

(NASDAQ: OCUL)

TRANSFORMING DRUG DELIVERY

LEVERAGING A NOVEL TECHNOLOGY PLATFORM

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Cowen Healthcare Conference
March 3, 2020



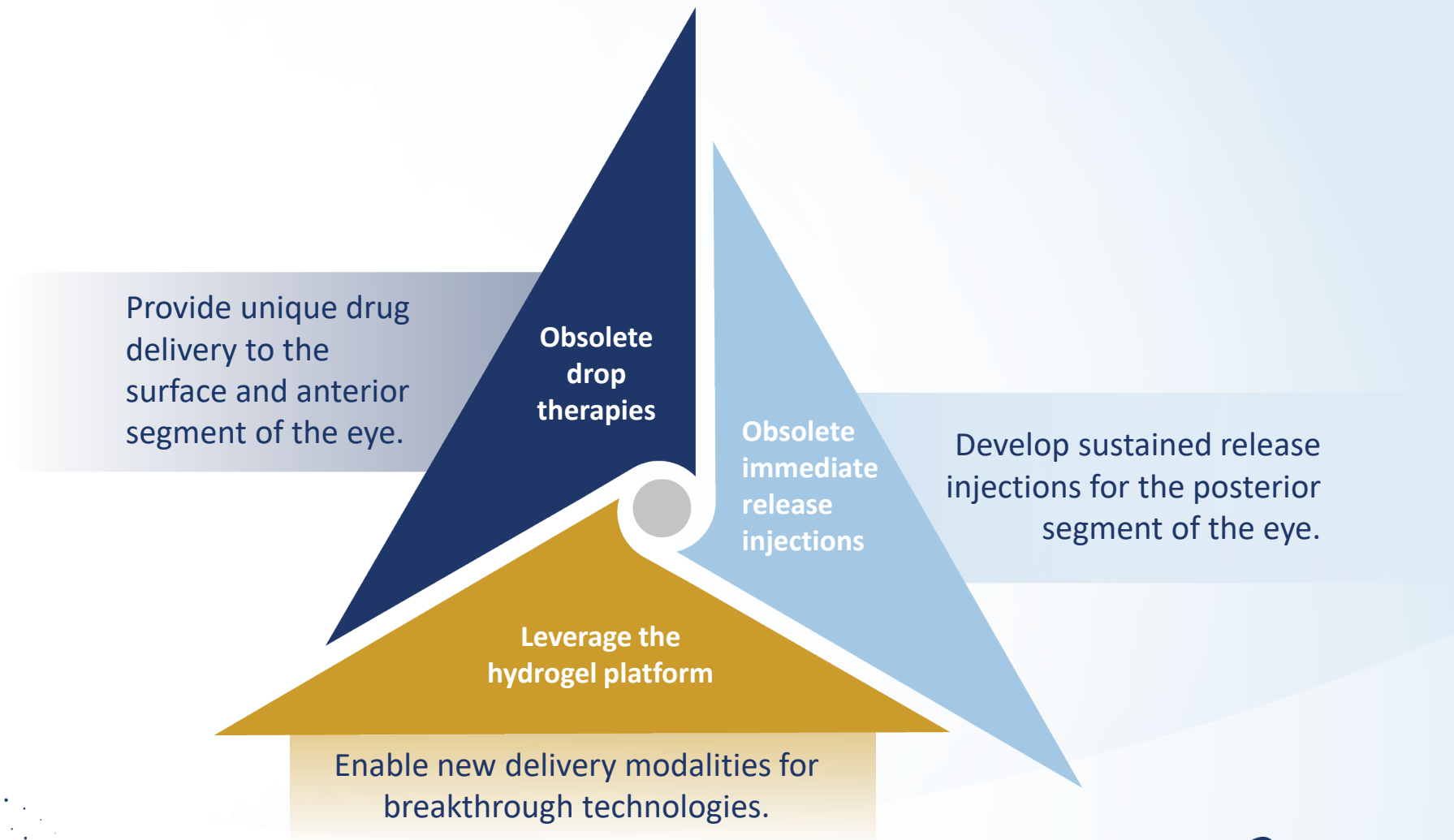
FORWARD LOOKING STATEMENTS

Any statements in this presentation about future expectations, plans, and prospects for the Company, including the commercialization of DEXTENZA®, ReSure Sealant, or any of the Company's product candidates, including the impact of and restructuring costs and potential savings associated with the Company's operational restructuring, workforce reduction and development program deferrals; the commercial launch of, and effectiveness of reimbursement codes for, DEXTENZA; the development and regulatory status of the Company's product candidates, such as the Company's development of and prospects for approvability of DEXTENZA for additional indications including allergic conjunctivitis, OTX-TP for the treatment of primary open-angle glaucoma and ocular hypertension, OTX-TIC for the treatment of primary open-angle glaucoma and ocular hypertension, OTX-TKI for the treatment of retinal diseases including wet AMD, and OTX-IVT as an extended-delivery formulation of the VEGF trap aflibercept for the treatment of retinal diseases including wet AMD; the ongoing development of the Company's extended-delivery hydrogel depot technology; the potential utility of any of the Company's product candidates; the potential benefits and future operation of the collaboration with Regeneron Pharmaceuticals, including any potential future payments thereunder; the sufficiency of the Company's cash resources and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend", "goal," "may", "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors. Such forward-looking statements involve substantial risks and uncertainties that could cause the Company's clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, those related to the implementation of the operational restructuring, the timing and costs involved in commercializing DEXTENZA, ReSure Sealant or any product candidate that receives regulatory approval, including the conduct of post-approval studies, the ability to retain regulatory approval of DEXTENZA, ReSure Sealant or any product candidate that receives regulatory approval, the ability to maintain reimbursement codes for DEXTENZA, the initiation, timing and conduct of clinical trials, availability of data from clinical trials and expectations for regulatory submissions and approvals, the Company's scientific approach and general development progress, the availability or commercial potential of the Company's product candidates, the sufficiency of cash resources, the Company's existing indebtedness, the ability of the Company's creditors to accelerate the maturity of such indebtedness upon the occurrence of certain events of default, the outcome of the Company's ongoing legal proceedings and need for additional financing or other actions and other factors discussed in the "Risk Factors" section contained in the Company's quarterly and annual reports on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this presentation represent the Company's views as of the date of this presentation. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so except as required by law. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this presentation.

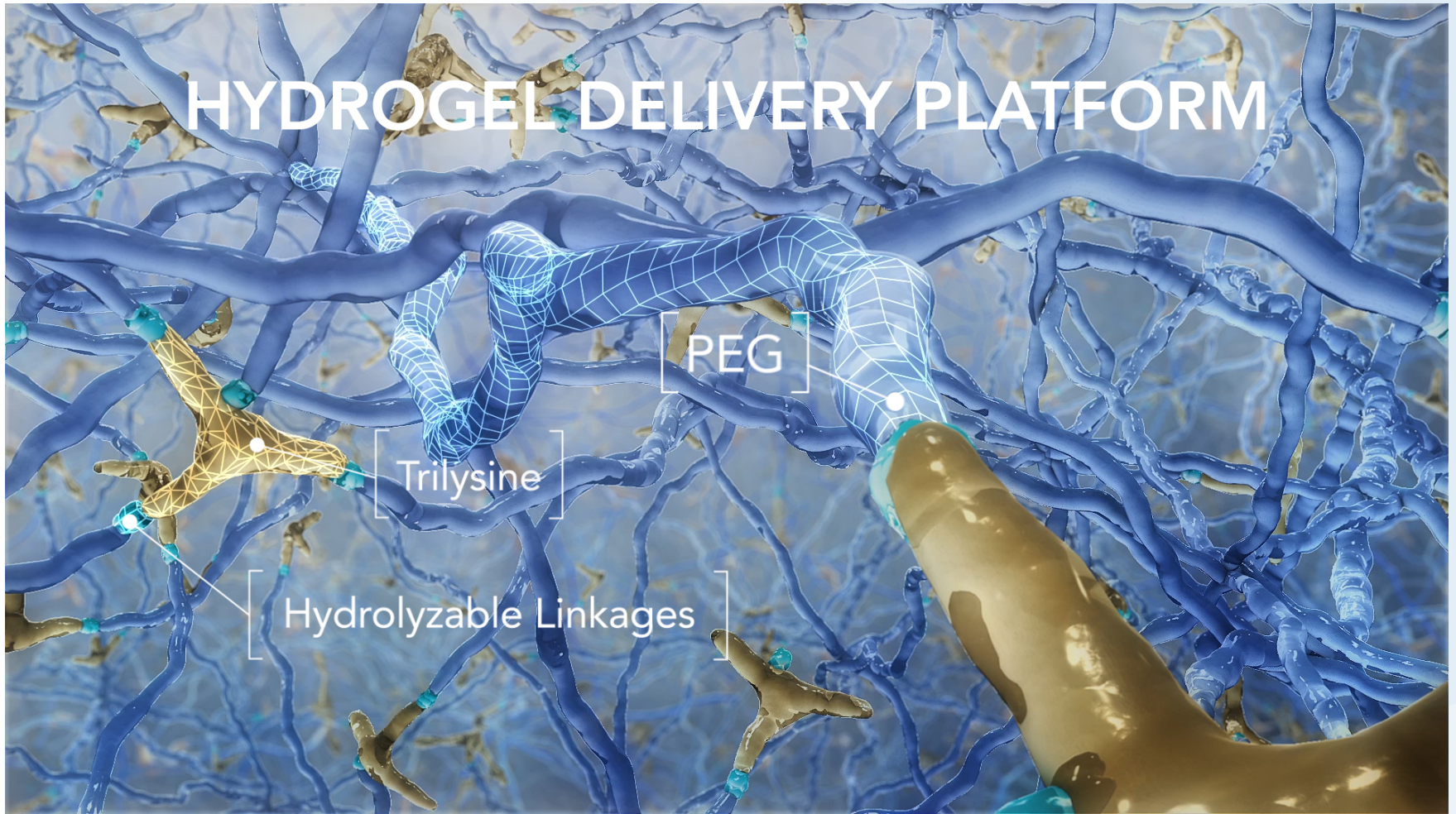


POSITIONED TO DEVELOP BREAKTHROUGH TREATMENTS

WE BELIEVE THE LAUNCH OF DEXTENZA® IS ONLY THE BEGINNING



TRANSFORMING DRUG DELIVERY WITH A NOVEL TECHNOLOGY PLATFORM



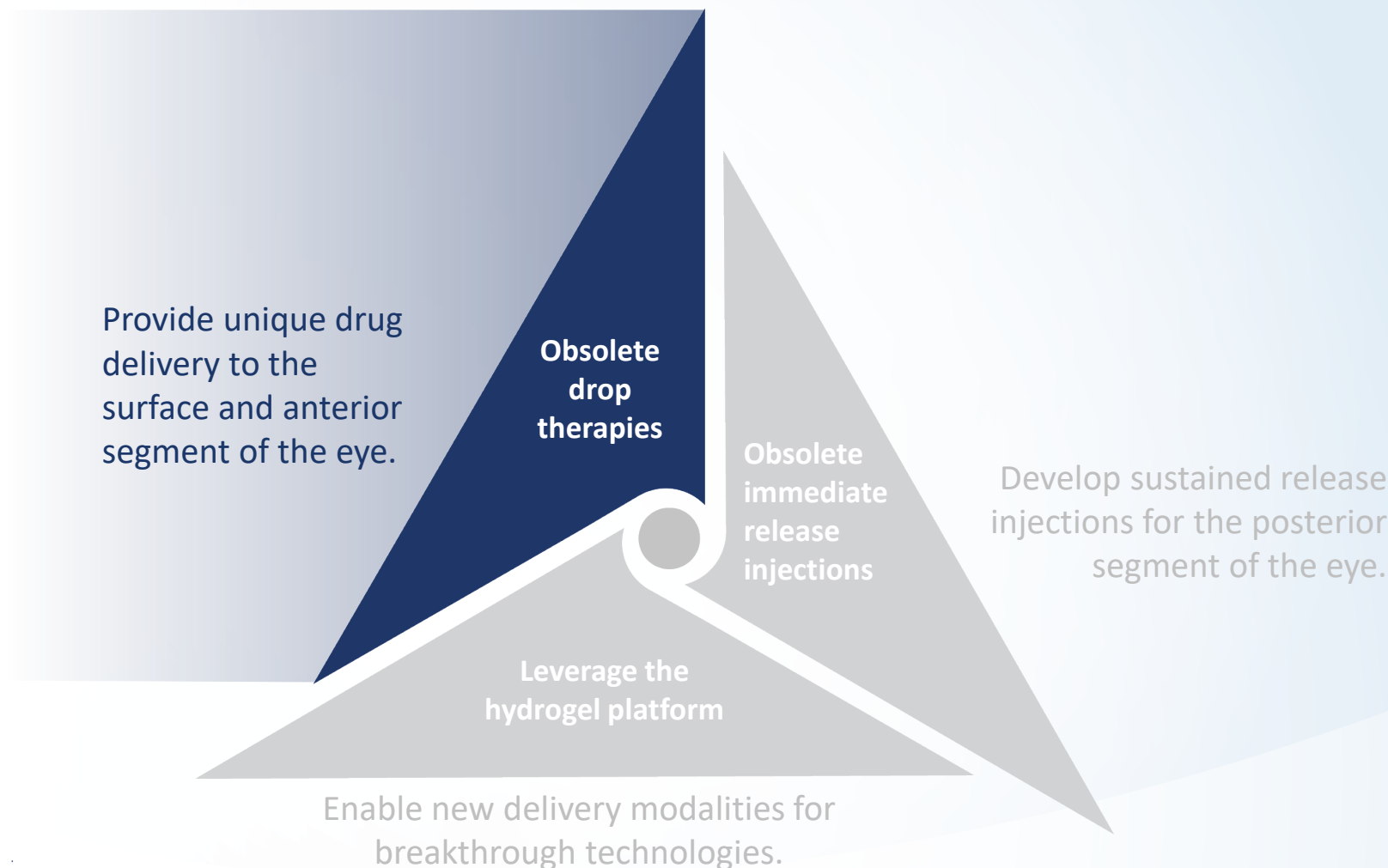
PIPELINE AT A GLANCE

PRODUCT/PROGRAM	DISEASE STATE	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	REGULATORY APPROVAL
INTRACANALICULAR INSERTS						
Dextenza® (dexamethasone ophthalmic insert) 0.4mg	Post-surgical ocular inflammation and pain					
Dextenza® (dexamethasone ophthalmic insert) 0.4mg	Allergic conjunctivitis					
Dextenza® (dexamethasone ophthalmic insert) 0.4mg	Episodic dry eye disease					
OTX-CSI (cyclosporine)	Dry eye disease					
OTX-TP (travoprost insert)	Glaucoma and ocular hypertension					
OTX-BPI (bupivacaine)	Acute ocular pain					
OTX-BDI (besifloxacin & dexamethasone)	Post-op pain, inflammation & anti-bacterial					
INTRACAMERAL IMPLANT						
OTX-TIC (travoprost implant)	Glaucoma and ocular hypertension					
INTRAVITREAL IMPLANTS						
OTX-TKI (tyrosine kinase inhibitor implant)	Wet AMD, DME and RVO†					
OTX-IVT* (anti-VEGF antibody implant)	Wet AMD, DME and RVO†					

* In Partnership with REGENERON

† Wet Age-related Macular Degeneration (Wet AMD), Diabetic Macular Edema (DME), Retinal Vein Occlusion (RVO)

POSITIONED TO DEVELOP BREAKTHROUGH TREATMENTS



AN INNOVATIVE INTRACANALICULAR INSERT



Dexamethasone bathes the ocular surface



DEXTENZA® STATUS

FDA APPROVED

DEXTENZA® is a corticosteroid indicated for the treatment of ocular inflammation and pain following ophthalmic surgery.

REIMBURSEMENT AND CODING

- Product **J-code 1096**
- Procedure **CPT code 0356T**
- Reimbursement support services DEXTENZA360™

LIFECYCLE MANAGEMENT

- Multiple active investigator-initiated trials (IIT) program
- Ongoing Phase 3 trial on allergic conjunctivitis potentially leading to sNDA filing

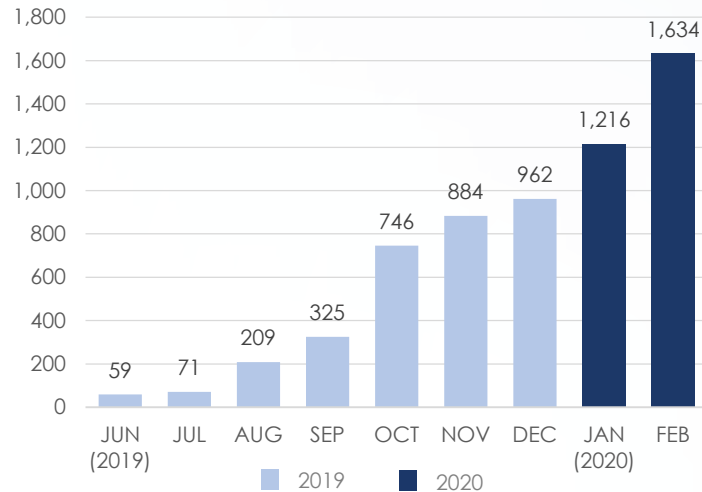
Provides a tapered delivery of
**preservative-free steroid onto the
ocular surface for 30 days**



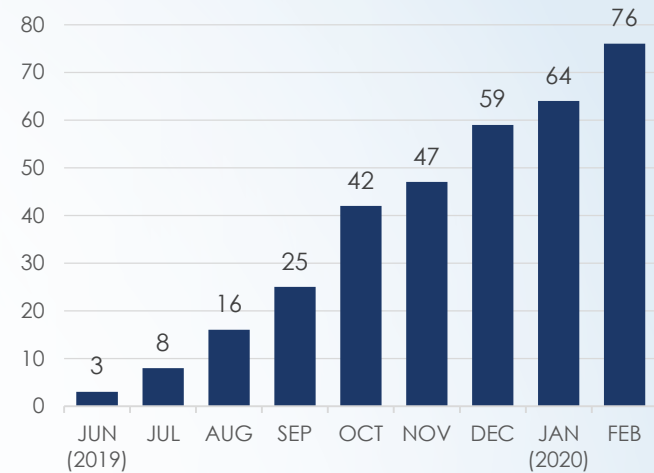
1. DEXTENZA [package insert]. Bedford, MA: Ocular Therapeutix Inc; 2019.
2. Data on file 00663. Ocular Therapeutix Inc.

DEXTENZA® LAUNCH METRICS

BILLABLE INSERTS



OF ORDERING ACCOUNTS BY MONTH



ACCOUNT ORDERING TRENDS

131

ASCs
ordered

91

ASCs
re-ordered

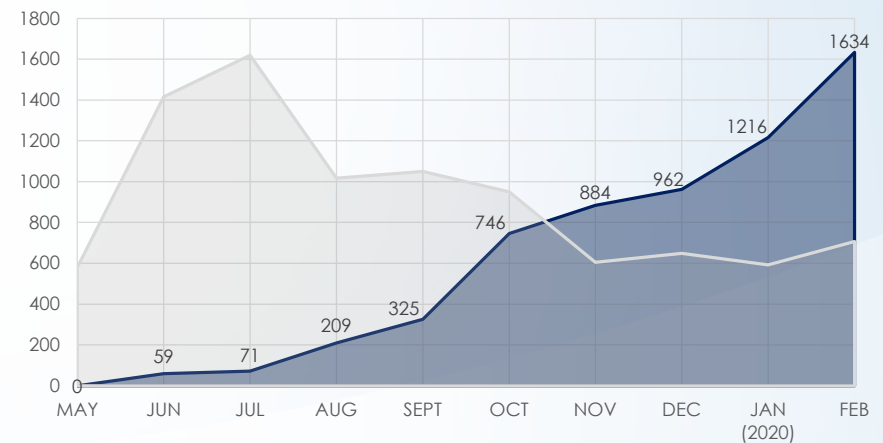
66

ASCs
re-ordered
more than
twice



Conversions

BILLABLE INSERTS VS SAMPLES



■ COMMERCIAL UNITS ■ SAMPLE UNITS

DEXTENZA®:

THE SURGICAL AND OCULAR SURFACE MARKETS ARE DISTINCT

Dextenza®

(dexamethasone ophthalmic insert) 0.4 mg

SURGICAL

Current indication

Inflammation & Pain

Setting

ASC and HOPD

Reimbursement:

3-year pass through

Potential

\$150-200M*

OCULAR SURFACE

Possible future indications

Allergic conjunctivitis & dry eye

Setting

Office

Reimbursement

Evergreen

Potential

\$500M - \$1B+*

*Based on Market Analogs



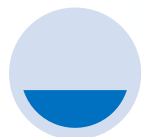
MICHAEL GOLDSTEIN, MD

CHIEF MEDICAL OFFICER, OCULAR THERAPEUTIX



GLAUCOMA: PATIENT ADHERENCE IS PROBLEMATIC

Poor adherence has been shown to be associated with disease progression and blindness



Glaucoma is an incurable, generally painless, chronic disease



Approximately 2.7 million individuals over 40 years old are affected in the U.S.



An estimated 3 million+ will be affected by 2020¹

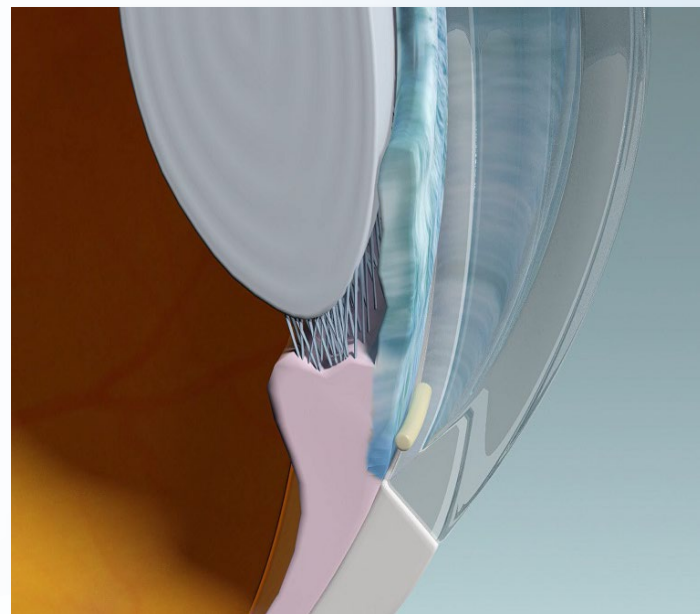
- The primary goal of glaucoma treatment is to reduce intraocular pressure
- Various medications can significantly lower intraocular pressure and reduce the progression of glaucoma, but these are almost always life-long medications that must be taken on a daily basis
- Adherence to glaucoma therapies is particularly poor, with reported rates of non-adherence ranging from 30–80%^{2,3,4}
- Poor adherence has been shown to be associated with disease progression and blindness^{5,6}

1) Tham YC, Li X, Wong TY, et al. Global Prevalence of Glaucoma and Projections of Glaucoma Burden through 2040: A Systematic Review and Meta-Analysis. *Ophthalmol* 2014;121:2081-90. 2) Olthoff CM, et al. Noncompliance with ocular hypotensive treatment in patients with glaucoma or ocular hypertension: an evidence-based review. *Ophthalmology*. 2005; 112:953–61. 3) Schwartz GF, et al. Adherence and persistence with glaucoma therapy. *Surv Ophthalmol*. 2008; 53(suppl1):557–68. 4) Nordstrom BL, et al. Persistence and adherence with topical glaucoma therapy. *Am J Ophthalmol*. 2005; 140:598–606. 5) Rossi GC, et al. Do adherence rates and glaucomatous visual field progression correlate? *Eur J Ophthalmol*. 2011; 21:410–4. 6) Sleath B, et al. The relationship between glaucoma medication adherence, eye drop technique, and visual field defect severity. *Ophthalmology*. 2011; 118:2398–402.

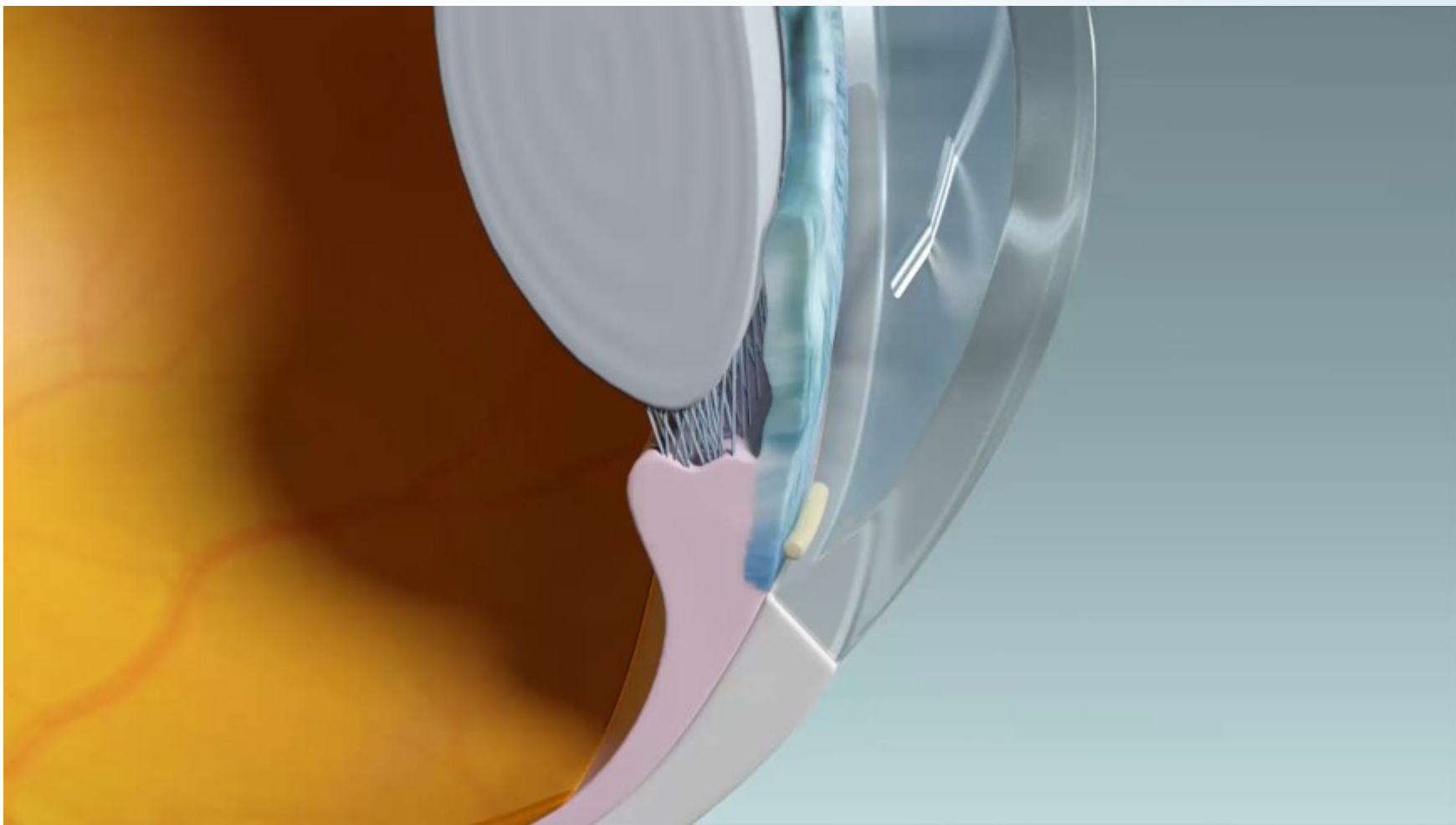
INTRACAMERAL DRUG DELIVERY

FACTORS FOR CONSIDERATION IN DESIGNING A LONG DURATION INTRACAMERAL IMPLANT:

- ❑ **Clinically-meaningful decrease in IOP**
Well-tolerated with clinically-meaningful efficacy
- ❑ **Extended duration of therapy**
4 months or more
- ❑ **Consistently bioresorbable**
Duration of drug and duration of carrier vehicle
- ❑ **Implant location and limited movement**
Limited movement and cosmetically invisible, but able to be monitored
- ❑ **Corneal health**
Gentle to the endothelium



OTX-TIC – INTRACAMERAL INJECTION

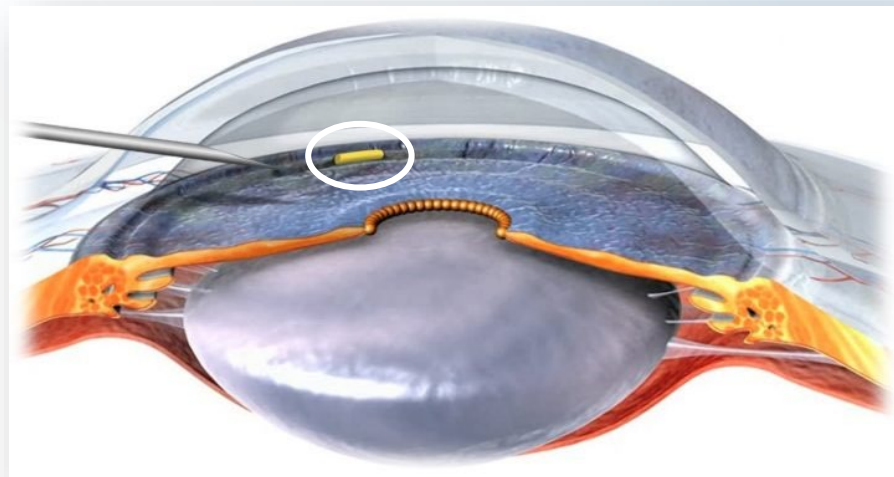


INTRACAMERAL INJECTION IN A PHASE 1 CLINICAL TRIAL FOR THE TREATMENT OF GLAUCOMA

OTX-TIC (travoprost implant) for intracameral injection

Description:

- Travoprost loaded microparticles in hydrogel
- Preservative-free
- Administered via a single injection with proprietary injector (27G)
- Implant resides in the iridocorneal angle, hydrates in 2 minutes
- Fully biodegradable



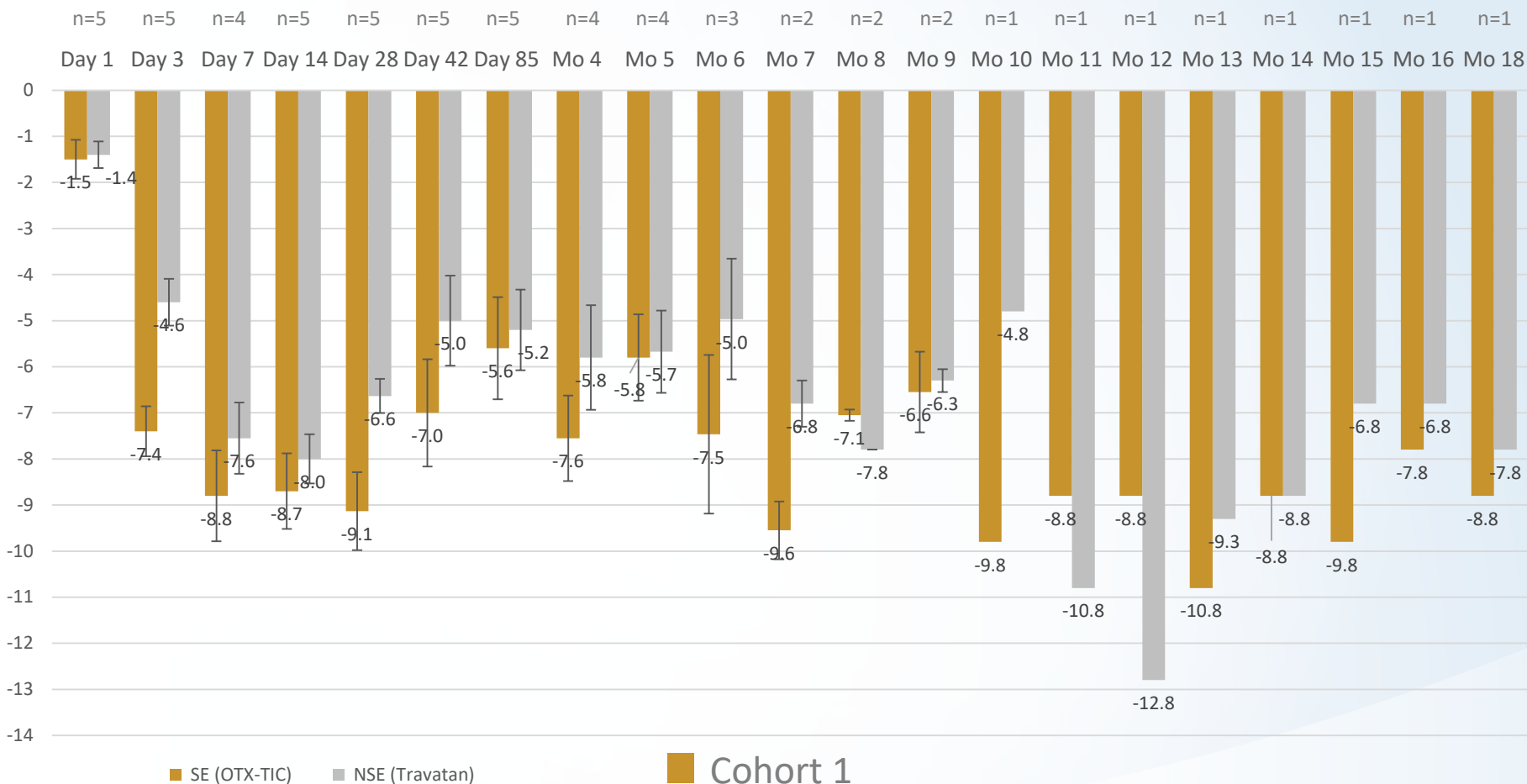
In preclinical models (beagle dogs):

- Steady state in vitro and in vivo release through 4 months, which correlates to a duration of 4-6 months in humans
- Demonstrated IOP lowering effect of approximately 25-30% through 4 months



COHORT 1: MEAN IOP CHANGE FROM BASELINE

8AM MEASUREMENTS



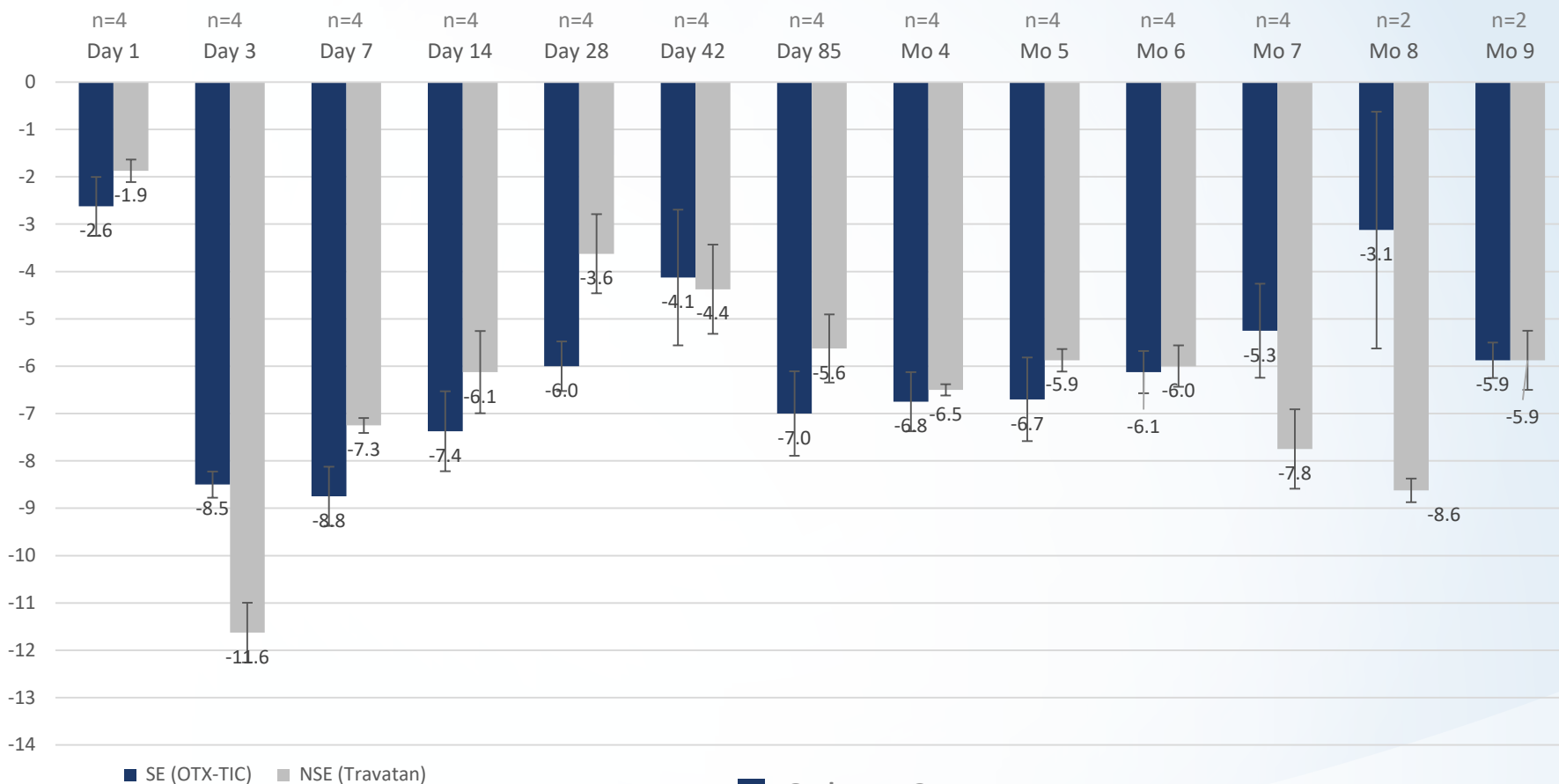
Cohort 1
15µg

NB: Interim look; Unmonitored data.

*If the study eye was given other IOP lowering medication, the IOP value was removed from the analysis.

COHORT 2: MEAN IOP CHANGE FROM BASELINE

8AM MEASUREMENTS



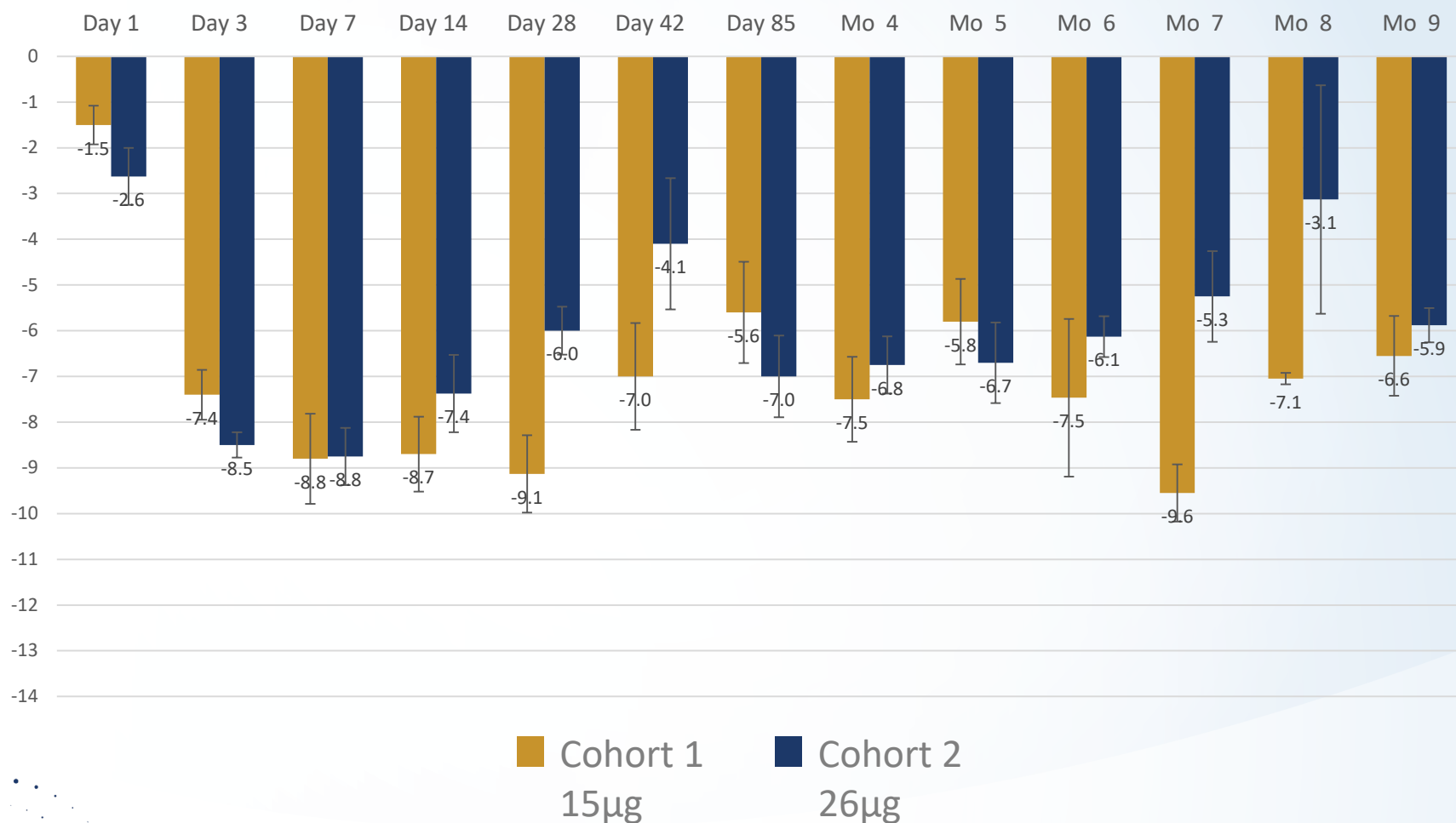
Cohort 2
26µg

NB: Interim look; Unmonitored data.

*If the study eye was given other IOP lowering medication, the IOP value was removed from the analysis.

COHORT 1 VS COHORT 2: IOP CHANGE FROM BASELINE

8AM MEASUREMENTS



OTX-TIC PHASE 1 INTERIM FINDINGS

✓ Clinically-meaningful decrease in IOP

Mean IOP values were decreased in patients receiving both OTX-TIC and topical travoprost as early as two days following administration, and mean IOP values remained decreased from baseline values

✓ Extended duration of therapy

Many subjects exhibited duration of IOP lowering effect of 6 months or longer

✓ Consistently bioresorbable

Implant biodegraded in most subjects by 6-7 months

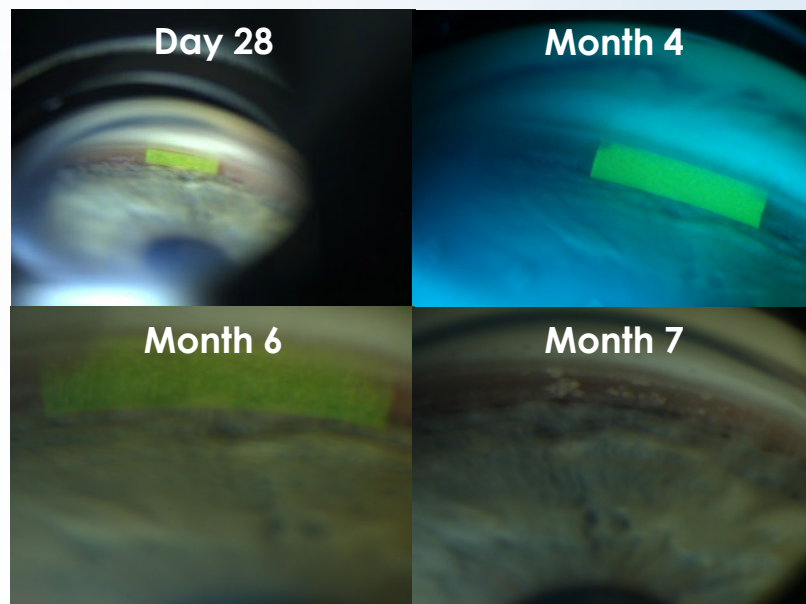
✓ Implant location and limited movement

Implant was not observed to move at slit lamp and was visible at all exams in all patients;
In one subject, there was slight rotation noted at the Day 14 visit as compared to the Day 7 visit

✓ Corneal health

Endothelial cell counts and pachymetry assessments indicate no changes from baseline

IMPLANTS VISUALIZED IN ALL SUBJECTS AT ALL VISITS THROUGH 7 MONTHS



POSITIONED TO DEVELOP BREAKTHROUGH TREATMENTS

Provide unique drug delivery to the surface and anterior segment of the eye.

Obsolete drop therapies

Obsolete immediate release injections

Develop sustained release injections for the posterior segment of the eye.

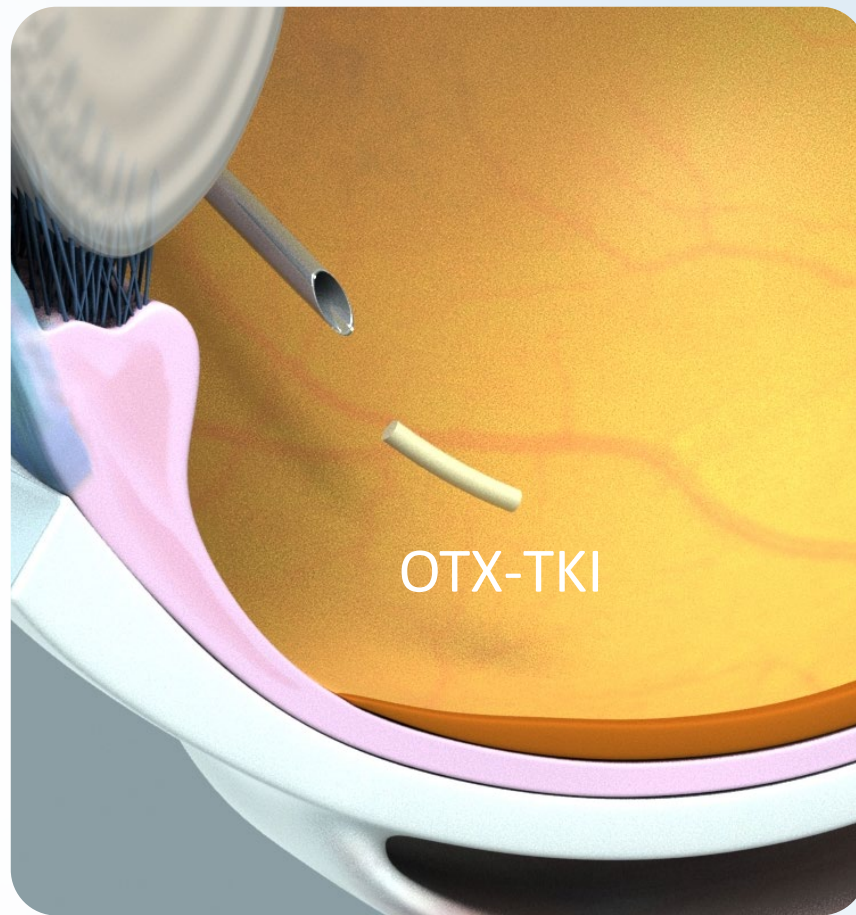
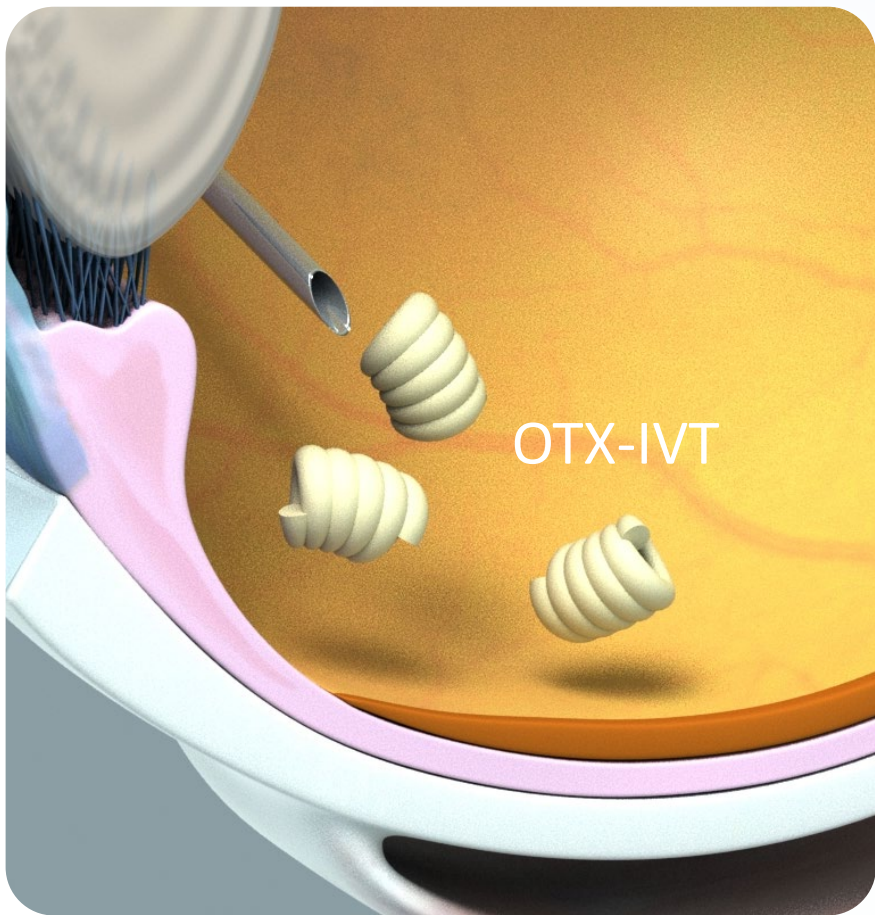
Leverage the hydrogel platform

Enable new delivery modalities for breakthrough technologies.



SUSTAINED RELEASE INJECTIONS

INTRAVITREAL IMPLANTS



UNMET NEED IN RETINAL DISEASE

PROBLEM WITH IMMEDIATE-RELEASE INJECTIONS

- While anti-VEGF drugs are effective, they are also rapidly cleared from the vitreous humor; therefore, to reach and to maintain effective concentrations, repeated administrations every 4-8 weeks are necessary¹
- Repeated intravitreal injections may cause side effects such as infection, endophthalmitis, hemorrhage, damage to the lens, retinal detachment, and poor patient tolerance over time¹
- Discomfort, eye pain, decreased vision, increased photosensitivity, and floaters are just some of the patient complaints with injections¹
- Repeated injections also have a significant impact on patients emotionally and financially as well as the time and transportation burden placed on care givers²

A New Therapy is Needed.

New Mechanism of Action

TKIs act upstream of Anti-VEGF

Longer Duration of Action

TKIs are potent small molecules

Research Question:

Does axitinib
(a tyrosine kinase inhibitor)
injected into the eye have
biological activity?

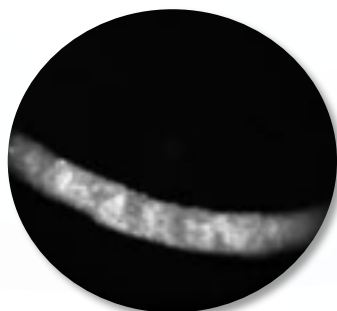
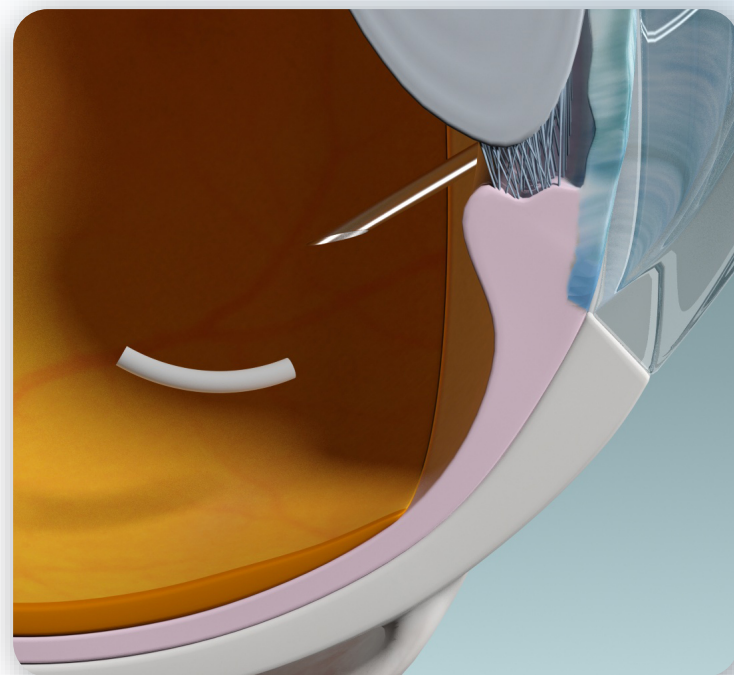
1. Bochot A, Fattal E. Liposomes for intravitreal drug delivery: a state of the art. *J Control Release*. 2012;161(2):628-634.

2. Boyle J, Vukicevic M, Koklanis K, Itsiopoulos C, Rees G. Experiences of patients undergoing repeated intravitreal anti-vascular endothelial growth factor injections for neovascular age-related macular degeneration. *Psychol Health Med*. 2018;23(2):127-140.

DRUG DELIVERY TO THE INTRAVITREAL SPACE

FACTORS FOR CONSIDERATION IN DESIGNING A LONG DURATION INTRAVITREAL IMPLANT:

- ❑ **Clinically-meaningful decrease in retinal fluid**
Well-tolerated with clinically-meaningful efficacy
- ❑ **Extended duration of therapy**
3 months or more
- ❑ **Consistently bioresorbable**
Duration of drug and duration of carrier vehicle
- ❑ **Implant location and limited movement**
Compact geometry, designed to avoid optical impact, but able to be monitored



INTRAVITREAL INJECTION IN A PHASE 1 CLINICAL TRIAL FOR THE TREATMENT OF AGE-RELATED MACULAR DEGENERATION

OTX-TKI (tyrosine kinase inhibitor implant) for intravitreal injection

DESCRIPTION:

- Targeting sustained release for 3 to 6+ months
- Broader anti-angiogenic profile than anti-VEGF alone and longer duration (small molecule)
- Systemic TKI efficacy established in oncology
- Small fiber (27-30G needle) with minimal/no visual impact; product candidate can be monitored by physician
- Potential to provide an additional option for patients and providers
- Different target than traditional VEGF therapies
- Preservative-free

IN PRECLINICAL MODELS (RABBIT CHALLENGE):

- Sustained, steady state in vitro and in vivo release for up to 12 months with a single insert
- PK, PD and tolerability with no negative safety signals reported to date



Video shown in real time
in simulated vitreous humor



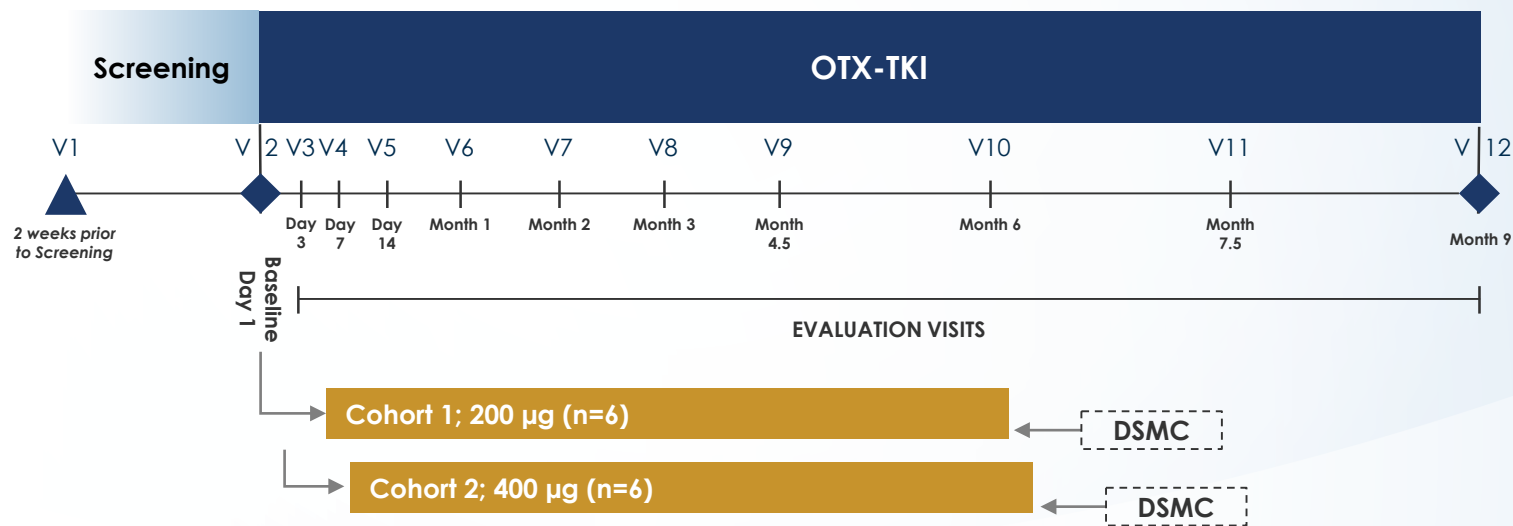
OTX-TKI PHASE 1 STUDY

DESIGN

- Open-label, dose-escalation, feasibility study
- 5 sites in Australia
- 9-month study
- One eye per patient will be treated
- Key Inclusion criteria:
 - ✓ Active primary SFNV secondary to AMD

OBJECTIVES

- Safety, tolerability, and biological activity
- Safety evaluations at all visits; mean change in CSFT measured by SD-OCT, BCVA, and clinically-significant leakage on FA and/or OCT-A at 6 months



MEAN CHANGE IN CENTRAL SUBFIELD THICKNESS VALUES

STUDY EYE BY COHORT



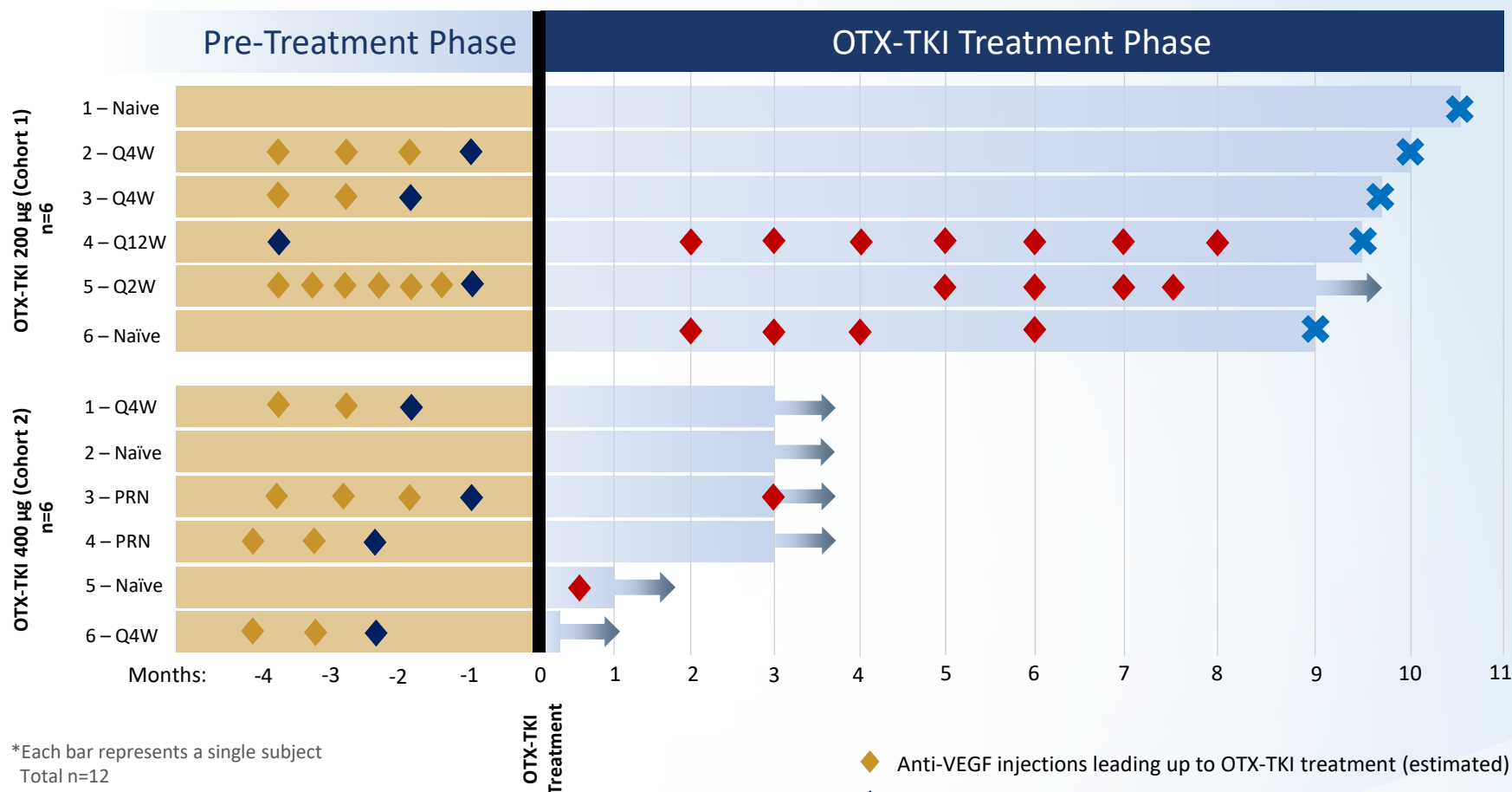
*Cohort 1 subjects 02-001 and 01-001 CSFT compared to Baseline visit; remainder of subjects CSFT compared to Screening visit

*Cohort 2 subjects CSFT all compared to Baseline visit

*All BCVA values compared to Baseline visit

NOTE: Values shown are Mean \pm SEM;
Interim review, unmonitored data

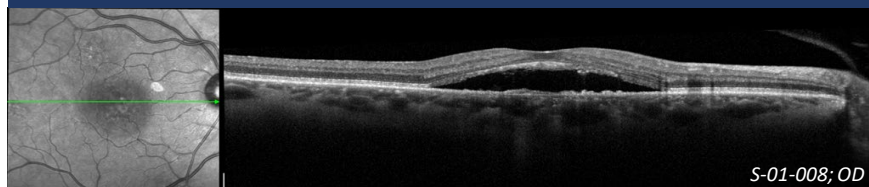
INDIVIDUAL SUBJECT DURABILITY ASSESSMENT



COHORT 2: SD-OCT EVALUATION

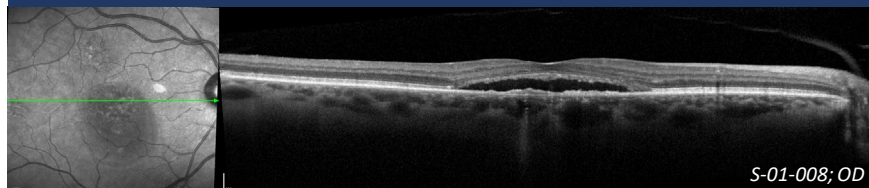
Subject 1: History of EYLEA Q4 Weeks

BASELINE



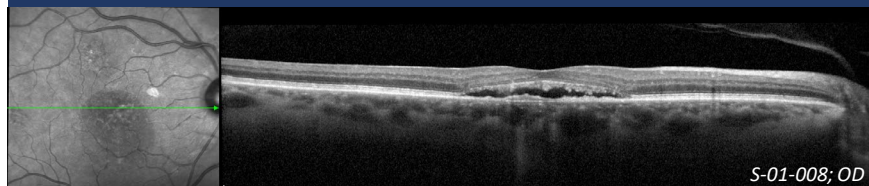
CSFT: 473 μm

MONTH 2



CSFT: 368 μm

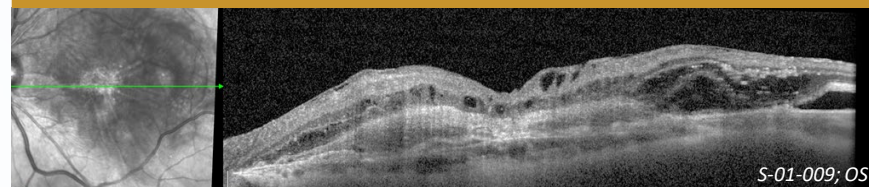
MONTH 3



CSFT: 337 μm

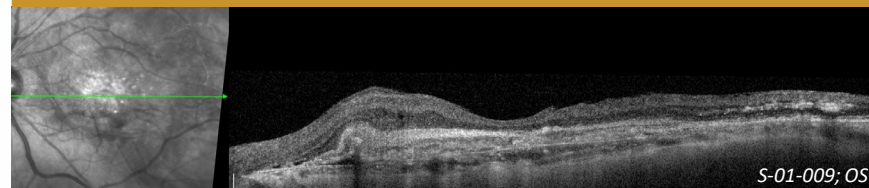
Subject 2: Treatment Naïve

BASELINE



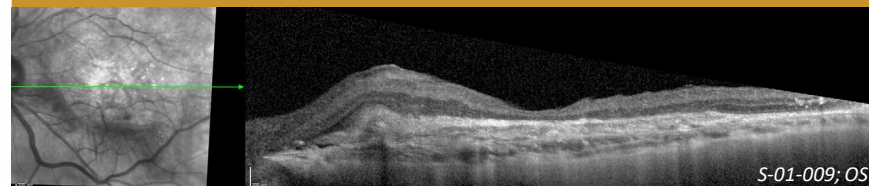
CSFT: 513 μm

MONTH 2



CSFT: 343 μm

MONTH 3



CSFT: 316 μm



COHORT 1 & 2

SAFETY OVERVIEW

TOTAL ADVERSE EVENTS

Number of subjects with:	OTX-TKI 200 µg N=6	OTX-TKI 400 µg N=6	Total (n=12)
Adverse Events (AEs)	17	12	29
Treatment-related AEs	2	1	3
Injection procedure-related AEs	1	4	5
By severity			
<i>Mild</i>	14	10	24
<i>Moderate</i>	2	2	4
<i>Severe</i>	0	0	0
Ocular AEs	15	8	23
Ocular AEs in the study eye	11	6	17
Ocular Serious AEs	0	0	0
Treatment-related SAEs	0	0	0
Injection procedure-related SAEs	0	0	0



OTX-TKI CONCLUSIONS: TO BE CONFIRMED

❑ **Clinically-meaningful decrease in retinal fluid**

To date, OTX-TKI was generally well tolerated and observed to have a favorable safety profile in both cohorts. In the higher dose cohort, some subjects showed a decrease in intraretinal or subretinal fluid at 2 months

❑ **Extended duration of therapy**

Patients still being followed in cohort 2, to be determined

❑ **Consistently bioresorbable**

Implant biodegraded in 5 of 5 subjects by 9-10 months in cohort 1

❑ **Implant location and limited movement**

Implant was able to be monitored; patients did not report visual impact

Study is ongoing; continued long-term evaluation of both cohorts

- Need to establish durability of treatment
- Identify Maximum Tolerated Dose (MTD)
- Understand utility of OTX-TKI with anti-VEGF injection

RECENT AND ANTICIPATED NEAR-TERM MILESTONES



DEXTENZA® launch is progressing well with strong clinical uptake.

**Opportunity to treat the ocular surface diseases with DEXTENZA®.
Allergic conjunctivitis Phase 3 top-line data expected Q2, 2020.**

**First two OTX-TIC cohorts showed a clinically meaningful decrease in
IOP in both cohorts with an extended duration of biological activity.**

**OTX-TKI was generally well tolerated and observed to have a favorable
safety profile in both cohorts treated to date. Some subjects in the
higher cohort showed evidence of biological activity with decreased
intraretinal or subretinal fluid.**



(NASDAQ: OCUL)

TRANSFORMING DRUG DELIVERY LEVERAGING A NOVEL TECHNOLOGY PLATFORM

THANK YOU

Ocular
Therapeutix™