

(NASDAQ: OCUL)

# TRANSFORMING DRUG DELIVERY

## LEVERAGING A NOVEL TECHNOLOGY PLATFORM

ANTONY MATTESSICH, CHIEF EXECUTIVE OFFICER

June 2021

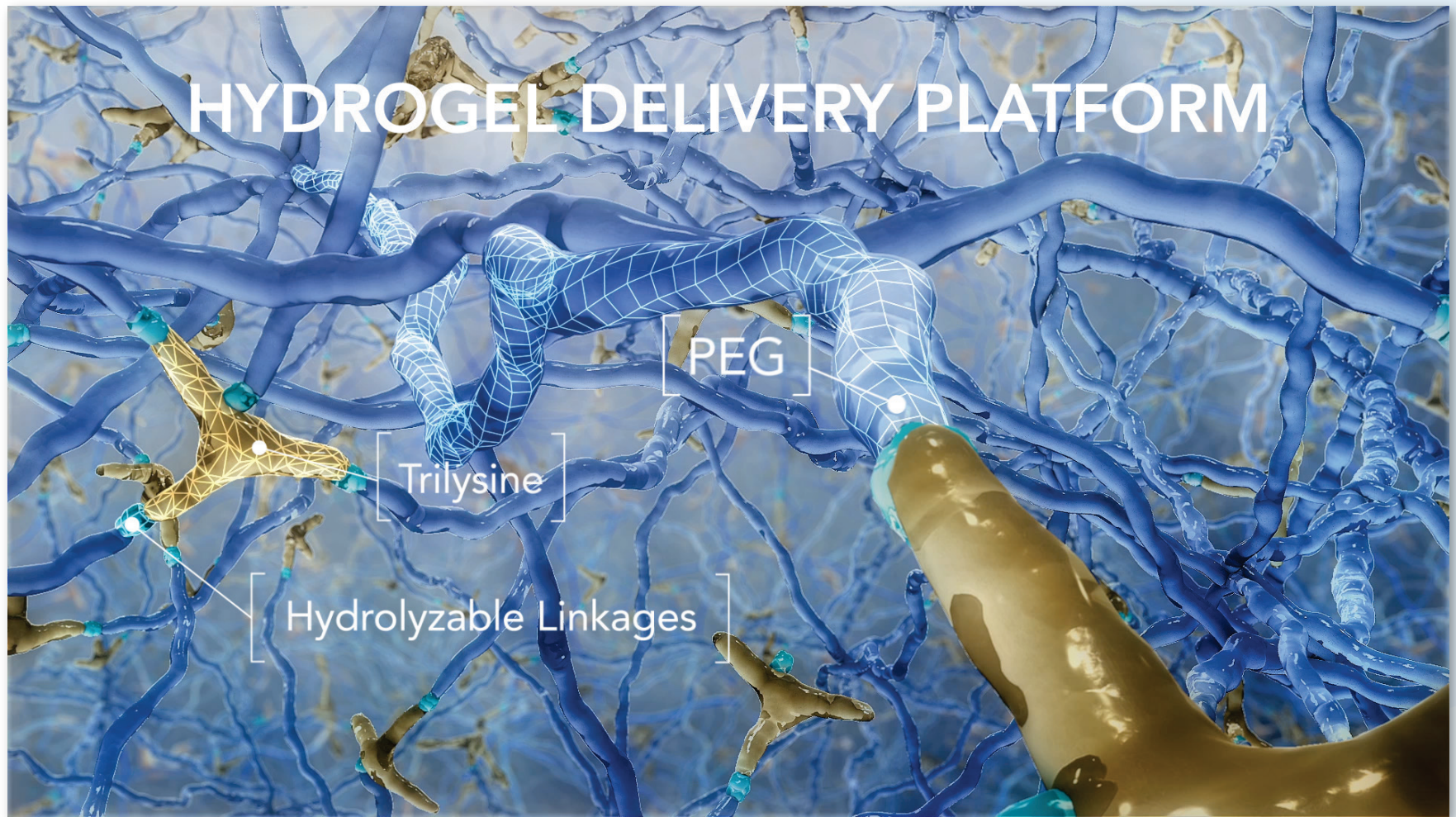


# FORWARD LOOKING STATEMENTS






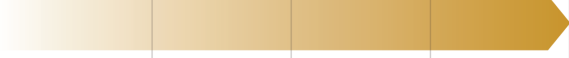


Any statements in this presentation about future expectations, plans, and prospects for the Company, including the commercialization of DEXTENZA®, ReSure® Sealant, or any of the Company's product candidates; the commercial launch of, and effectiveness of reimbursement codes for, DEXTENZA; the conduct of post-approval studies of 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 the target action date under PDUFA scheduled for October 18, 2021, for allergic conjunctivitis, OTX-DED for the short-term treatment of the signs and symptoms of dry eye disease, OTX-CSI for the chronic treatment of dry eye disease, OTX-TIC for the treatment of primary open-angle glaucoma or ocular hypertension, OTX-TKI for the treatment of retinal diseases including wet AMD, and OTX-AFS 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 size of potential markets for our product candidates; 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; projected net product revenue, in-market sales, and other financial and operational metrics of DEXTENZA; potential market sizes for indications targeted by the Company's product candidates, if approved; the expected impact of the COVID-19 pandemic on the Company and its operations; 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, 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 and outcomes 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 Company's ability to generate its projected net product revenue and in-market sales on the timeline expected, if at all, 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 severity and duration of the COVID-19 pandemic including its effect on the Company's and relevant regulatory authorities' operations, any additional financing needs 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, whether as a result of new information, future events or otherwise, 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.



# TRANSFORMING DRUG DELIVERY WITH A NOVEL TECHNOLOGY PLATFORM



# PIPELINE AT A GLANCE

PRODUCT/PROGRAM	THERAPEUTIC FOCUS	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	REGULATORY APPROVAL
<b>RETINA</b>						
<b>OTX-TKI</b> (axitinib intravitreal implant)	Wet AMD, DME and RVO†					
<b>OTX-AFS</b> (aflibercept suprachoroidal injection) In collaboration with <b>REGENERON</b>	Wet AMD, DME and RVO†					
<b>GLAUCOMA</b>						
<b>OTX-TIC</b> (travoprost intracameral implant)	Glaucoma and ocular hypertension					
<b>OCULAR SURFACE DISEASES</b>						
<b>OTX-CSI</b> (cyclosporine intracanalicular insert)	Dry eye disease					
<b>OTX-DED</b> (dexamethasone intracanalicular insert)	Episodic dry eye disease					
<b>Dextenza®</b> (dexamethasone ophthalmic insert) 0.4mg	Allergic conjunctivitis					
<b>SURGICAL</b>						
<b>Dextenza®</b> (dexamethasone ophthalmic insert) 0.4mg	Post-surgical ocular inflammation and pain					
<b>ReSure®</b> SEALANT	Cataract incision closure					

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



# MAINTAINING EXCLUSIVITY

## ☐ Expanding US patent portfolio to maintain exclusivity

PRODUCT	DISEASE STATE	PATENT STATUS	TIMELINE – LATEST PATENT EXPIRATION DATE*		
			2020	2030	2040
<b>Dextenza®</b> (dexamethasone ophthalmic insert) 0.4mg	Post surgical ocular inflammation and pain	Issued	Multiple		
<b>ReSure®</b> SEALANT	Post surgical clear corneal incisions	Issued	Multiple		
<b>DEXTENZA®</b> (dexamethasone ophthalmic insert) 0.4 mg	Allergic conjunctivitis	Pending			
<b>OTX-TIC</b> (travoprost intracameral implant)	Glaucoma and ocular hypertension	Pending			
<b>OTX-TKI</b> (axitinib intravitreal implant)	Wet AMD, DME and RVO	Pending			
<b>OTX-CSI</b> (cyclosporine intracanalicular insert)	Dry eye disease	Pending			
<b>OTX-DED</b> (dexamethasone intracanalicular insert)	Episodic dry eye disease	Pending			

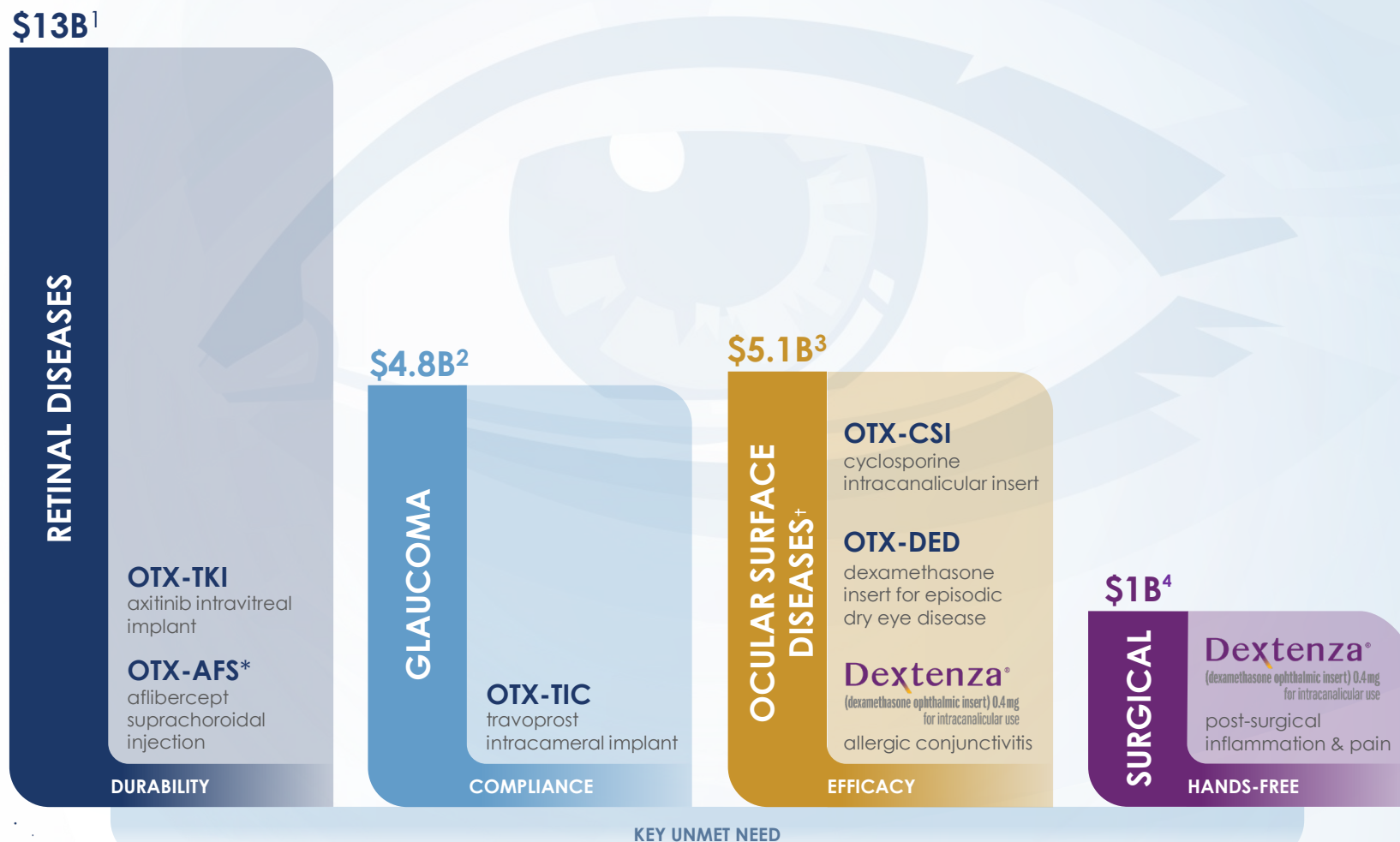
## ☐ Technical know-how of the core technology

## ☐ Drug delivery via non-conventional dosage forms

\*Includes expected expiration dates for pending patent applications if granted.

# TOTAL GLOBAL MARKETS

DEVELOPING PRODUCTS WITH THE POTENTIAL TO BECOME A STANDARD OF CARE FOR SELECT INDICATIONS IN SEVERAL OF THE LARGEST SEGMENTS IN OPHTHALMOLOGY



These data reflect the total global market for the respective indications. Our market opportunity for such indications reflects a portion of this market.

\* In collaboration with REGENERON; † Data shown here is only representative of Dry Eye and not other Ocular Surface Diseases

1. 2019 Retina Pharma Market Scope Report 2. 2019 Glaucoma Pharma Market Scope Report 3. 2019 Dry Eye Market Scope Report 4. Estimated using historical costs of topical eyedrops (not DEXTENZA) and the total addressable market based on the total US ocular surgical steroid market value 2019

# OTX-TKI (AXITINIB INTRAVITREAL IMPLANT)

## SUSTAINED RELEASE THERAPY FOR RETINAL DISEASES

### ISSUES WITH EXISTING TREATMENTS

- Require injections every 4-8 weeks<sup>1,2</sup>
- May cause endophthalmitis, hemorrhage, damage to the lens or retinal detachment due to repeated injections<sup>3</sup>
- Cause discomfort, eye pain, decreased vision, increased photosensitivity, and floaters<sup>3</sup>

### KEY PRODUCT ATTRIBUTES

- Targeting sustained release for 6 months or longer
- Broader anti-angiogenic profile (small molecule) than anti-VEGF alone
- Small fiber with minimal/no visual impact
- Preservative-free



### ONGOING PHASE 1 CLINICAL TRIAL

- Cohorts 1 (200µg), 2 (400µg) & 3 (two arms: 600µg & 400µg + anti-VEGF induction injection) fully enrolled
- To date, observed to have a generally favorable safety profile
- Adding fourth cohort (two arms: 600 µg single implant & 600µg single implant + anti-VEGF induction injection)

1. EYLEA Full Prescribing information 2019 2. Lucentis full Prescribing Information 2019 3. Bochot A, Fattal E. Liposomes for intravitreal drug delivery: a state of the art. *J Control Release*. 2012;161(2):628-634.



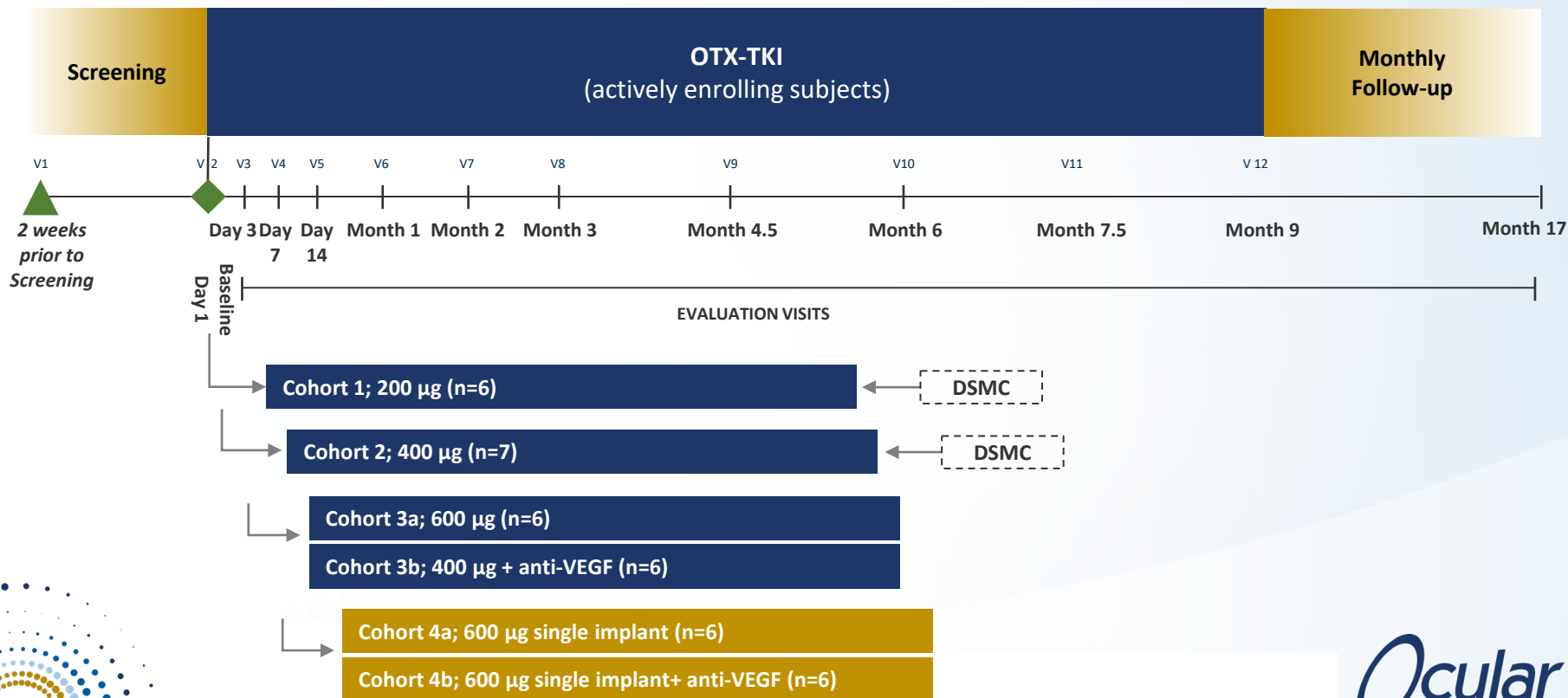
# OTX-TKI PHASE 1 STUDY

## DESIGN

- Open-label, dose-escalation, feasibility study
- 5 sites in Australia
- One eye treated per patient
- Key Inclusion criteria:
  - Active primary sub foveal neovascularization (SFNV) secondary to AMD – previously treated or naïve subjects but with retinal fluid present

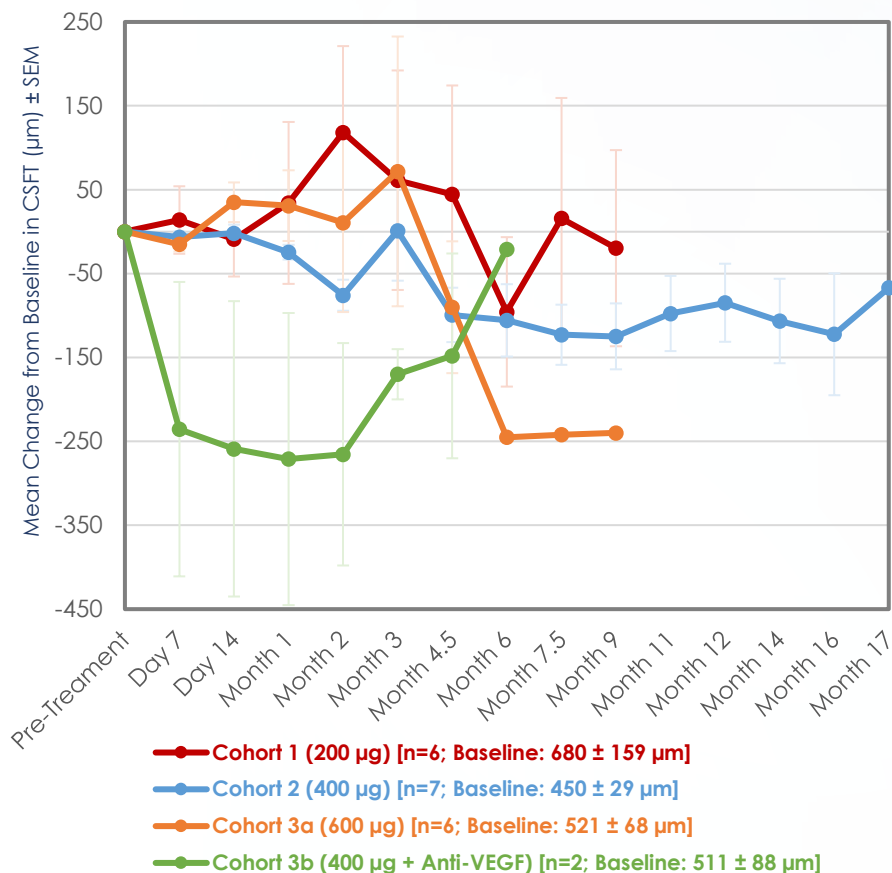
## OBJECTIVES

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

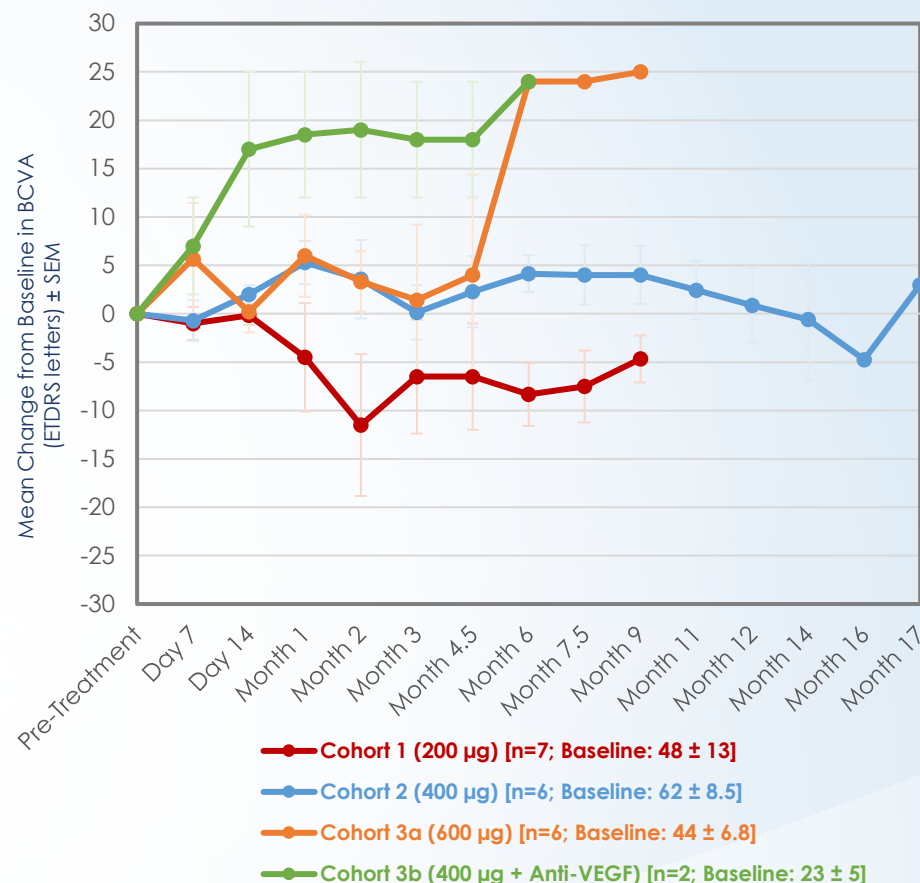


# ALL COHORTS: MEAN CHANGE IN CSFT AND BCVA

## CHANGE FROM BASELINE IN CSFT



## CHANGE FROM BASELINE IN BCVA



Cohort 1: n=6 until Month 9; Cohort 2: n=7 until Month 12, n=5 for Month 14, n=4 for Month 16 & n=1 for Month 17  
 Cohort 3a: n=6 until Month 2, n=4 for Months 3; n=3 for Month 4.5 & n=1 until Month 9; Cohort 3b: n=2 until Month 4.5; n=1 for Month 6

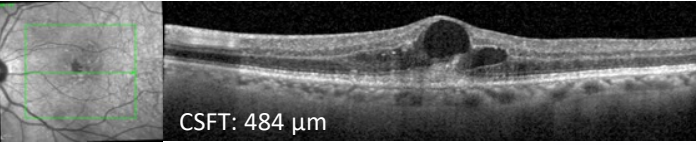
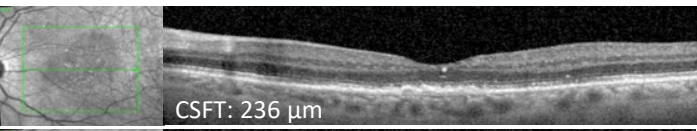
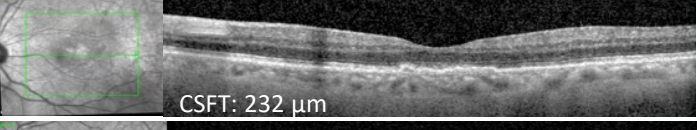
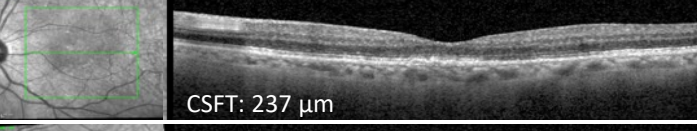
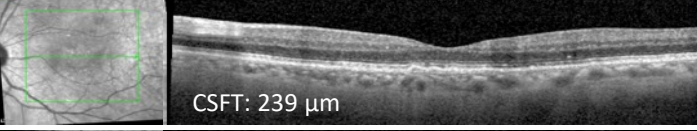
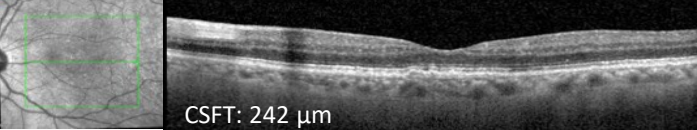
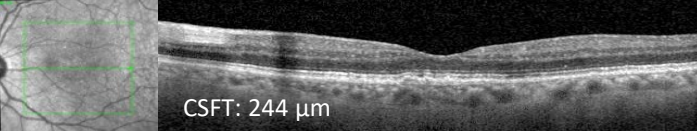
\*All BCVA and CSFT values compared to Baseline visit; NOTE: Interim review, unmonitored data; Data cut on April 12<sup>th</sup>, 2021

# OTX-TKI PHASE 1

## SD-OCT EVALUATION: COHORT 3

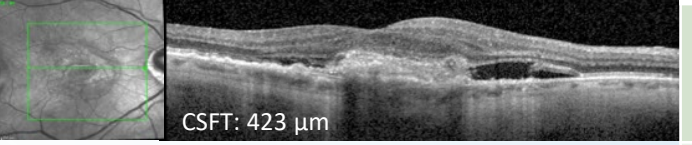
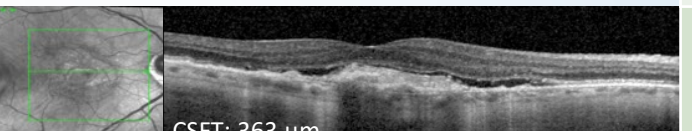
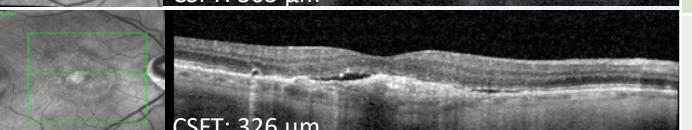
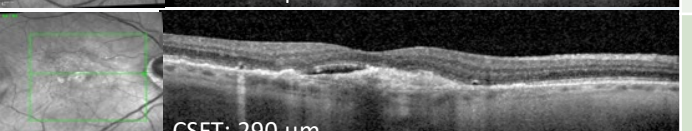
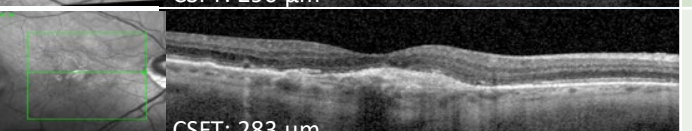
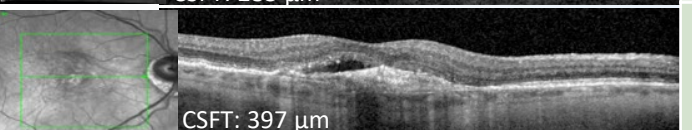
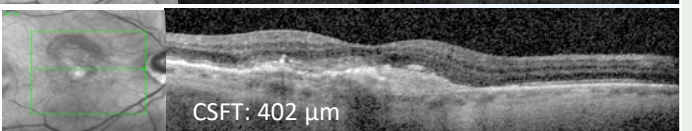
### Cohort 3a (600µg):

#### Subject 1 (OS): Treatment Naïve Subject

TIMEPOINT	SD-OCT	BCVA
BASLINE		0.58 (20/76)
MONTH 2		0.22 (20/33)
MONTH 3		0.24 (20/40)
MONTH 4.5		0.1 (20/25)
MONTH 6		0.1 (20/25)
MONTH 7.5		0.1 (20/25)
MONTH 9		0.08 (20/24)

### Cohort 3b (400µg + Anti-VEGF):

#### Subject 1 (OD): Treatment Naïve Subject

TIMEPOINT	SD-OCT	BCVA
BASLINE		1.14 (20/252)
DAY 7		1.1 (20/252)
MONTH 1		0.64 (20/87)
MONTH 2		0.62 (20/83)
MONTH 3		0.66 (20/91)
MONTH 4.5		0.66 (20/91)
MONTH 6		0.66 (20/91)

\*NOTE: Interim review, unmonitored data; Data cut on April 12<sup>th</sup>, 2021

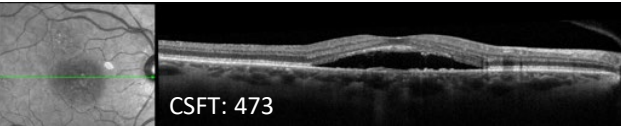
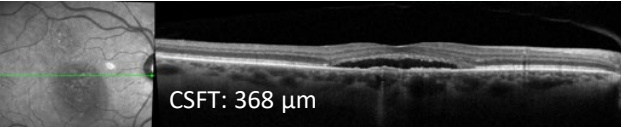
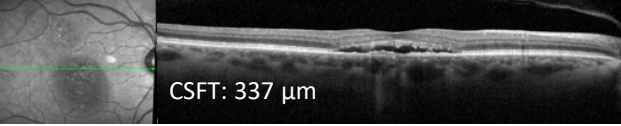
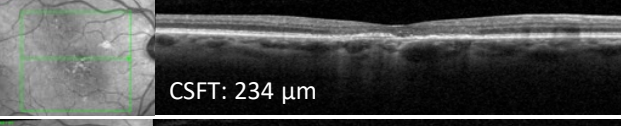
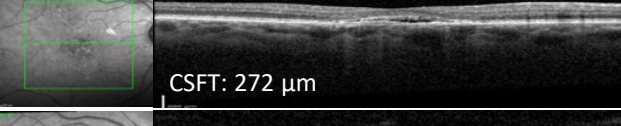
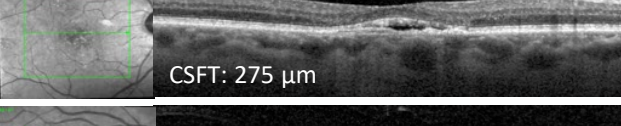



# OTX-TKI PHASE 1

## SD-OCT EVALUATION: COHORT 2

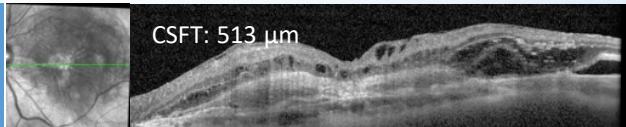
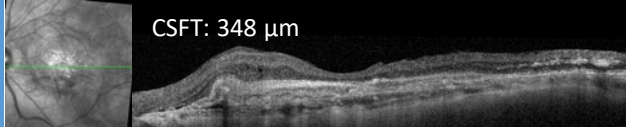
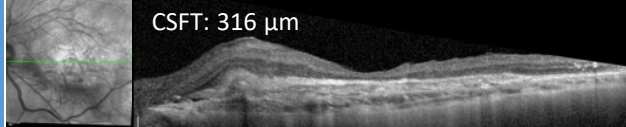
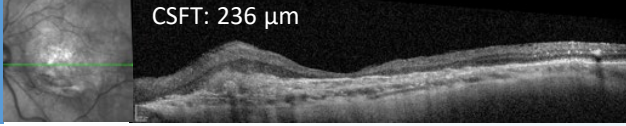
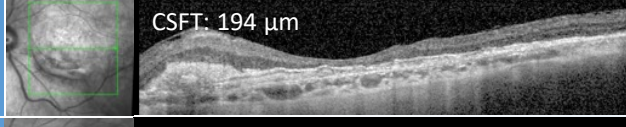
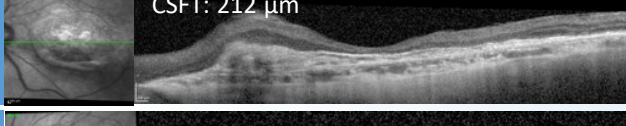
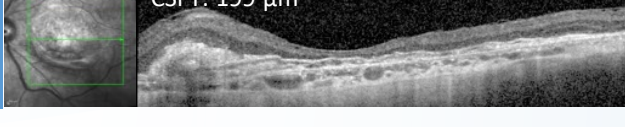
### Cohort 2 (400µg): Subject 1 (OD):

History of Aflibercept Q4 Weeks for 16 months

		BCVA
BASLINE		-0.04 (20/18)
MONTH 2		-0.06 (20/17)
MONTH 3		-0.06 (20/17)
MONTH 6		-0.08 (20/17)
MONTH 9		-0.06 (20/17)
MONTH 11		0.02 (20/21)
MONTH 13.5		-0.12 (20/15)

### Cohort 2 (400µg):

Subject 2 (OS): Treatment Naïve Subject

		BCVA
BASLINE		1.40 @ 1m (20/502)
MONTH 2		1.40 @ 1m (20/502)
MONTH 3		1.48 @ 1m (20/604)
MONTH 6		<b>Rescued at Month 4.5</b> 1.34 @ 1m (20/438)
MONTH 9		<b>Rescued at Month 6 &amp; 7.5</b> 1.44 @ 1m (20/551)
MONTH 11		1.44 @ 1m (20/551)
MONTH 16		1.44 @ 1m (20/551)

\*NOTE: Interim review, unmonitored data; Data cut on April 12<sup>th</sup>, 2021; BCVA presented in logMAR format with Snellen equivalent in parenthesis

# PHASE I RESULTS DEMONSTRATING DURATION OF EFFECT

## Percentage of Subjects Without Needing Rescue Medications

## Extended Follow-up

Cohorts	At 1 months % (n/N)	At 3 months % (n/N)	At 6 months % (n/N)	At 7.5 months % (n/N)	At 9 months % (n/N)	At 12 months % (n/N)	At 14 months % (n/N)	At 17 months % (n/N)
Cohort 1 (200 µg)	100 (6/6)	66.7 (4/6)	50 (3/6)	50 (3/6)	50 (3/6)	NA	NA	NA
Cohort 2 (400 µg)*	85.7 (6/7)	71.4 (5/7)	57.1 (4/7)	42.9 (3/7)	42.9 (3/7)	28.6 (2/7)	20 (1/5)*	50 (1/1)*
Cohort 3a (600 µg)*	83.3 (5/6)	100 (4/4)*	100 (1/1)*	100 (1/1)*	100 (1/1)*	TBD	TBD	TBD
Cohort 3b (400 µg + anti-VEGF)*	100 (2/2)*	100 (2/2)*	50 (1/2)*	TBD	TBD	TBD	TBD	TBD

\*Follow-up ongoing

\*NOTE: Interim review, unmonitored data; Data cut on April 12<sup>th</sup>, 2021

# OTX-TKI CONCLUSIONS TO DATE

## ❑ OTX-TKI was generally well tolerated

- To date, observed to have a favorable safety profile, with no ocular serious adverse events in treatment naïve & previously treated wet AMD patients
- No measurable systemic exposure to axitinib observed in cohorts 1-2

## ❑ Preliminary biological signal of clinically-meaningful decrease in retinal fluid

- Some subjects showed a decrease in intraretinal or subretinal fluid by 2 months in Cohorts 2 (400 µg) & 3a (600 µg)
- Combination of OTX-TKI + Anti-VEGF (Cohort 3b) showed a decrease in intraretinal or subretinal fluid immediately, as early as a week after treatment in two subjects

## ❑ Therapy durability suggests extended duration of action

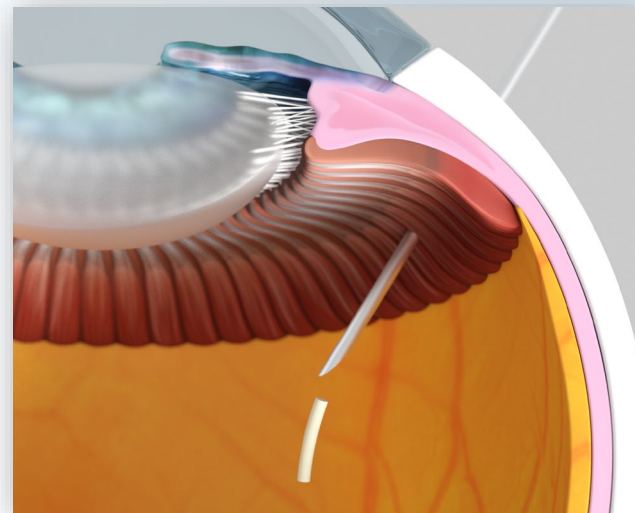
- Cohort 2 (400 µg): Several subjects demonstrated durability of therapy for up to 12 months
- Cohort 3: One subject has demonstrated durability of therapy for up to 9 months in the 600 µg group & another as demonstrated up to 6 months in the OTX-TKI + Anti-VEGF group

## ❑ Consistent bio-resorption observed

- Implant biodegraded in all subjects in Cohort 1 by 9-10.5 months

## ❑ Implant location observation suggests limited movement

- Implant has been able to be adequately monitored



**Plan to initiate US clinical trial  
in mid-2021**



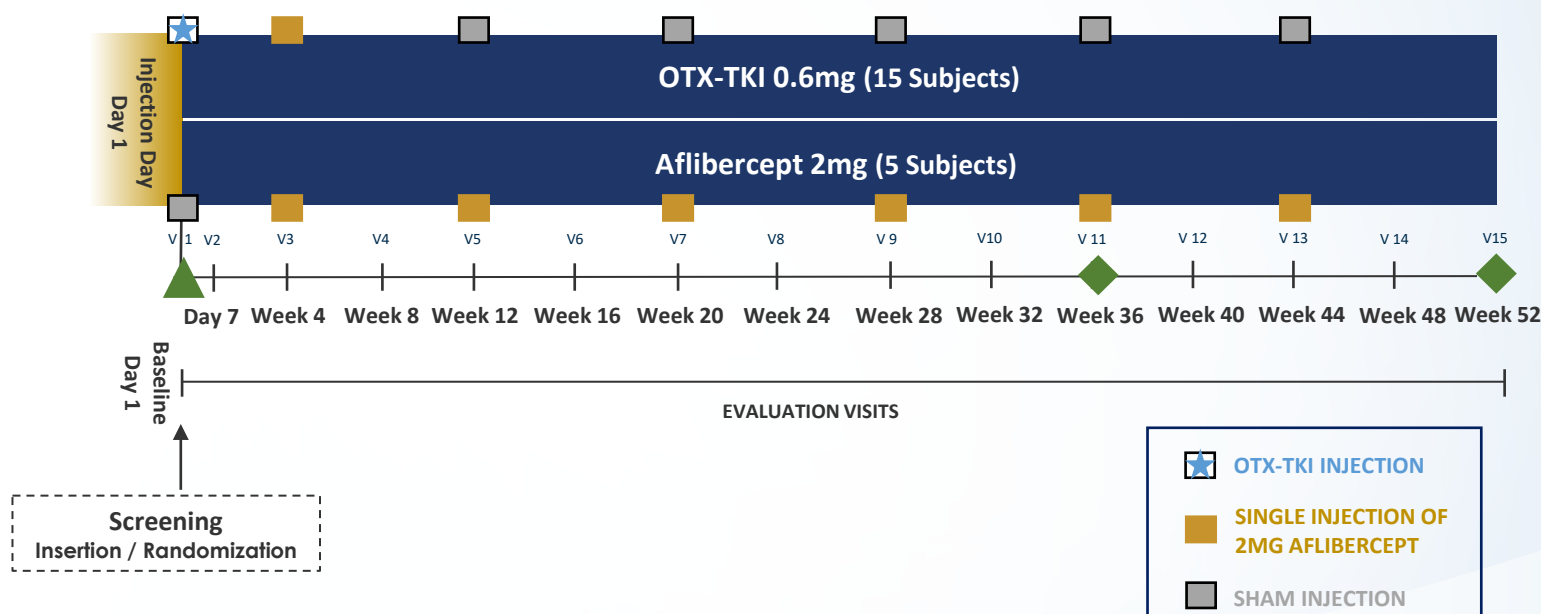
# OTX-TKI US STUDY

## DESIGN

- Prospective, multi-center, double-masked, parallel-group study
- Approximately 5 US sites
- One eye treated per patient
- Key Inclusion criteria:
  - Previously treated anti-VEGF injection

## OBJECTIVES

- Safety, tolerability, durability and biological activity
- BCVA, mean change in central subfield thickness (CSFT) measured by SD-OCT and safety evaluations at all visits



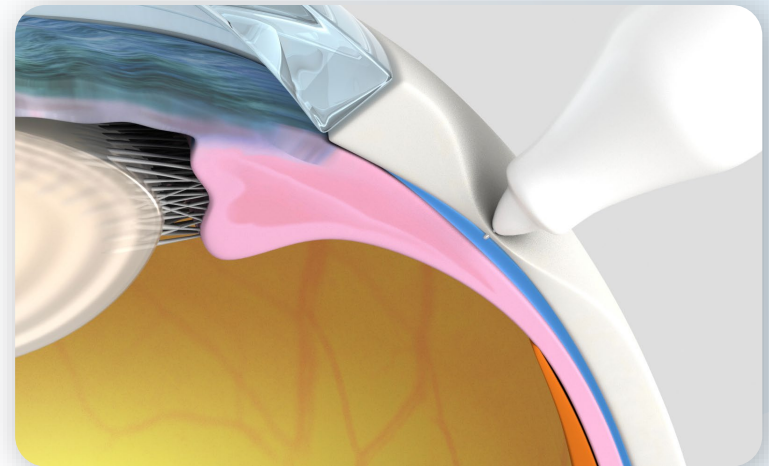
# REGENERON PARTNERSHIP

## OTX-AFS (AFLIBERCEPT SUPRACHOROIDAL INJECTION)\*

**REGENERON**

### AMENDED AGREEMENT TO DEVELOP A NOVEL, SUSTAINED-RELEASE FORMULATION OF EYLEA® (AFLIBERCEPT)

- **EYLEA is a vascular endothelial growth factor (VEGF) trap approved for the treatment of wet age-related macular degeneration (wet AMD) and other serious retinal diseases**
  - EYLEA is the global market leader with \$7.5 billion in revenue in 2019<sup>1</sup>
- **Evaluating opportunity to incorporate aflibercept with our sustained release hydrogel for injection in the suprachoroidal space**
  - Goal is to overcome limitations of intravitreal injections and extend aflibercept's duration of activity, thereby decreasing dosing frequency
- **Deal parameters**
  - Regeneron subsidizes Ocular's formulation efforts
  - Regeneron to fund personnel and material costs associated with pre-clinical development
  - Regeneron to fund up to \$305 million in milestone payments with royalties in high single digits to low-to-mid-teens as a % of net sales
  - Includes only large molecule anti-VEGFs

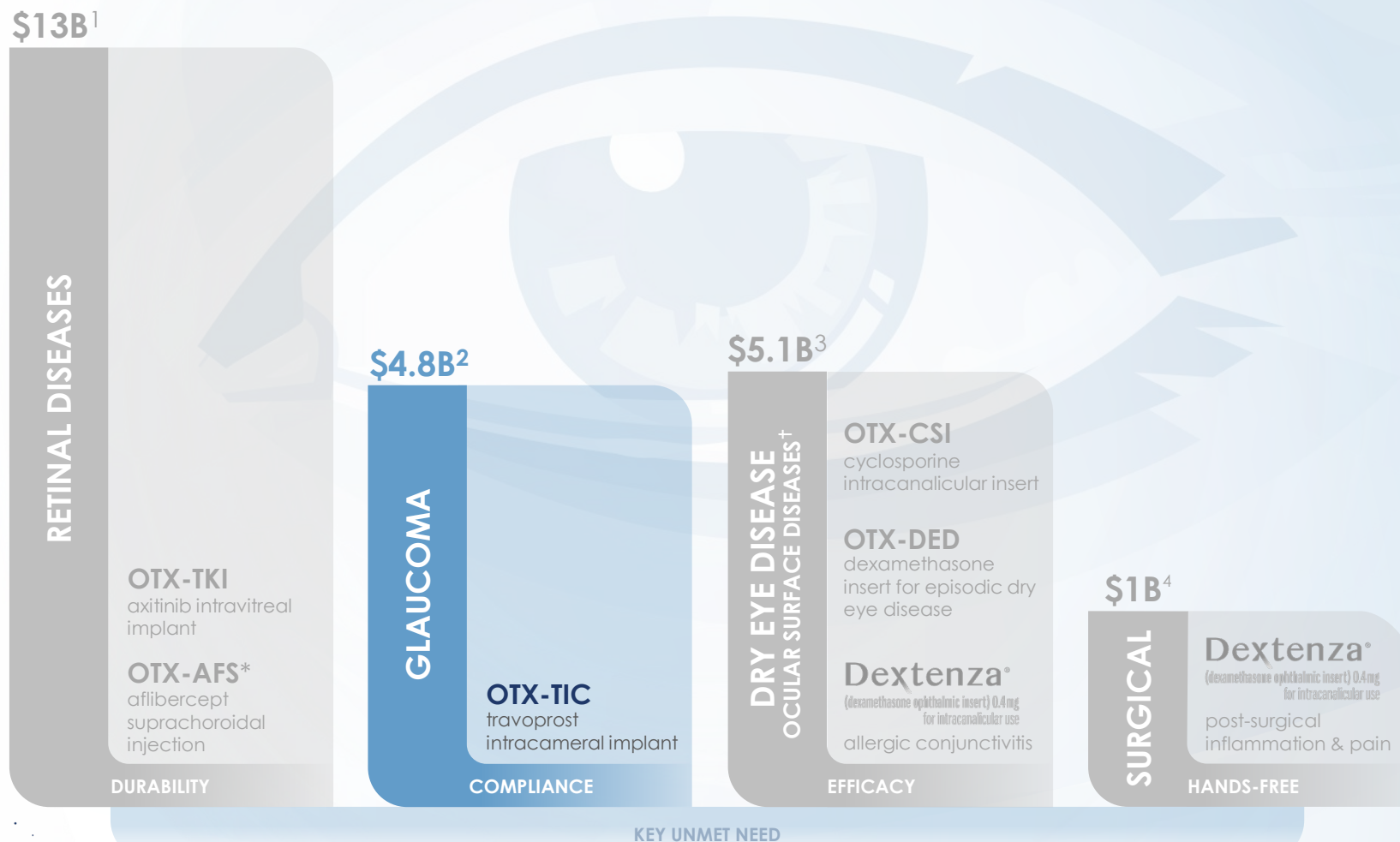


1. 2019 Regeneron annual report

\*Formerly known as OTX-IVT

# TOTAL GLOBAL MARKETS

DEVELOPING PRODUCTS WITH THE POTENTIAL TO BECOME A STANDARD OF CARE FOR SELECT INDICATIONS IN SEVERAL OF THE LARGEST SEGMENTS IN OPHTHALMOLOGY



These data reflect the total global market for the respective indications. Our market opportunity for such indications reflects a portion of this market.

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# OTX-TIC (TRAVOPROST INTRACAMERAL IMPLANT)

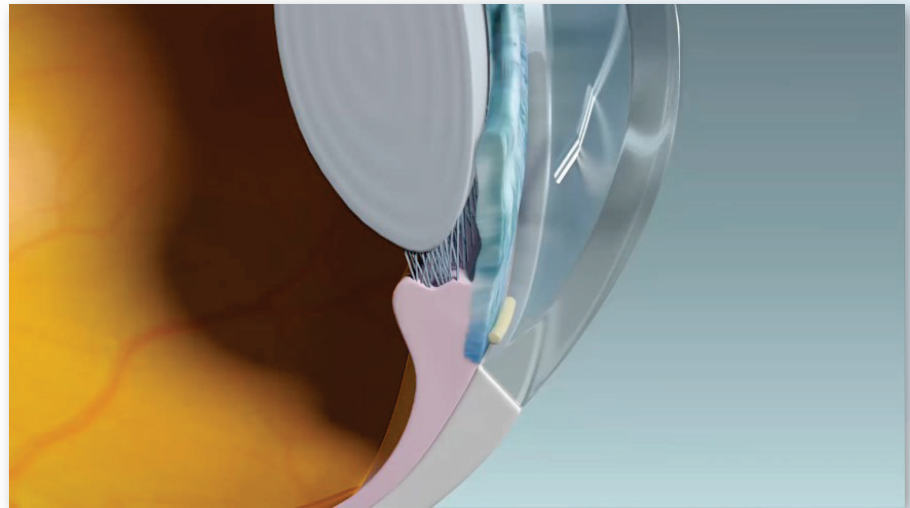
## ADDRESSES THE ISSUE OF PATIENT NON-COMPLIANCE WITH EYE DROPS

### ISSUES WITH EXISTING TREATMENTS

- High rates of non-adherence to glaucoma therapies
- Poor adherence has been shown to be associated with disease progression and blindness<sup>1,2</sup>
- Ocular hyperemia
- Life-long daily burden of patient administration

### KEY PRODUCT ATTRIBUTES

- Travoprost loaded microparticles embedded in hydrogel
- Administered with 27G or 26G needle
- Resides in the iridocorneal angle
- Fully biodegradable
- Preservative-free



1. Rossi GC, et al. Do adherence rates and glaucomatous visual field progression correlate? Eur J Ophthalmol. 2011; 21:410–4. 2. Sleath B, et al. The relationship between glaucoma medication adherence, eye drop technique, and visual field defect severity. Ophthalmology. 2011; 118:2398–402.

## Phase 1 Study Design

- ## Objectives

- ### Active Comparator:

- ## PHASE 1 TRIAL NOW FULLY ENROLLED



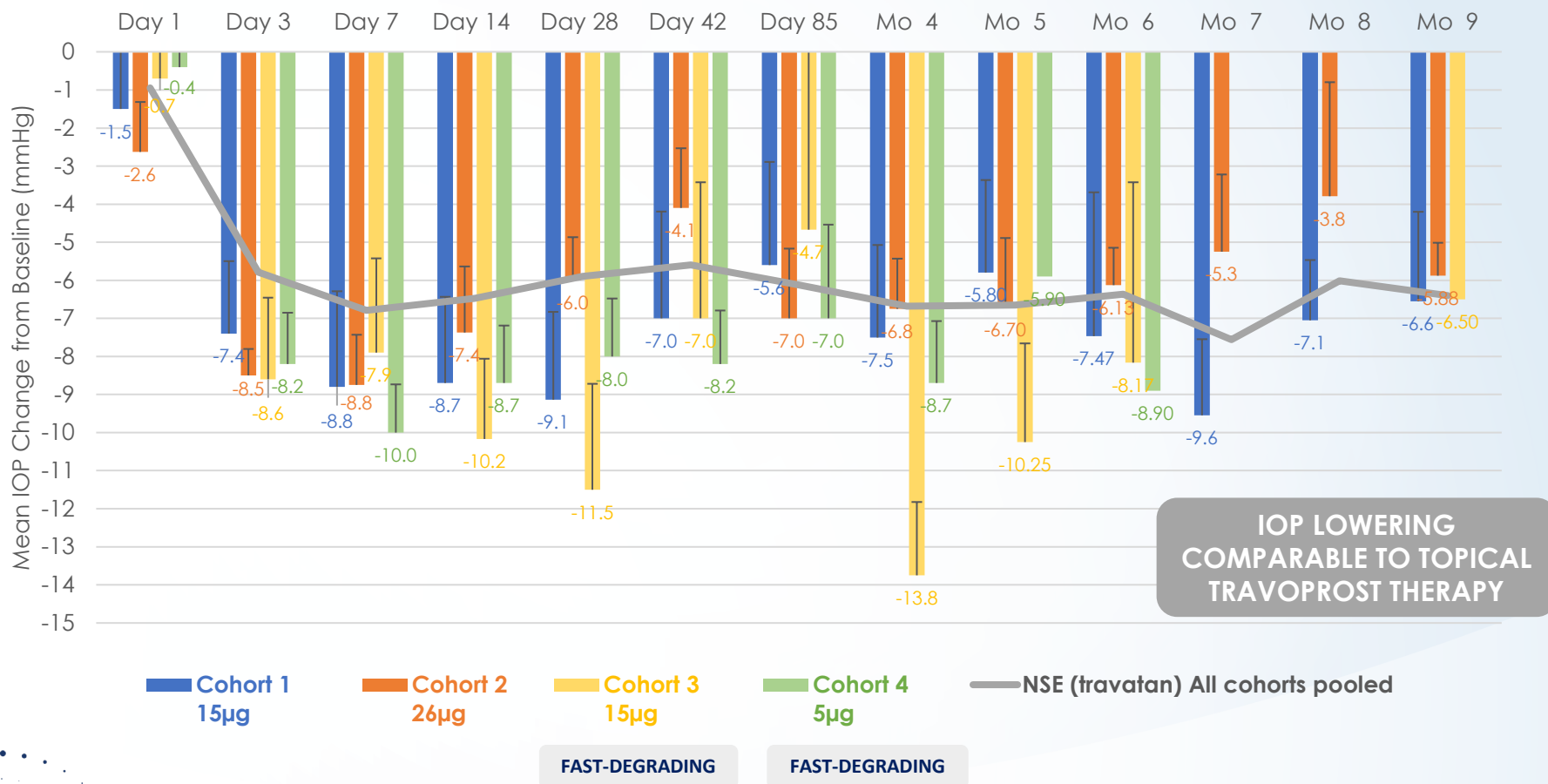
### Cohort 2: 26µg

**Cohort 3: 15μg**  
[Fast Degrading Hydrogel]

### Cohort 4: 5 $\mu$ g [Fast Degrading Hydrogel]

# ALL COHORTS: MEAN IOP CHANGE FROM BASELINE

IOP DECREASED AFTER 2 DAYS FOLLOWING OTX-TIC IMPLANTATION & LOWERING TO 7-11 MMHG RECORDED



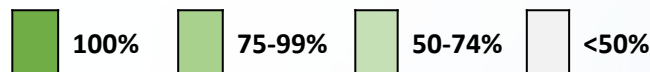
NB: Interim look as of 04/12/2021. Unmonitored data (8AM measurements). If the study eye was given other IOP lowering medication, the IOP value was removed from the analysis

# ALL COHORTS: DURATION OF EFFECT WITH ONE IMPLANT

**COHORT 2 SHOWED THE MOST CONSISTENT DURABLE RESPONSE IN ALL SUBJECTS UP TO MONTH 6 & 50% OF SUBJECTS UP TO MONTH 9**

	Day 42 % (n/N)	Day 85 % (n/N)	Month 4 % (n/N)	Month 5 % (n/N)	Month 6 % (n/N)	Month 7 % (n/N)	Month 8 % (n/N)	Month 9 % (n/N)	Month 10-22 % (n/N)
<b>Cohort 1 (15 µg) N=5</b>	100(5/5)	100(5/5)	80(4/5)	80(4/5)	60(3/5)	40 (2/5)	40 (2/5)	40 (2/4)	20 (1/5)
<b>Cohort 2 (26 µg) N=4</b>	100(4/4)	100(4/4)	100(4/4)	100(4/4)	100(4/4)	100(4/4)	75(3/4)	50(2/4)	NA
<b>Cohort 3 (15 µg) (Fast-degrading) N=5</b>	100(5/5)	60(3/5)	40 (2/5)	40 (2/5)	40 (2/5)	20 (1/5)	20 (1/5)	20 (1/5)	NA
<b>Cohort 4 (5 µg) (Fast-degrading) N=5<sup>‡</sup></b>	100(5/5)	100(5/5)	80(4/5)	75(3/4) ‡	75(3/4) ‡	NA	NA	NA	NA
<b>Total<sup>‡</sup></b>	100 (18/18)	89 (16/18)	72 (13/18)	72 (13/18)	63 (10/16)	50 (7/14)	43 (6/14)	39 (5/13)	20 (1/5)

<sup>‡</sup>Last subject in Cohort 4 past Month 4 timepoint so far and follow-up is ongoing

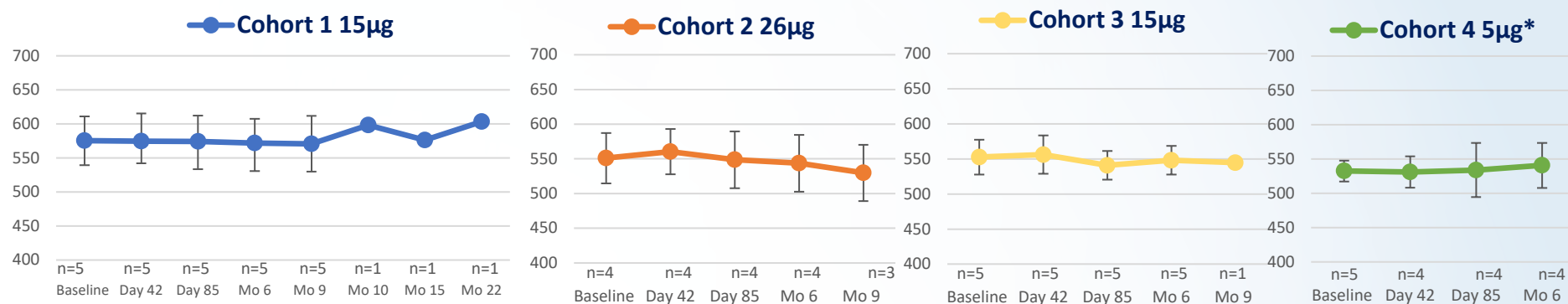




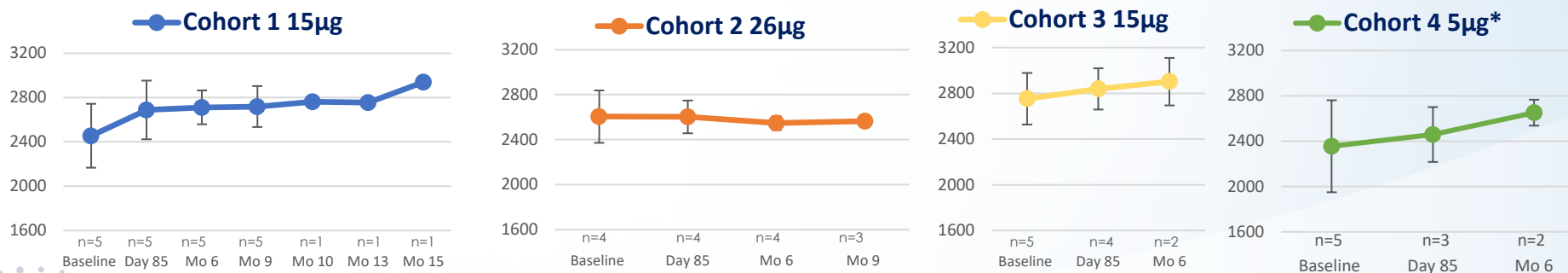
# ALL COHORTS: NO EFFECT OBSERVED ON CORNEAL HEALTH

## PACHYMETRY & ENDOTHELIAL CELL COUNTS INDICATE NO CLINICALLY-MEANINGFUL CHANGE FROM BASELINE

### PACHYMETRY ( $\mu\text{m}$ )



### ENDOTHELIAL CELL COUNTS (AUTOMATED)

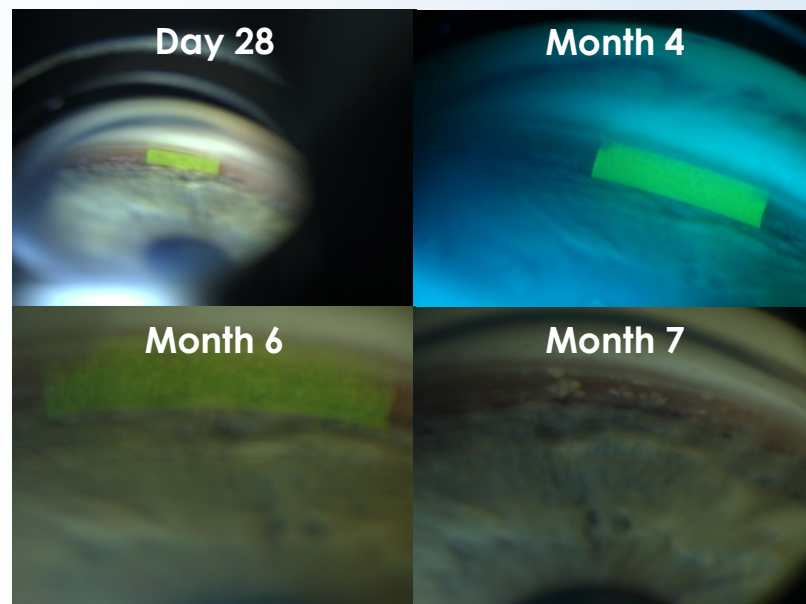


NB: Interim look as of 04/12/2021; Unmonitored data.\*Cohort 4: Ongoing follow-up

# OTX-TIC PHASE 1 INTERIM FINDINGS

- ✓ **Clinically-meaningful decrease in IOP**  
Mean IOP values were decreased in patients receiving OTX-TIC as early as two days following administration, and mean IOP decrease was comparable to topical travoprost therapy
- ✓ **Extended duration of therapy**  
Many subjects exhibited 6+ months duration of IOP-lowering effect in Cohorts 1 & 2, and between 3-6 months in Cohorts 3 & 4 (fast degrading implant) with a single implant; Longest and most consistent IOP lowering in Cohort 2
- ✓ **Consistently bioresorbable**  
Implant biodegraded in 5-7 months (Cohorts 1 & 2); Fast degrading implants biodegraded in 3-5 months (Cohorts 3 & 4)
- ✓ **Implant location and limited movement**  
Implant was not observed to move at slit lamp and was visible at all exams in all patients using gonioscopy
- ✓ **Corneal health**  
Endothelial cell counts, pachymetry assessments, and slit lamp examinations indicate no changes from baseline

## VISUALIZATION OF IMPLANT

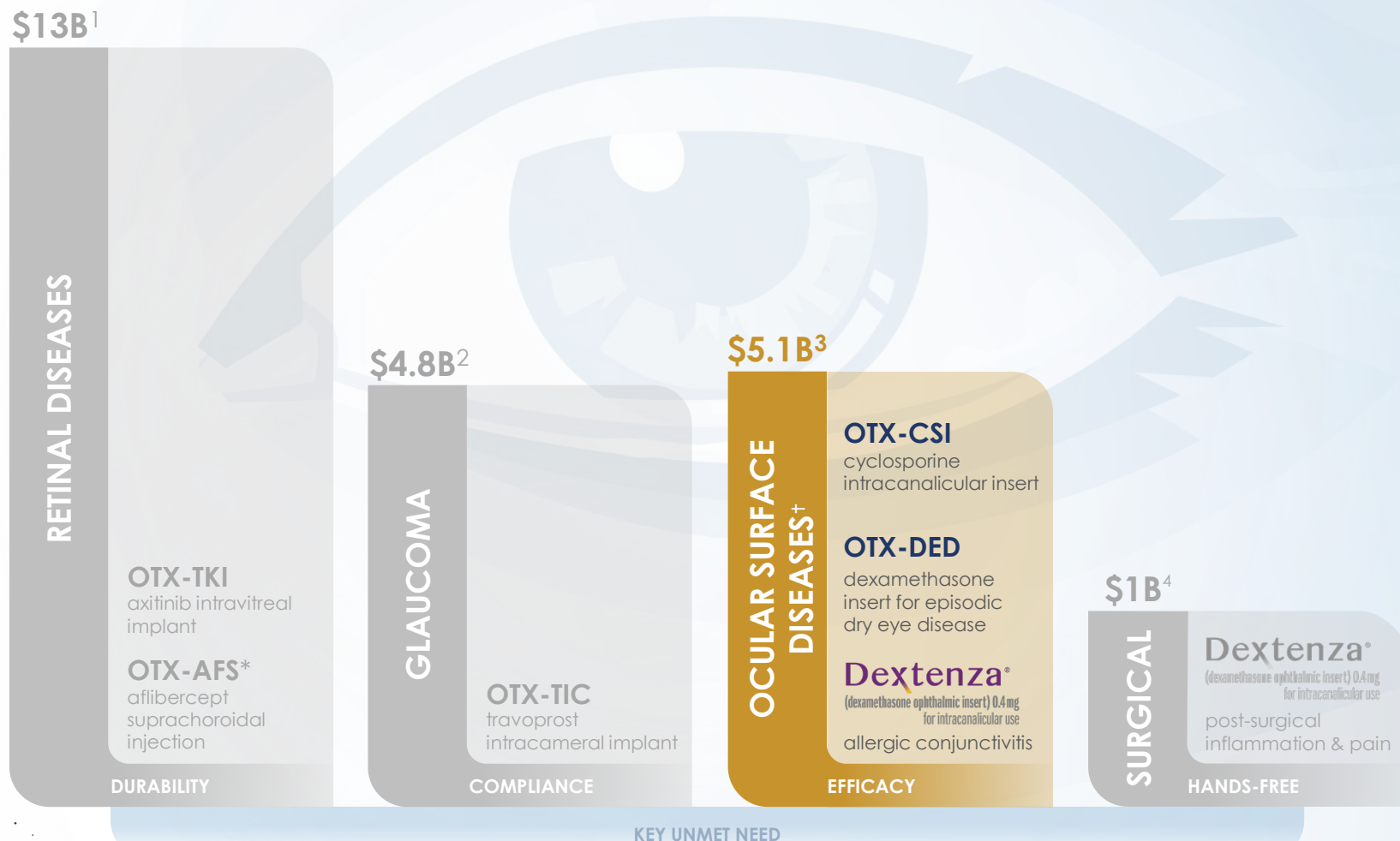


**Plan to initiate Phase 2 clinical trial in Q4 2021**



# TOTAL GLOBAL MARKETS

DEVELOPING PRODUCTS WITH THE POTENTIAL TO BECOME A STANDARD OF CARE FOR SELECT INDICATIONS IN SEVERAL OF THE LARGEST SEGMENTS IN OPHTHALMOLOGY



These data reflect the total global market for the respective indications. Our market opportunity for such indications reflects a portion of this market.

\* In collaboration with REGENERON; † Data shown here is only representative of Dry Eye and not other Ocular Surface Diseases

1. 2019 Retina Pharma Market Scope Report 2. 2019 Glaucoma Pharma Market Scope Report 3. 2019 Dry Eye Market Scope Report 4. Estimated using historical costs of topical eyedrops (not DEXTENZA) and the total addressable market based on the total US ocular surgical steroid market value 2019

# INTRACANALICULAR INSERTS

AN INNOVATION IN DRUG DELIVERY TO THE OCULAR SURFACE



Dexamethasone bathes the ocular surface





# OTX-CSI (CYCLOSPORINE INTRACANALICULAR INSERT)

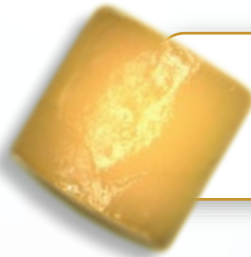
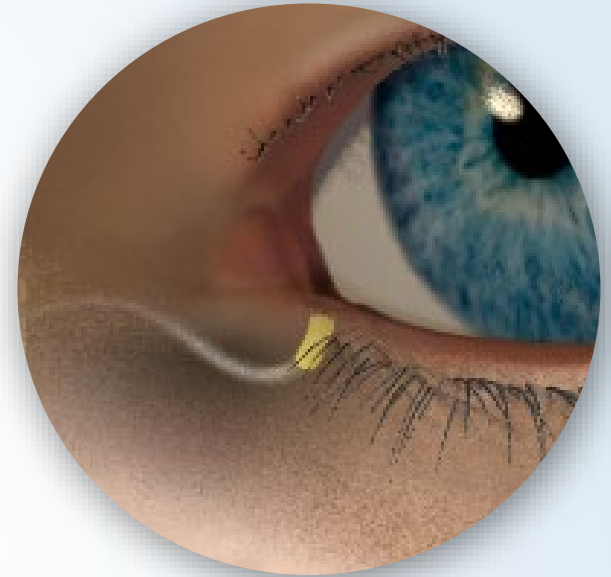
## SUSTAINED RELEASE THERAPY FOR DRY EYE DISEASE

### ISSUES WITH EXISTING TREATMENTS

- Slow onset of action for therapy
- High level of burning, stinging and irritation upon administration
- Burden of patient administration

### KEY PRODUCT ATTRIBUTES

- Cyclosporine loaded in hydrogel
- Preservative-free
- Designed to deliver therapy up to 12 weeks with a single insert
- Occludes the punctum
- Fully biodegradable insert



**INITIATED OTX-CSI PHASE 2  
TRIAL IN Q3 2020**



# PHASE 1 STUDY OBJECTIVE AND DESIGN

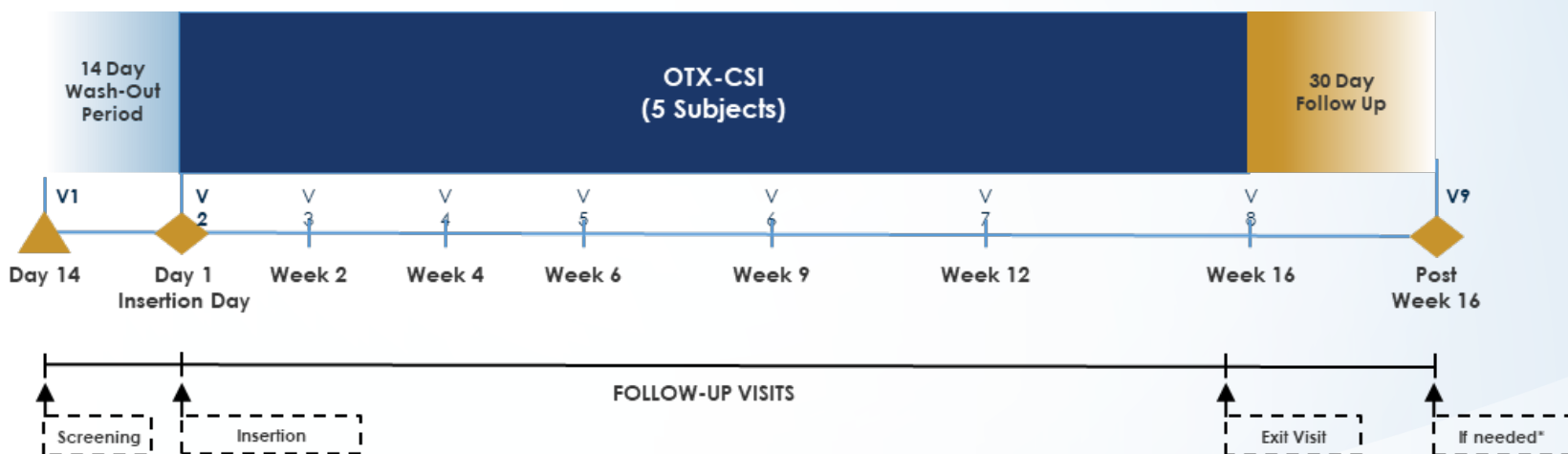
## OBJECTIVE: EVALUATING THE SAFETY, TOLERABILITY, AND EFFICACY OF OTX-CSI FOR THE TREATMENT OF SUBJECTS WITH DRY EYE DISEASE

### Design

- Phase 1, Prospective, Open-label study
- Key Inclusion criteria:
  - DED diagnosis in both eyes for  $\geq 6$  months
  - VAS eye dryness severity score  $\geq 30$

### Endpoints

- Schirmer Test (without anesthesia) at Week 12
- Eye Dryness Score (visual analogue scale [VAS])
- Total Corneal Fluorescein Staining (tCFS) using NEI scale
- Presence of OTX-CSI or HV insert at all post-baseline visits
- Adverse Events (Ocular and Non-ocular)

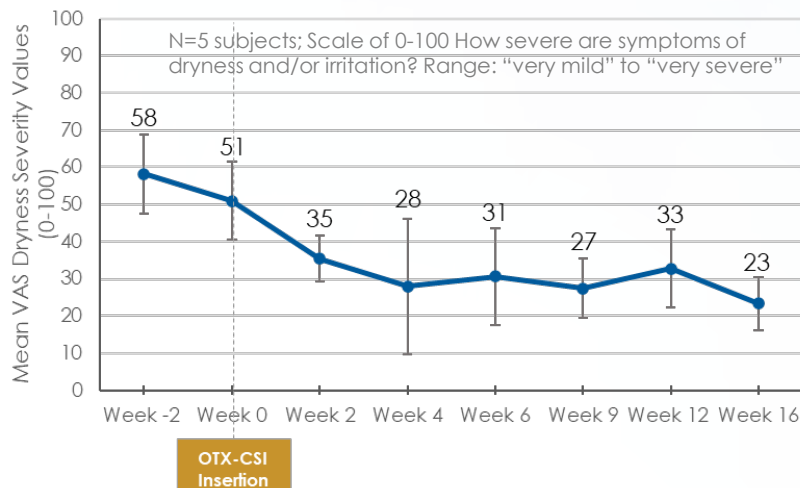


\*Subject remains in study until insert is no longer visible and no evidence of biological activity

# OTX-CSI PHASE 1 TRIAL RESULTS

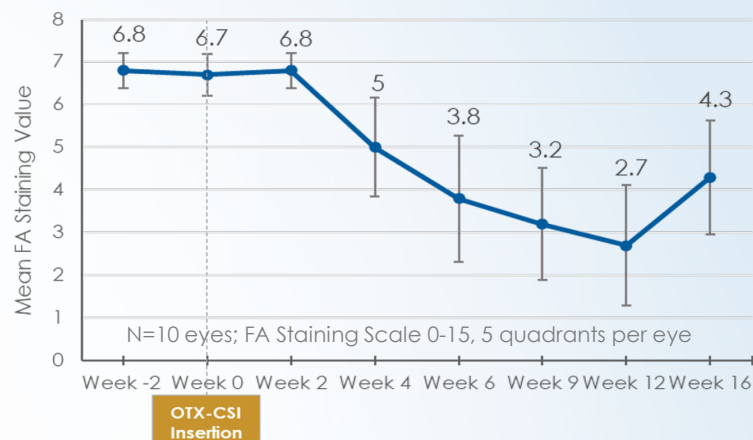
## SUBJECTS REPORTED IMPROVEMENT IN DRYNESS SEVERITY ON A SCALE OF 0-100 (VERY MILD TO VERY SEVERE) OVER 16 WEEKS

### Mean Absolute Values

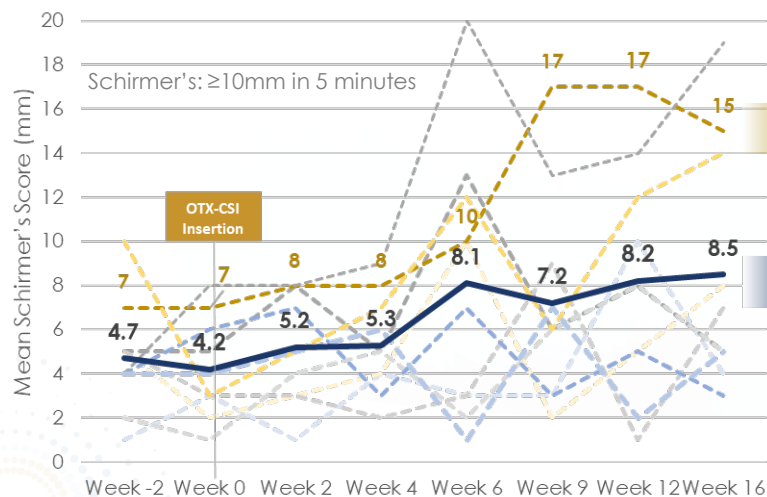


## IMPROVED TOTAL CORNEAL FLUORESCEIN STAINING VALUES WEEK 4 TO 16 COMPARED TO BASELINE

### Mean Absolute Values



## SUBJECTS SHOWED AN IMPROVEMENT IN MEAN SCHIRMER'S TEST SCORES FROM WEEK 0 TO WEEK 16



20% (1/5) of subjects showed  $\geq 10$ mm increase from baseline (7 mm at Week 0 to 17 mm at Week 12)

Mean Schirmer's Score improved from 4.2mm at Week 0 to 8.2mm at Week 12

--- Individual Eye Data (N=10 eyes)

— Mean Data

**OTX-CSI WAS GENERALLY OBSERVED TO HAVE A FAVORABLE SAFETY PROFILE & WAS WELL TOLERATED**

No AEs of stinging, burning, irritation, tearing, or blurred vision were reported over the 16-week period

# PHASE 2 STUDY OBJECTIVE AND DESIGN

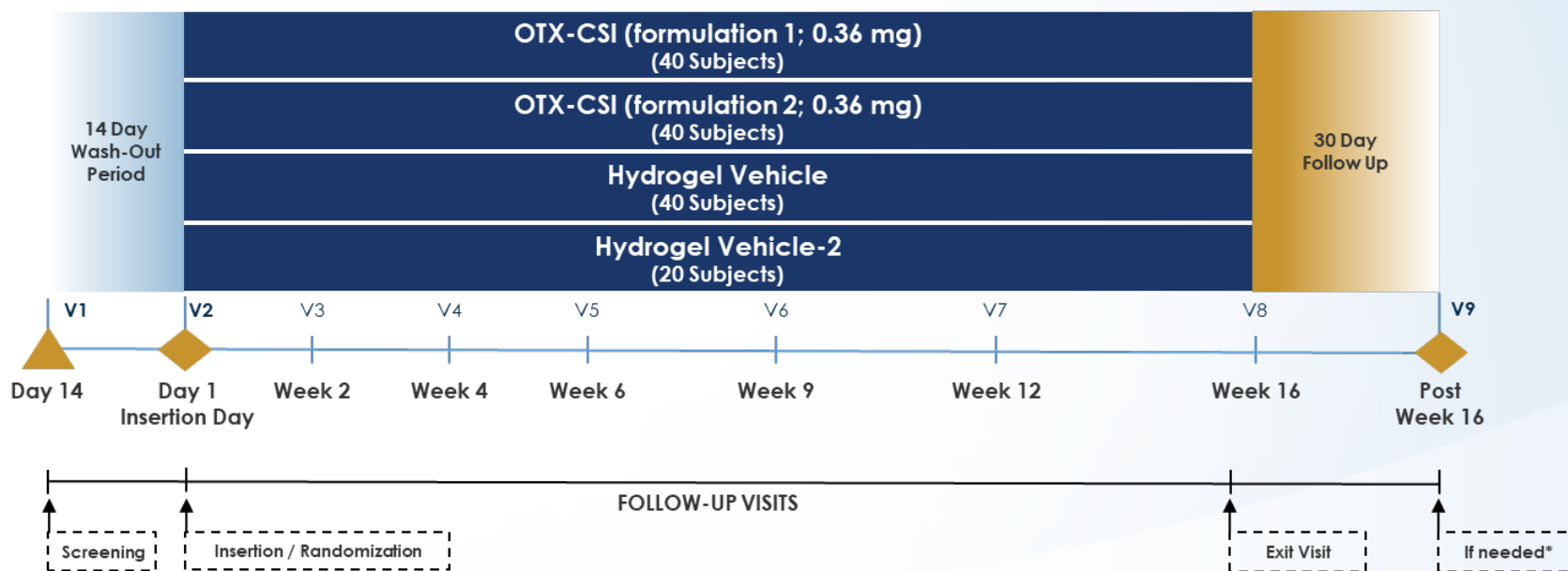
## OBJECTIVE: EVALUATING THE SAFETY, TOLERABILITY, AND EFFICACY OF OTX-CSI FOR THE TREATMENT OF SUBJECTS WITH DRY EYE DISEASE

### Design

- Prospective, Randomized, Double-Masked, Vehicle-controlled study
- Key Inclusion criteria:
  - DED diagnosis in both eyes for  $\geq 6$  months
  - VAS eye dryness severity score  $\geq 30$

### Endpoints

- Schirmer Test (without anesthesia) at Week 12
- Eye Dryness Score (visual analogue scale [VAS])
- Total Corneal Fluorescein Staining (tCFS) using NEI scale
- Presence of OTX-CSI or HV insert at all post-baseline visits
- Adverse Events (Ocular and Non-ocular)



\*Subject remains in study until insert is no longer visible and no evidence of biological activity

Study to Evaluate the Safety, Tolerability, and Efficacy of OTX-CSI in Subjects With Dry Eye Disease. ClinicalTrials.gov.  
<https://clinicaltrials.gov/ct2/show/NCT04362670>. Accessed October 16, 2020.

Fully enrolled



# OTX-DED (DEXAMETHASONE INTRACANALICULAR INSERT)

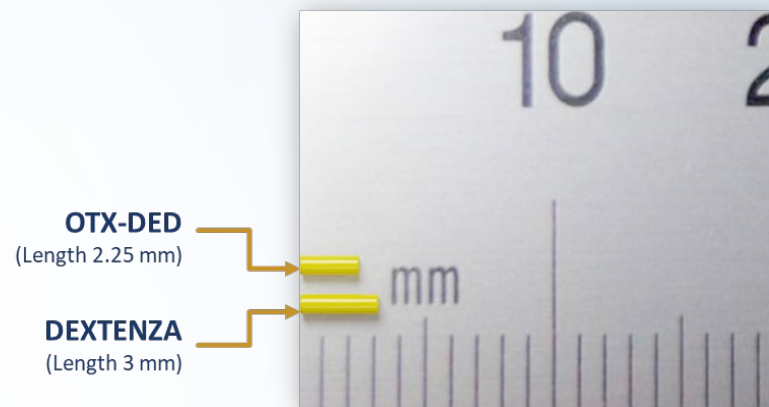
## OFF-LABEL STEROIDS ARE CURRENTLY USED TO TREAT EPISODIC DRY EYE

### ISSUES WITH EXISTING TREATMENTS

- Approved therapies for the chronic treatment of DED are known for slow onset of action & burning/stinging upon application
- Topical steroids (which are not FDA-approved for DED except for the recently FDA approved Eysuvis™-Kala Pharmaceuticals, Inc)<sup>1</sup> can be abused and contain preservatives causing ocular toxicity

### KEY PRODUCT ATTRIBUTES

- Dexamethasone loaded in hydrogel
- Preservative-free
- Occludes the canaliculus providing more rapid onset of action
- Fully biodegradable insert
- Leverages strong safety profile of DEXTENZA®



Rendering showing OTX-DED is shorter in length than DEXTENZA

**First Patient Dosed in Phase 2 Clinical Trial in Q1 2021**

1. Kala Pharmaceuticals, Inc. Accessed March 5, 2021. <https://investors.kalarx.com/news-releases/news-release-details/kala-pharmaceuticals-announces-fda-approval-eyvisuvis-short-term/>

# PHASE 2 STUDY OBJECTIVE AND DESIGN

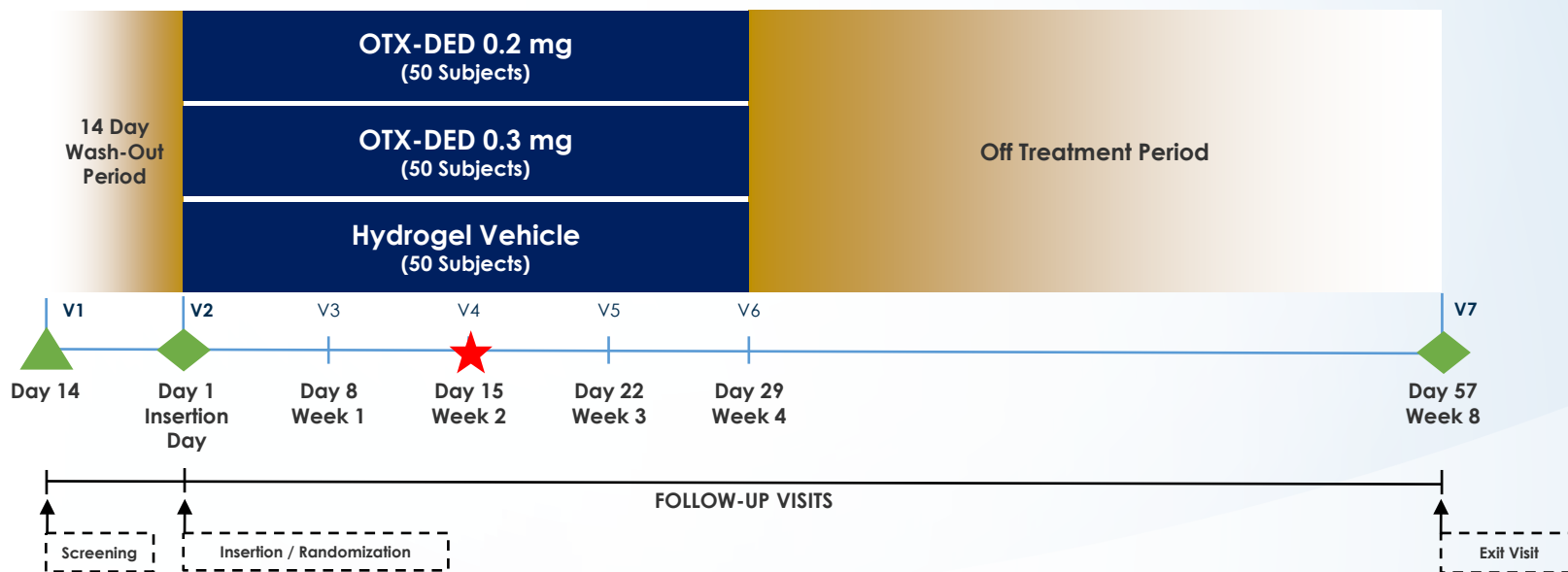
## OBJECTIVE: EVALUATING THE EFFICACY AND SAFETY OF OTX-DED FOR THE SHORT-TERM TREATMENT OF SIGNS AND SYMPTOMS OF DRY EYE DISEASE

### Design

- Prospective, Randomized, Double-Masked, Vehicle-controlled study
- Key Inclusion criteria:
  - DED diagnosis in both eyes for  $\geq 6$  months
  - VAS eye dryness severity score  $\geq 30$
  - Bulbar conjunctival hyperemia grade  $\geq 2$  (CCLRU scale)

### Endpoints

- Conjunctival Hyperemia at Week 2
- Eye Dryness Score (visual analogue scale [VAS])
- Total Corneal Fluorescein Staining (tCFS) using NEI scale
- Adverse Events (Ocular and Non-ocular)



# DEXTENZA FOR THE TREATMENT OF ALLERGIC CONJUNCTIVITIS

## AN IN-OFFICE INDICATION FOR DEXTENZA

### ISSUES WITH EXISTING TREATMENTS

- Corticosteroids are effective in treating both signs and symptoms of acute and chronic allergy
- Corticosteroids are not often prescribed due to the ability to abuse and/or overuse the treatment
- Treatment requires frequent administration of eyedrops, and hands touching the face several times per day

### KEY PRODUCT ATTRIBUTES

- A non-abusable formulation
- Preservative-free
- Leverages strong safety profile for DEXTENZA in the treatment inflammation and pain following ophthalmic surgery

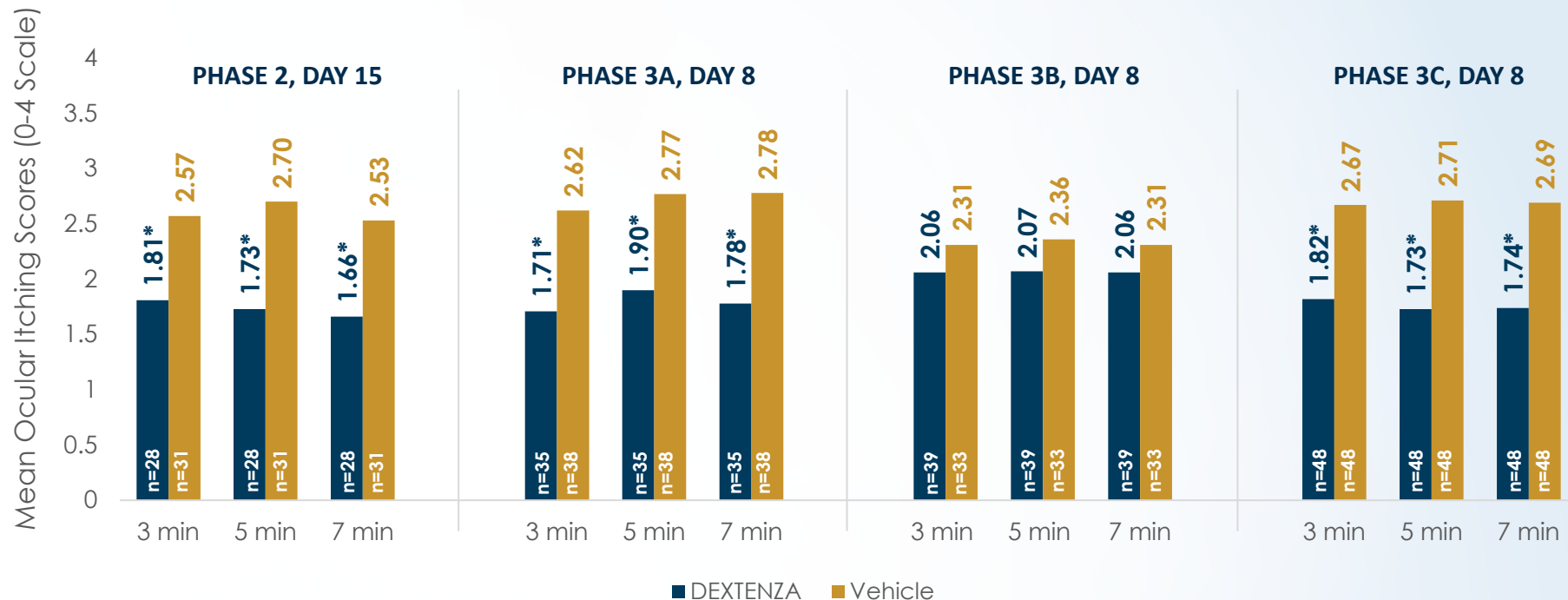
**Dextenza**<sup>®</sup>  
(dexamethasone ophthalmic insert) 0.4 mg  
for intracanalicular use



**PDUFA Date for sNDA: 18 October 2021**



# RESULTS: PRIMARY EFFICACY ENDPOINT MEAN OCULAR ITCHING SCORES ACROSS ALL STUDIES

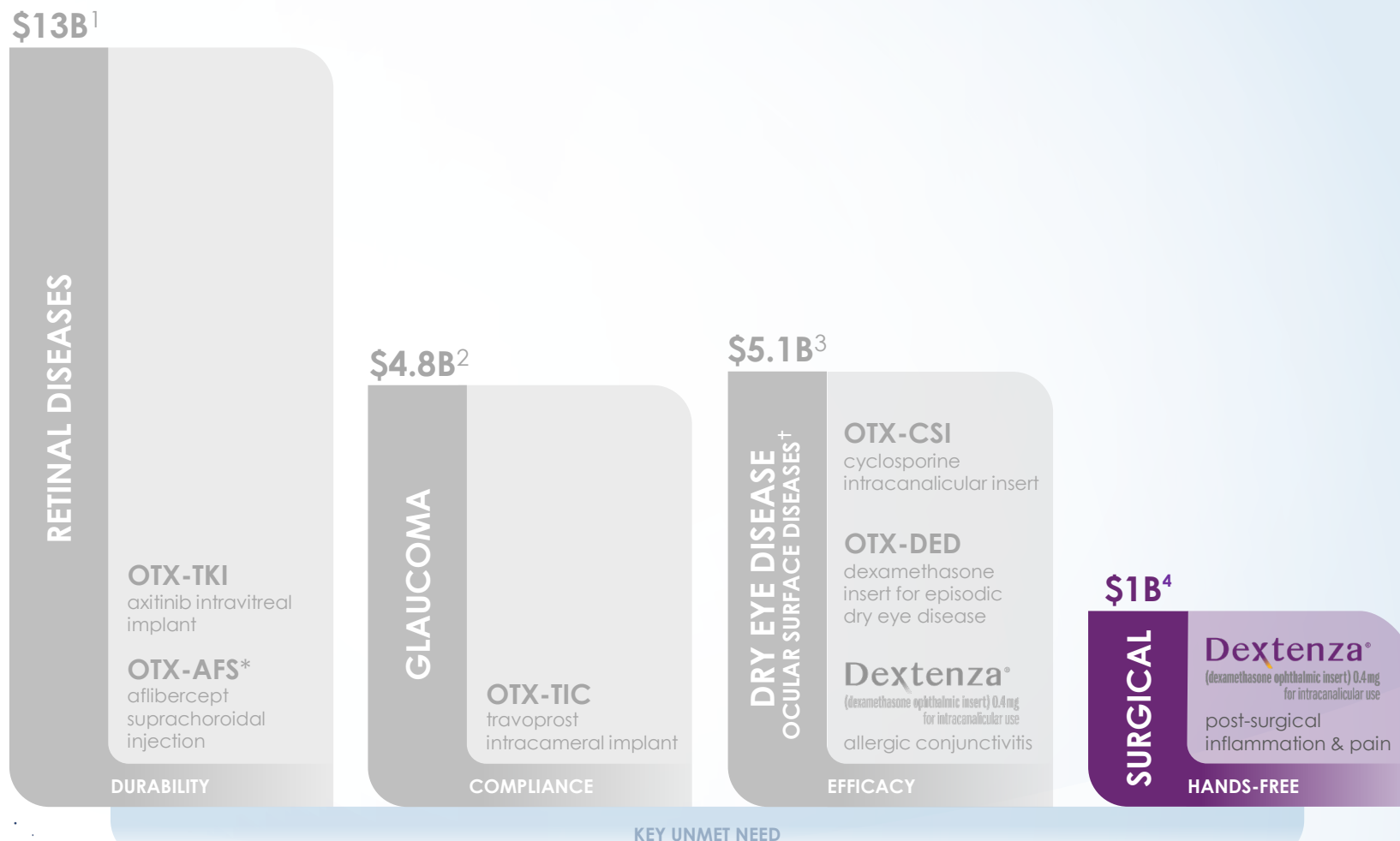


\*Statistically Significant;  $P \leq 0.0025$ ; Population: ITT + LOCF (Phase 2) & ITT + MCMC (subject level imputation, Phase 3)



# GLOBAL MARKET VALUES

DEVELOPING PRODUCTS WITH THE POTENTIAL TO BECOME A STANDARD OF CARE FOR SELECT INDICATIONS IN SEVERAL OF THE LARGEST SEGMENTS IN OPHTHALMOLOGY



These data reflect the total global market for the respective indications. Our market opportunity for such indications reflects a portion of this market.

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1. 2019 Retina Pharma Market Scope Report 2. 2019 Glaucoma Pharma Market Scope Report 3. 2019 Dry Eye Market Scope Report 4. Estimated using historical costs of topical eyedrops (not DEXTENZA) and the total addressable market based on the total US ocular surgical steroid market value 2019

# THE UNMET NEED IN TREATMENT OF PAIN AND INFLAMMATION FOLLOWING SURGERY

## EYE DROPS HAVE POOR CORNEAL RESIDENCE TIME<sup>3,4</sup>



LESS THAN  
**5%**  
OF DROPS  
REACH  
OCULAR  
TISSUE

## STERIOD DROPS ARE THE MOST COMPLEX POST-OP CATARACT TREATMENT REGIMEN

Common clinical approach: 4 weeks with taper<sup>1</sup>

	SUN	MON	TUES	WED	THURS	FRI	SAT	TAPER
Week 1	3 drops	3 drops	3 drops	3 drops	3 drops	3 drops	3 drops	~ 28 drops
Week 2	2 drops	2 drops	2 drops	2 drops	2 drops	2 drops	2 drops	~ 21 drops
Week 3	1 drop	1 drop	1 drop	1 drop	1 drop	1 drop	1 drop	~ 14 drops
Week 4	1 drop	1 drop	1 drop	1 drop	1 drop	1 drop	1 drop	~ 7 drops

~70  
DROPS<sup>2</sup>

Ocular rebound inflammation may develop secondary to rapid tapering or abrupt discontinuation of steroids<sup>3</sup>

# DEXTENZA® (DEXAMETHASONE OPHTHALMIC INSERT)

## A HANDS-FREE ALTERNATIVE TO EYE DROPS

FDA approved for the treatment of ocular inflammation and pain following ophthalmic surgery

## REIMBURSEMENT AND CODING

**Product Code:** J1096 **Procedure Code:** 0356T

- Medicare Administrative Contractor coverage provides physician reimbursement for procedure of ~\$100
- AMA granted permanent Category 1 CPT code effective Jan 2022 (applies to DEXTENZA and all future products in canaliculus)

**1**  
**INNOVATIVE  
INSERT**

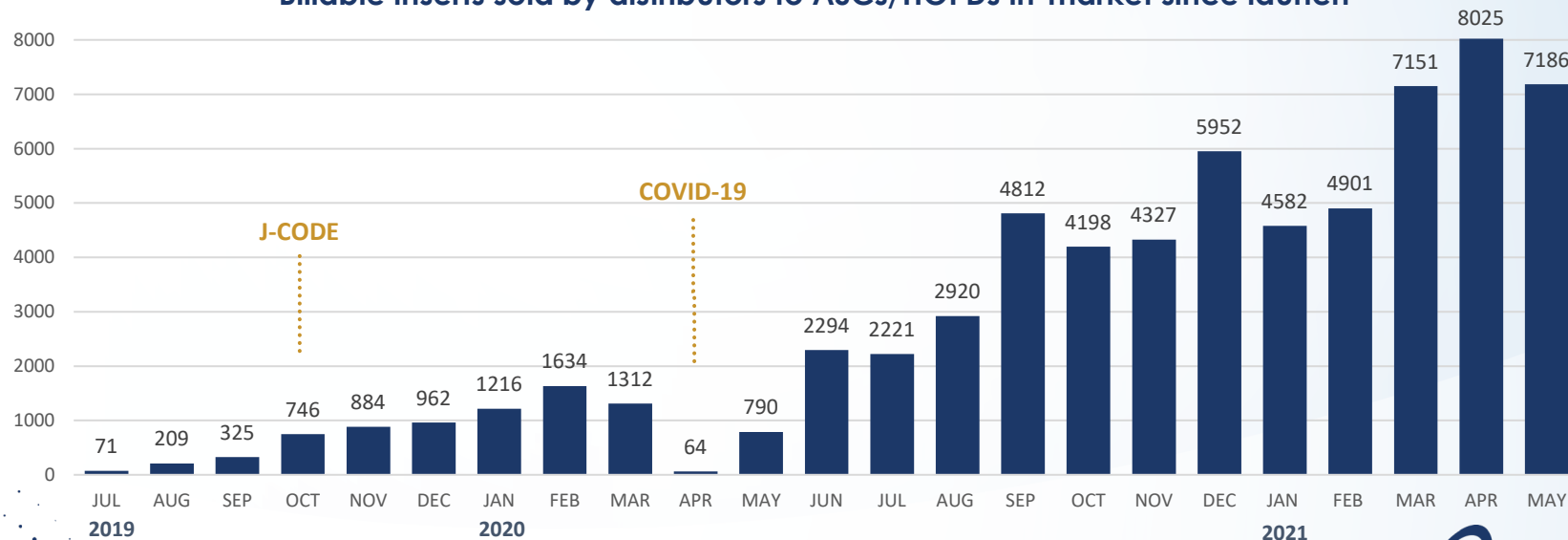
**VS**

**~70**  
**DROPS<sup>1,2</sup>**

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

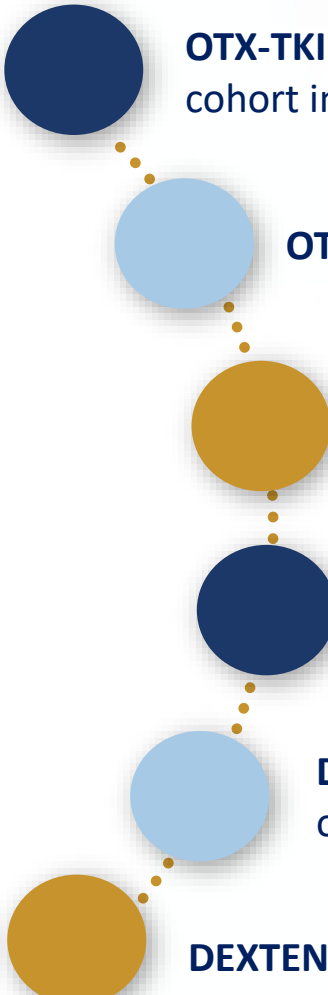
**Strong momentum continues into 2021**

**Billable inserts sold by distributors to ASCs/HOPDs in-market since launch**



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

# ANTICIPATED 2021-2022 MILESTONES



**OTX-TKI (wet AMD)** – Plan to initiate US Phase 1 clinical trial & extend a fourth cohort in Australia trial using the single 600µg implant in mid-2021

**OTX-TIC (glaucoma)** – Plan to initiate Phase 2 clinical trial in Q4 2021

**OTX-CSI (dry eye)** – Expect topline data from Phase 2 clinical trial in Q4 2021

**OTX-DED (episodic dry eye)** – Expect topline data from Phase 2 clinical trial 1H 2022

**DEXTENZA® (inflammation and pain)** – Expect to continue strong growth of in-market sales

**DEXTENZA® (allergic conjunctivitis)** – PDUFA date for sNDA: 18 October 2021



(NASDAQ: OCUL)

# TRANSFORMING DRUG DELIVERY LEVERAGING A NOVEL TECHNOLOGY PLATFORM

THANK YOU

*Ocular*  
Therapeutix™