dosing of Nyxol at bedtime reduces pupil size for up to 24 to 36 hours |
# Summary of Nyxol Trial Results

**Demonstrated Efficacy Response & Well Tolerated Safety Profile Across 3 Indications**

<table>
<thead>
<tr>
<th>Primary Endpoint</th>
<th>Efficacy Data</th>
<th>Key Secondary Endpoint(s)</th>
<th>Safety &amp; Tolerability</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>RM</strong></td>
<td>Return to baseline pupil diameter at 90 minutes after dilation</td>
<td>Met primary endpoint MIRA-3: 58% Nyxol vs. 6% placebo MIRA-2: 49% Nyxol vs. 7% placebo (p&lt;0.0001)</td>
<td>Efficacy across all mydriatic agents, iris color and 1 or 2 drops</td>
</tr>
<tr>
<td><strong>P: Nyxol</strong></td>
<td>≥3 line gain in near vision with loss of no more than 1 line in distance vision</td>
<td>Met planned Phase 3 primary endpoint VEGA-1: 29% Nyxol vs. 12% placebo at 12 hrs post-Nyxol dose (p=0.02)</td>
<td>Durable near vision (18 hrs) Optimal pupil size Pupillary light reflex</td>
</tr>
<tr>
<td><strong>P: Nyxol+LDP</strong></td>
<td></td>
<td>Met primary endpoint Met planned Phase 3 primary endpoint VEGA-1: 61% post-LDP dose + post-Nyxol dose (12 hrs) vs. 14% placebo at 30 mins (p&lt;0.0001)</td>
<td>Durable near vision gain Optimal pupil size Pupillary light reflex</td>
</tr>
<tr>
<td><strong>NVD</strong></td>
<td>≥3 lines (eye test) of improvement in mesopic low contrast best-corrected distance visual acuity (mLCVA)</td>
<td>Met primary endpoint LYNX-1: 13% Nyxol vs. 3% placebo at Day 8 (p=0.05) and 21% in Nyxol vs. 3% placebo at Day 15 (p&lt;0.01)</td>
<td>Improvement visual acuity measures (distance and near)</td>
</tr>
</tbody>
</table>

*Trend toward statistical significance even in smaller POS arm from time 0 to time 6 hours (n=30); larger sample size for all arms planned in Phase 3 program*
Mydriasis or dilated eyes causes:
- heightened sensitivity to light
- inability to focus
- reading, working, and driving difficulty
- halos and glare
- headaches

I have to visit my retina MD for my monthly injections, where I am dilated. Being dilated every month is a huge burden on my day.
Reversal of Mydriasis (RM) Market Opportunity

With No Commercially Available Treatment, Nyxol Could Achieve Meaningful Near-Term Revenues

Source: GlobalData Market Research Survey

Calculation: 100M Annual Eye Dilations X 65% X 80% X $10 per patient = $500+M Opportunity. Values Rounded, Not Exact.

ECP - Eyecare Practitioner

RM - Reversal of Mydriasis

~$500M+
Estimated US Reversal Mydriasis Market Opportunity

Cash Pay
Market Research
$10 to $20+

$100M-$200M
Nyxol Revenue Opportunity

No Competition

100M Annual Eye Dilations

MIRA Trials Represent 95M Dilation Drops Used in Practice

63M Report Moderate to Severe Impact to Daily Function

50M Patients Likely to Request Drop

Patient Willingness to Pay $10-$20+

$500M+
US RM Market Opportunity

Source: GlobalData Market Research Survey
Calculation: 100M Annual Eye Dilations X 65% X 80% X $10 per patient = $500+M Opportunity. Values Rounded, Not Exact. ECP - Eyecare Practitioner
Target Label Indication
The treatment of pharmacologically induced mydriasis produced by adrenergic (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents, or a combination thereof.
Primary Endpoint Achieved in Two FDA Registration Phase 3 Trials

Rapid, Consistent and Sustained Reversal of Pupil Dilation with Nyxol

MIRA-3 Phase 3 Trial

Percent of Subjects Returning to ≤ 0.2 mm of Baseline PD Study Eye (mITT)

<table>
<thead>
<tr>
<th>Time Post-Treatment with Nyxol/Placebo (Hours)</th>
<th>Placebo (%)</th>
<th>Nyxol (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0.5</td>
<td>4%</td>
<td>2%</td>
</tr>
<tr>
<td>1</td>
<td>6%</td>
<td>2%</td>
</tr>
<tr>
<td>1.5</td>
<td>14%</td>
<td>7%</td>
</tr>
<tr>
<td>2</td>
<td>17%</td>
<td>2%</td>
</tr>
<tr>
<td>3</td>
<td>36%</td>
<td>2%</td>
</tr>
<tr>
<td>4</td>
<td>86%</td>
<td>91%</td>
</tr>
<tr>
<td>6</td>
<td>89%</td>
<td>72%</td>
</tr>
<tr>
<td>24</td>
<td>1%</td>
<td>0%</td>
</tr>
</tbody>
</table>

MIRA-2 Phase 3 Trial

Percent of Subjects Returning to ≤ 0.2 mm of Baseline PD Study Eye (mITT)

<table>
<thead>
<tr>
<th>Time Post-Treatment with Nyxol/Placebo (Hours)</th>
<th>Placebo (%)</th>
<th>Nyxol (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0.5</td>
<td>3%</td>
<td>2%</td>
</tr>
<tr>
<td>1</td>
<td>2%</td>
<td>7%</td>
</tr>
<tr>
<td>1.5</td>
<td>11%</td>
<td>7%</td>
</tr>
<tr>
<td>2</td>
<td>18%</td>
<td>2%</td>
</tr>
<tr>
<td>3</td>
<td>30%</td>
<td>14%</td>
</tr>
<tr>
<td>4</td>
<td>45%</td>
<td>59%</td>
</tr>
<tr>
<td>6</td>
<td>66%</td>
<td>90%</td>
</tr>
<tr>
<td>24</td>
<td>89%</td>
<td>92%</td>
</tr>
</tbody>
</table>

Source: (Left panel) MIRA-3 Table 14.2.1.1 (mITT); (Right panel) MIRA-2 Table 14.2.1.1 (mITT). Data include all three mydriatics (Phenylephrine, Tropicamide, Paremyd).
Commercial Strategy With Cash Pay Model

*Simple, Value-Driven, Capital-Efficient and Accelerates Adoption*

- Preservative-Free Single Unit Vial (5-pack)
- Strategic and/or Distribution Partners
- Sell Direct to Office
- Physician Sells to Patient

### Low Adoption Hurdles:
- No reimbursement or payors/PBMs
- No training, dilations are routine
- No specialty/retail pharmacies
- No competition

*58% of physicians would start prescribing Nyxol within 1st year*

Source: GlobalData Market Research Survey
NYXOL®
FOR
PRESBYOPIA

- Nyxol Alone
- Nyxol with LDP Adjunctive Therapy

88% of patients expressed an interest in presbyopia-correcting drops across all ages and income groups

FDA approval of AbbVie Eye Drop a New Moment in Presbyopia
10/29/21
Presbyopia is a Large, Attractive Market Opportunity

*Vuity™ (Allergan) Approval Sets Stage for Growth of New Category by Global Pharma*

~128M Presbyopes in the US

<table>
<thead>
<tr>
<th>Condition</th>
<th>Number of Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Emmetropes</td>
<td>66M</td>
</tr>
<tr>
<td>Myopes</td>
<td>30M</td>
</tr>
<tr>
<td>Hyperopes</td>
<td>9M</td>
</tr>
<tr>
<td>Pseudophakes</td>
<td>14M</td>
</tr>
<tr>
<td>Low (&lt; -6.0D)</td>
<td>9M</td>
</tr>
<tr>
<td>High (&gt; -6.0D)</td>
<td>9M</td>
</tr>
</tbody>
</table>

Addressable Market (25%)

<table>
<thead>
<tr>
<th>Condition</th>
<th>Number of Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Emmetropes (post-Lasik)</td>
<td>17M</td>
</tr>
<tr>
<td>Myopes Low</td>
<td>8M</td>
</tr>
<tr>
<td>Myopes High</td>
<td>2M</td>
</tr>
<tr>
<td>Hyperopes Low</td>
<td>4M</td>
</tr>
<tr>
<td>Hyperopes High</td>
<td>2M</td>
</tr>
</tbody>
</table>

US Market Opportunity

33M Patients

~$8B+*

Market Revenues

* Assumes 3 refills per annum

Sources:
3. NEI/NIH [https://www.nei.nih.gov/sites/default/files/health-pdfs/Presbyopia.pdf](https://www.nei.nih.gov/sites/default/files/health-pdfs/Presbyopia.pdf) Values in Figure Rounded.

~$8B+

Estimated US Presbyopia Market Opportunity

Cash Pay

Vuity™ Price $79

$500 M+

Nyxol Revenue Opportunity

(Additional text not shown in the image)
Nyxol® and Nyxol + Low Dose Pilocarpine Presbyopia Eye Drops

Differentiated MOA with Two Potential Product Labels for Functional Near Vision Improvement

0.75% Nyxol

- Iris Dilator Muscle Inhibition

Iris Sphincter and Ciliary Muscles Activation

0.4% LDP

Nyxol with LDP as Adjunctive Therapy for Presbyopia

Two Drops Tunable Option

Nyxol as a Single Agent for Presbyopia

Single Durable Drop

Evening drop

Daytime drop

Optimal Pupil Target is 2-3 mm

Source: Nyxol® data from 12 completed trials; Pilocarpine product label and literature
Presbyopia Eye Drops Competitive Landscape

* Nyxol alone potential differentiation:
  1) New MOA class (iris dilator muscle)
  2) Favorable safety and tolerability (e.g.: no headaches, no accommodative spasm, no risk of retinal detachment)
  3) 24-hour durability
  4) Broad range of patients including high myopes
  5) Improvement in night vision disturbances

* Nyxol+LDP may offer added efficacy and tunability

- **Phase 1**
  - Orasis CSF-1 (Low dose pilo)
- **Phase 2**
  - Visus Brimochol® (carbachol + brim)
- **Phase 3**
  - Orasis CSF-1 (Low dose pilo)
  - Allergan VUITY™: 1.25% pilo
  - Eyenovia MicroLine (2% pilo)
  - Ocuphire Nyxol + 0.4% pilo
  - Ocuphire Nyxol (0.75% phentolamine)
  - Novartis EV-06 / Dioptin

- **Other Cholinergic Agonists***
  - Lenz Aceclidine; Aceclidine + brim
  - Lenz Aceclidine; Aceclidine + brim
  - Lenz Aceclidine; Aceclidine + brim

- **Alpha Antagonist & low dose pilocarpine***
  - Ocuphire Nyxol + 0.4% pilo
  - Ocuphire Nyxol (0.75% phentolamine)

- **Alpha Antagonist**
  - Ocuphire Nyxol (0.75% phentolamine)
  - Ocuphire Nyxol + 0.4% pilo

- **Lens Softening**
  - Novartis EV-06 / Dioptin

- **Cholinergic Agonist* (pilocarpine)**
  - Allergan VUITY™: 1.25% pilo
  - Eyenovia MicroLine (2% pilo)

* act on sphincter and ciliary muscles in dose-dependent manner

Corporate Websites, Grzybowski, A, Markeviciute A, Zemaitiene R. A Review of Pharmacological Presbyopia Treatment. 2020
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, Age 42
Market Opportunity in Dim Light or Night Vision Disturbances

No Currently Approved Treatments in Development with Opportunity for Growth

US NVD Population ~38 M

<table>
<thead>
<tr>
<th>Number of Patients</th>
<th>Night Myopia</th>
<th>Cortical Cataracts</th>
<th>Post-Lasik</th>
<th>Post-IOL Implant</th>
</tr>
</thead>
<tbody>
<tr>
<td>26M</td>
<td>9M</td>
<td>1M</td>
<td>2M</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Moderate to Severe NVD</th>
<th>11M</th>
<th>4M</th>
<th>0.5M</th>
<th>0.3M</th>
</tr>
</thead>
<tbody>
<tr>
<td>Addressable Market (60%)</td>
<td>7M</td>
<td>2M</td>
<td>0.3M</td>
<td>0.2M</td>
</tr>
</tbody>
</table>

~10 M Patients

<table>
<thead>
<tr>
<th>US Market Opportunity*</th>
<th>~$2 B+</th>
</tr>
</thead>
<tbody>
<tr>
<td>* Assumes 3 refills per annum</td>
<td>Market Revenues</td>
</tr>
</tbody>
</table>

~$2B+
Estimated US DLD Market Opportunity

Cash Pay
Pricing similar to Presbyopia

$300+ M
Nyxol Revenue Opportunity

No Approved Treatments
No Competition

GlobalData Market Research Report, 2020; Revenue potential based currently on Vuity Presbyopia pricing
APX3330

ORAL TABLET

Diabetic Retinopathy

Diabetic Macular Edema

"I could lose my hearing, I could lose talking, but ... it's frightening to lose my eyesight."

Patient Diagnosed with DR
Diabetic Retinopathy At a Glance
Larger Disease to Manage with Growing Diabetes

There are **8M** adults in the U.S. with DR\(^1\)

**The number of people with DR expected to increase more than **
**14M** by 2050

**DR/DME affects about **
**1 in 4** people with type 1 and type 2 diabetes

**DR is the leading cause of blindness among working-age adults**

If untreated, DR can **rob people of their vision** prematurely\(^2,\(^3\)

**56%** of patients reported anxiety related to anti-VEGF treatment

**$13B** (2020)
Global Intravitreal Injection Revenues

Majority of mild to moderate patients with DR are **not treated with anti-VEGF due to injection fear and burden**

Source:
1. American Diabetes Association; International Diabetes Federation; Healthline;
3. Patient survey adapted from Lions International Foundation and International Diabetes Foundation-Europe; Meltzer 2000
Broad Opportunities to Treat Retinal Diseases with APX Platform

APX3330 May Treat Patients Across Retinal Diseases as Single Agent or Adjunctive Therapy

34 Million Diabetics in US

<table>
<thead>
<tr>
<th>Condition</th>
<th>APX3330</th>
<th>Anti-VEGF treatments</th>
</tr>
</thead>
<tbody>
<tr>
<td>Diabetic Retinopathy</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mild NPDR</td>
<td>~8M+</td>
<td></td>
</tr>
<tr>
<td>Moderate to Severe NPDR (DRSS 43-53)</td>
<td>1M</td>
<td></td>
</tr>
<tr>
<td>PDR (DRSS &gt;60)</td>
<td>1M</td>
<td></td>
</tr>
</tbody>
</table>

Addressable Market

US Market Opportunity

~$10B+ Market Revenues

APX3330

APX2009 / APX2014 / APX3330 (Local Delivery)

Inflammatory component is common across these retina indications as well & potentially addressable by MOA of Ref-1

Potential First Oral Rx for Retina Diseases with Multi-Billion Revenue Opportunity

Source:
1. American Diabetes Association; International Diabetes Federation; Healthline; *Ocuphire internal analysis and assumptions;
3. Patient survey adapted from Lions International Foundation and International Diabetes Foundation-Europe; Meltzer 2000
4. Estimates are provided by the National Eye Institute, FactSheet, Global Data, and Research and Markets. Estimated values are rounded.
5. Estimated prevalence in the U.S.; DME- Diabetic Macular Edema; Age-related Macular Degeneration; Geographic Atrophy; Retinal Vein Occlusion

DR

DME

DME

Wet AMD

Dry AMD

GA

RVO
APX3330 History and Ref-1 Inhibition Mechanism

Inhibition Mechanism

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

Ref-1 (reduction-oxidation effector factor-1) is a novel target discovered by Dr. Mark R. Kelley at Indiana University School of Medicine

APX3330 is a small molecule oral drug candidate and a first-in-class inhibitor of Ref-1

APX3330 previously developed by Eisai for multiple hepatic inflammatory indications and later by Apexian for advanced solid tumors in 11 Phase 1 and 2 trials

– Similar oncology origin as approved anti-VEGFs

MOA uniquely decreases both abnormal angiogenesis and inflammation by blocking pathways downstream of Ref-1

Extensively studied in over 20 in-vitro and animal studies with favorable efficacy and safety
APX3330 Product Candidate Profile for Multiple Retinal Indications

**Oral, First-In-Class Ref-1 Inhibitor with Favorable Human Safety Data**

### Expected Efficacy Data
- Novel MOA for treating retina
  - ↓ Inflammation
  - ↓ Abnormal Angiogenesis
- Convenient Oral Dosing for Patient Compliance
- Allow Daily vs. Episodic Exposure
  - Oral pill may reduce the burden of frequent anti-VEGF injections

### Favorable Safety Profile
- ~10,000 Subject-exposure days* at ≥ 600 mg/day dose
- Few Systemic Adverse Effects
  - ~ 5% Mild Diarrhea
  - ~ 5% Mild Skin Rash (reversible)
- No Treatment-Related Organ Toxicity
  - (Liver, Cardiovascular {BP, HR}, Kidney, Neurologic, Pulmonary)
- No Ocular Effects
  - No observed ocular AEs

---

*11 completed Phase 1 and Phase 2 clinical trials by Eisai and Apexian; along with ongoing ZETA-1 trial by Ocuhire (*includes ~103 subject)
Ongoing, Randomized, Double-Masked, Placebo-Controlled 24-Week Trial (Similar To Eylea P3 DR Trial)

ZETA-1

25 US sites

90-100 participants with moderately severe-to-severe NPDR or mild PDR

Noncentral DME is permitted

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)
- DRSS change at week 12
- Rescue subjects
- Safety and tolerability

Exploratory:
- Labs / PK

APX3330 600mg/day (BID)

103 Subjects Enrolled; Top Line Expected in 4Q 2022

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)

ZETA-1 Clinical Trial is Sponsored by Ocuphire Pharma https://clinicaltrials.gov/ct2/show/NCT04692688?term=ZETA-1&draw=2&rank=1
Masked Safety Findings from Ongoing ZETA-1 Trial

**Favorable Safety Profile (as of 9/15/2022)** Observed with 600 mg Oral Daily Doses in Diabetic Subjects

<table>
<thead>
<tr>
<th>Subject Details</th>
<th>TEAE Distribution</th>
<th>SAE Distribution</th>
</tr>
</thead>
<tbody>
<tr>
<td>103 Subjects Enrolled</td>
<td>169 TEAEs in 62/103 Subjects (60%)</td>
<td>0 Treatment-Related SAEs involving liver, heart, kidney, brain, lung, or vital signs</td>
</tr>
<tr>
<td>95 Subjects completed thru week 12</td>
<td>30 Treatment-Related</td>
<td>169 TEAEs in 12/103 Subjects</td>
</tr>
<tr>
<td>91 Subjects completed thru week 24</td>
<td>149 Unrelated</td>
<td>0 Treatment-Related</td>
</tr>
<tr>
<td>3 withdrew due to an AE³</td>
<td>6 lost to follow-up</td>
<td>16 Unrelated²</td>
</tr>
<tr>
<td>18 Mild</td>
<td>92 Mild</td>
<td>0 Withdrawed due to an AE³</td>
</tr>
<tr>
<td>12 Moderate¹</td>
<td>48 Moderate</td>
<td>6 Lost to follow-up</td>
</tr>
<tr>
<td>0 Severe</td>
<td>9 Severe</td>
<td>3 Withdrawed consent or site closure</td>
</tr>
<tr>
<td>16 SAEs in 12/103 Subjects</td>
<td>0 Treatment Related</td>
<td>16 Unrelated²</td>
</tr>
<tr>
<td>12 events in 8 subjects: diarrhea, worsening DME (OD and OS), pruritis, urticaria, blurry vision, decrease in hemoglobin level, ischemic diabetic maculopathy and central vision scotoma (in same subject), photophobia (OD and OS) and hypoesthesia (in same subject)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2. Cellulitis (2 events in same subject), dyskinesia, transient ischemic event, COVID-19 and acute respiratory failure (same subject), progression of multivessel coronary artery disease, cholelithiasis, osteomyelitis, vertigo, chest pain, infection of toe and ulcer of toe and embolism (3 events in same subject), multi-system organ failure, worsening bradycardia</td>
<td></td>
<td></td>
</tr>
<tr>
<td>3. DME, Dyspnea, Pre-Syncope.</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Note: ZETA-1 Interim Data as of database 9/15/22 with monitoring to be completed before final database lock; assumes 50% subjects on APX3330 Exposure

Oral APX3330 safety profile consistent with that seen in prior trials
Milestones

Ophthalmic Pharma Trends

Investment Summary
**Track Record of Achieving Milestones ➔ Exciting News Cadence**

*Multiple Positive Data Readouts in 2021-1H 2022 with Multiple Catalyst Ahead*

### 2021 – 1H 2022

- **Positive** Phase 3 Data for RM (MIRA-2)
- **Positive** Nyxol+LDP Phase 2 Data for Presbyopia (VEGA-1)
- **Positive** Nyxol Alone Phase 2 Data for Presbyopia
- **Positive** 2\(^{nd}\) Phase 3 Data for RM (MIRA-3)
- **Positive** Pediatric Data for RM (MIRA-4)
- **Positive** Phase 3 Data for NVD (LYNX-1)

### 2H 2022 – 2023

- Submit Nyxol NDA for RM
- Report Phase 2b Data for DR/DME (ZETA-1)
- Initiate VEGA-2 & VEGA-3 Phase 3 Presbyopia Trials
- Initiate LYRA-1 Long-term Safety Trial
- Potential NDA Approval for RM
- Establish Commercial Partnership
- Report VEGA-2 Presbyopia Data

**Ongoing Partnering Discussions with Leading Ophthalmic Companies (including Europe and Asia)**
# Ophthalmology is Attractive Biotechnology Sector

**Active Partnering and Constructive FDA Ophthalmology Division**

## Deal Activity

<table>
<thead>
<tr>
<th>Date</th>
<th>Partnering</th>
<th>Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>April 2021</td>
<td>Novartis / Alcon</td>
<td>$355 M</td>
</tr>
<tr>
<td>September 2021</td>
<td>REGENXBIO / Allergan</td>
<td>$1.75 B</td>
</tr>
<tr>
<td>October 2021</td>
<td>Théa / Curacle</td>
<td>~$2 B</td>
</tr>
<tr>
<td>December 2021</td>
<td>Rayner / Omidria</td>
<td>~$1 B</td>
</tr>
<tr>
<td>December 2021</td>
<td>Novartis / Gyroscope</td>
<td>~$1.5B</td>
</tr>
<tr>
<td>January 2022</td>
<td>Théa / Akorn</td>
<td>Undisclosed</td>
</tr>
<tr>
<td>May 2022</td>
<td>Alcon / Kala</td>
<td>$385 M</td>
</tr>
<tr>
<td>August 2022</td>
<td>Alcon / Aerie</td>
<td>$770 M</td>
</tr>
</tbody>
</table>

## New Product Approvals

<table>
<thead>
<tr>
<th>Company</th>
<th>Product</th>
</tr>
</thead>
<tbody>
<tr>
<td>Allergan</td>
<td>Vuity™</td>
</tr>
<tr>
<td>Oyster Point</td>
<td>Tyrvaya™</td>
</tr>
<tr>
<td>Bausch Health</td>
<td>Xipere™</td>
</tr>
<tr>
<td>Roche</td>
<td>Susvimo™</td>
</tr>
<tr>
<td>Novartis</td>
<td>Beovu™</td>
</tr>
<tr>
<td>Roche</td>
<td>Vabysmo™</td>
</tr>
<tr>
<td>Ocular Therapeutics</td>
<td>Dextenza™</td>
</tr>
<tr>
<td>Santen</td>
<td>Verkazia™</td>
</tr>
<tr>
<td>Samsung Bioepis</td>
<td>Byooviz™</td>
</tr>
<tr>
<td>Coherus</td>
<td>Cimerli™</td>
</tr>
</tbody>
</table>

### Aging Population
- Low Cost, Quick Enrollment, Shorter Duration Trials
- Favorable Regulatory Environment

---

30

---

**Active Partnering in 2021**
Ocuphire Pharma (OCUP)

Stock Price¹  ~$2-2.40
Shares Out.²  20.1M
Market Cap¹  $40-50M
Avg. Daily 90k Shares
Volume
Cash²  $17M
Cash Runway 12 months +
1 As of September 2022
2 End of 2Q22 (10-Q)

Two Lead Assets for Front & Back of Eye Therapies w/Novel MOAs & Patent Coverage to 2034+
- Nyxol eye drops
  - Reversal of Mydriasis (“RM”) – eye dilation
  - Presbyopia – age-related blurry near vision
  - Night Vision Disturbance (“NVD”) – halos, glares, starbursts
- APX3330 oral tablets
  - Diabetic retinopathy (“DR”) – diabetes-related retinal (eye) disease

Four Large Markets (~$20B US total) w/Unmet Needs, Limited to No Competition

Nyxol NDA Filing Planned in Q4 2022 for RM, Successful Execution of 5 Late-Stage Trials w/Positive Results
- RM 2023 commercialization potential (~$500M US market)

Topline Results Expected in Q4 2022 for APX3330 in Retinal Phase 2b Trial

Analyst Coverage:
- John Newman, Canaccord Genuity
- Kristen Kluska, Cantor Fitzgerald
- James Molloy, Alliance Global Part.
- Matthew Caufield, H.C. Wainwright
- Sean Kim, Jones Trading
Appendix
Ocuphire Management Team

Decades of Biotech and Drug Development Experience

Charlie Hoffmann, MBA
VP, Corporate Development and Operations

Mina Sooch, MBA
President & CEO and Founder

Amy Rabourn, CPA
VP, Finance

Ronil Patel, MS
VP, Business Development and Market Strategy

Daniela Oniciu, PhD
Global Head, R&D, Chemistry and Product Development

Mitch Brigell, PhD
Head, Clinical Development and Strategy

Barbara Withers, PhD
VP, Clinical and Regulatory Strategy

Bindu Manne
Head, Market Development and Commercialization

Chris Ernst
Global Head, QA and Manufacturing

Laura Gambino
Director, Project Management

Drey Coleman
VP, Clinical Operations

Charlie Hoffmann, MBA
VP, Corporate Development and Operations

Mina Sooch, MBA
President & CEO and Founder

Ronil Patel, MS
VP, Business Development and Market Strategy

Daniela Oniciu, PhD
Global Head, R&D, Chemistry and Product Development

Mitch Brigell, PhD
Head, Clinical Development and Strategy

Bindu Manne
Head, Market Development and Commercialization

Chris Ernst
Global Head, QA and Manufacturing

Laura Gambino
Director, Project Management

Drey Coleman
VP, Clinical Operations

Charlie Hoffmann, MBA
VP, Corporate Development and Operations

Mina Sooch, MBA
President & CEO and Founder

Ronil Patel, MS
VP, Business Development and Market Strategy

Daniela Oniciu, PhD
Global Head, R&D, Chemistry and Product Development

Mitch Brigell, PhD
Head, Clinical Development and Strategy

Bindu Manne
Head, Market Development and Commercialization

Chris Ernst
Global Head, QA and Manufacturing

Laura Gambino
Director, Project Management

Drey Coleman
VP, Clinical Operations
Ocuphire Board of Directors

*Seasoned Directors with Decades of Drug Development, M&A/Financings, and Ophthalmology*

- Cam Gallagher, MBA
  Chair, Board Director

- Mina Sooch, MBA
  Vice-Chair, Board Director
  President & CEO

- Sean Ainsworth, MBA
  Lead Independent Director,
  Board Director

- James Manuso, PhD/MBA
  Board Director

- Richard Rodgers, MBA
  Board Director

- Jay Pepose, MD, PhD
  Board Director

- Susan Benton, MBA
  Board Director
Ocuphire's World-Class Medical Advisory Board

Fortunate for the Insights of Leading KOLs & Drug Candidate Co-Founders (Ant. Seg | Retina)

- **Refractive Specialist**
  - Jay Pepose, MD, PhD
    - UCLA School of Medicine

- **Refractive Specialist**
  - Mitch Jackson, MD
    - University of Chicago

- **Refractive Specialist**
  - Marguerite McDonald, MD, PhD
    - Columbia University

- **Optometry**
  - Paul Karpecki, OD
    - Indiana University

- **Optometry**
  - Douglas Devries, OD
    - University of Nevada

- **Refractive Specialist**
  - Y. Ralph Chu, MD
    - Northwestern University

- **Refractive Specialist**
  - James Katz, MD
    - University of Illinois

- **Glaucoma Specialist**
  - Thomas Samuelson, MD
    - University of Minnesota

- **Refractive Specialist**
  - Michael Allingham, MD, PhD
    - University of North Carolina

- **Retinal Specialist**
  - Peter Kaiser, MD
    - Harvard Medical School

- **Retinal Specialist**
  - David Boyer, MD
    - Chicago Medical School

- **Retinal Specialist**
  - David Brown, MD
    - Baylor University

- **Retinal Specialist**
  - David Lally, MD
    - Vanderbilt University

- **Retinal Specialist**
  - Jeffrey Heier, MD
    - Boston University

- **Retinal Specialist**
  - Michael Allingham, MD, PhD
    - University of North Carolina

- **Retinal Specialist**
  - David Lally, MD
    - Vanderbilt University

- **Retinal Specialist**
  - Eliot Lazar, MD
    - Georgetown University

- **Retinal Specialist**
  - Mark Kelley, PhD
    - Indiana University Co-Founder Apexian/APX3330

- **Retinal Specialist**
  - David Boyer, MD
    - Chicago Medical School

- **Retinal Specialist**
  - David Brown, MD
    - Baylor University

- **Retinal Specialist**
  - David Lally, MD
    - Vanderbilt University
# Summary of MIRA FDA Registration Trial Designs

Randomized, Double-_masked, Placebo-Controlled, Parallel, Multi-Center, One-Day Trials

<table>
<thead>
<tr>
<th></th>
<th>MIRA-2 1st Phase 3</th>
<th>MIRA-3 2nd Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>US Sites</td>
<td>12</td>
<td>16</td>
</tr>
<tr>
<td>Subjects Enrolled</td>
<td>185</td>
<td>368</td>
</tr>
<tr>
<td>Eligibility</td>
<td>Healthy ≥ 12 years old</td>
<td>Healthy ≥ 12 years old</td>
</tr>
<tr>
<td>Randomization</td>
<td>1:1</td>
<td>2:1</td>
</tr>
<tr>
<td>Mydriatic Agents</td>
<td>2.5% Phenylephrine, 0.5% Tropicamide and Paremyd</td>
<td>2.5% Phenylephrine, 0.5% Tropicamide and Paremyd</td>
</tr>
<tr>
<td>Positive Data Readout</td>
<td>1Q 2021</td>
<td>1Q 2022</td>
</tr>
</tbody>
</table>

**Primary Endpoint**
- % of subjects (study eye) returning to baseline (within 0.2 mm) pupil diameter (PD) at 90 min

**Secondary Endpoints**
- % of subjects returning to baseline PD at 0min to 24h (overall, by mydriatic agent, by iris color)
- Mean time to return to baseline PD
- Mean change in pupil diameter at all timepoints
- Distance-corrected near vision
- Accommodation (Tropicamide/Paremyd)
- Safety and tolerability

**Total Subjects Enrolled**
- >550

**Total Exposure To Nyxol**
- >330

Over 300 subjects have been treated with Nyxol and evaluated at 24-hours in the MIRA trials → satisfying regulatory requirements for drug safety exposure for the acute RM indication.
Planned NDA Package Incorporates Positive Data from MIRA Trials
Nyxol Significantly Reduced PD in Subjects Ages 3 & Over with Favorable Safety and Tolerability

- Pivotal trials met primary endpoint of return to baseline PD at 90 minutes after dilation
  - MIRA-3 Phase 3 (58% Nyxol vs. 6% placebo, p<0.0001)
  - MIRA-2 Phase 3 (49% Nyxol vs 7% placebo; p<0.0001)
- MIRA-4 pediatric (age 3-17) trial 64% Nyxol vs. 25% placebo
- Met key endpoints with high statistical significance in MIRA-2 & MIRA-3
  - Efficacy across all 3 mydriatic agents – phenylephrine, tropicamide, and Paremyd®
  - Efficacy in both light and dark iris colors
  - Efficacy with 1 or 2 drops
  - Over 60% subjects returned to baseline accommodation at 2-3 hours
  - Accelerated return to normal distance-corrected near visual acuity

Efficacy

- No deaths, serious AEs, or withdrawals due to AEs
- All treatment related AEs were mild in severity
- The only AE occurring in ≥ 5% of subjects treated with Nyxol was mild and transient conjunctival hyperemia and instillation site discomfort (11% Nyxol vs. 0% placebo)
- No distance visual acuity loss
- No change in vital signs
- Completion of MIRA-4 study satisfies Pediatric Research Equity Act (PREA) requirement

Safety
Presbyopia VEGA-1 Phase 2 Trial

*Completed Randomized, Double-Masked, Placebo-Controlled, Multi-Center One-Week Trial*

**VEGA-1**

- 17 US sites
- 150 presbyopic patients

**Endpoints**

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

**Secondary:**
- Primary endpoint for Nyxol alone vs placebo
- % of subjects with \( \geq 2 \) and \( \geq 3 \) lines gained at time points from 30 min to 6 hours
- % of subjects achieving 20/40 or better vision
- No loss of distance vision
- Pupil diameter at time points
- Safety and tolerability (redness)

**Eligibility Criteria**

- Males or females \( \geq 40 \) and \( \leq 64 \) years of age
- DCNVA of 20/50 Snellen equivalent or worse in photopic conditions in each eye & binocularly
- BCDVA of 20/20 Snellen equivalent or better in each eye under photopic conditions
- No limitation on axial length or diopters for myopia patients

**Phase 2 Enrollment Completed Feb to May 2021 – 150 Subjects Reported Topline Results in June 2021 and Jan 2022**
Summary of Positive VEGA-1 Phase 2 Results

*Nyxol and Nyxol + LDP has Demonstrated Efficacy Response & Well Tolerated Safety Profile*

<table>
<thead>
<tr>
<th>Efficacy</th>
<th>Durability*</th>
<th>Functional Vision (20/40 or better)</th>
<th>Sustained Reduction in Pupil Diameter</th>
<th>Benefit in a Breadth of Patients</th>
<th>Safety &amp; Tolerability</th>
</tr>
</thead>
<tbody>
<tr>
<td>Met planned P3 endpoint at 12 hours post-Nyxol (29%; p=0.02)</td>
<td>Durable near vision gain through 18 hours</td>
<td>56% at 12 hours post-Nyxol</td>
<td>18+ hours (post-dose of Nyxol)</td>
<td>Ages 40-64</td>
<td>No headaches, No myopia exclusions, Light and dark irides</td>
</tr>
<tr>
<td>Met primary endpoint at 1 hour post-LDP (60%; p=0.004)</td>
<td>Durable near vision gain through 18 hours with enhanced near vision gain for at least 6 hours</td>
<td>84% at 13 hours post-Nyxol and 1 hour post-LDP</td>
<td>18+ hours (post-dose of Nyxol)</td>
<td>Ages 40-64</td>
<td>Light and dark irides</td>
</tr>
</tbody>
</table>

*Trend toward statistical significance even in smaller Nyxol arm from time 0 to time 6 hours (n=30); larger sample size for all arms planned in Phase 3 program*
VEGA-1: Planned P3 Efficacy Endpoint Met by Nyxol and Nyxol+LDP

Nyxol Single Drop and LDP Combination Provide Statistically Significant 3-line Near Vision Gain

1. Nyxol as a Single Drop for Presbyopia

Percent of Subjects with ≥15 Letter Gain In Near & <5 Letters Loss In Distance Vision in Photopic Binocular DCNVA
Time 0=12 Hours Post-Nyxol Dose at Visit 2

- Placebo (n=74): 12%
- Nyxol (n=73): 29%

p=0.02

53% of subjects achieved ≥10 letter improvement in DCNVA at 12 hours (p=0.005 vs placebo) and a similar trend at other time points

2. Nyxol with LDP Adjunctive Therapy

Percent of Subjects with ≥15 Letter Gain In Near & <5 Letters Loss In Distance Vision in Photopic Binocular DCNVA
Time 30 Minutes at Visit 2

- Placebo (n=43): 14%
- Nyxol+LDP (n=43): 61%
- Nyxol (n=30): 33%
- LDP (n=31): 26%

p<0.0001, p=0.03, p=0.008

79% of subjects achieved ≥10 letter improvement in DCNVA at 1 Hour (p=0.005 vs placebo) and a similar trend at other time points

Nyxol+LDP is statistically superior to Nyxol alone and LDP alone

VEGA TLR Table 14.2.2.2.1; Table 14.2.2.2; Table 14.2.1.7; Table 14.2.1.2
NVD LYNX-1 Phase 3 Registration Design

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

**LYNX-1**

- 19 US sites
- 140 - 160 patients with NVD

Eligibility Screening* → Randomization → 1:1

**Endpoints**

**Primary:** % of subjects with \( \geq 3 \) 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)

**Phase 3 Initiated in Dec 2020; 145 Patients Enrolled**

Top Line Results Reported May 19, 2022

*Inclusion Criteria includes subjects with baseline mesopic LCVA of 20/63 or worse
Summary of Positive LYNX-1 Phase 3 Results For Nyxol Eye Drops

Data Support a Favorable Benefit/Risk Profile For Subjects with NVD

Efficacy

- Met primary endpoint at Day 8 with 13% of subjects gaining 15 or more ETDRS letters of mesopic low contrast distance visual acuity vs. 3% on placebo (p<0.05)
- Nyxol's 3 line efficacy increased after 14 days of evening dosing, with 21% responders compared to 3% on placebo (p<0.01)
- Nyxol statistically significantly reduced pupil diameter by a mean of ~1 mm on Day 8 and Day 15
- Significant improvements in low contrast distance vision under photopic conditions were also observed
- Nyxol demonstrated benefit in mesopic high contrast near vision

Safety

- No deaths or serious AEs
- AEs occurring in >5% of Nyxol treated subjects included: instillation site irritation (9% vs 0% placebo), installation site pain (13% vs 0% placebo), dysgeusia (11% vs 0% placebo) and conjunctival hyperemia (9% vs 3% placebo)
- 84% of the AEs considered related to Nyxol were mild
- No statistical difference in conjunctival hyperemia between treatment arms with evening dosing at Day 8 and Day 15

Source: mITT Population, LYNX-1 Trial