

(NASDAQ: OCUL)

# TRANSFORMING DRUG DELIVERY

## LEVERAGING A NOVEL TECHNOLOGY PLATFORM

ANTONY MATTESSICH, CHIEF EXECUTIVE OFFICER

Ophthalmology Innovation Summit (OIS)  
Virtual Public Company Showcase  
July 16, 2020

*Ocular*  
Therapeutix™

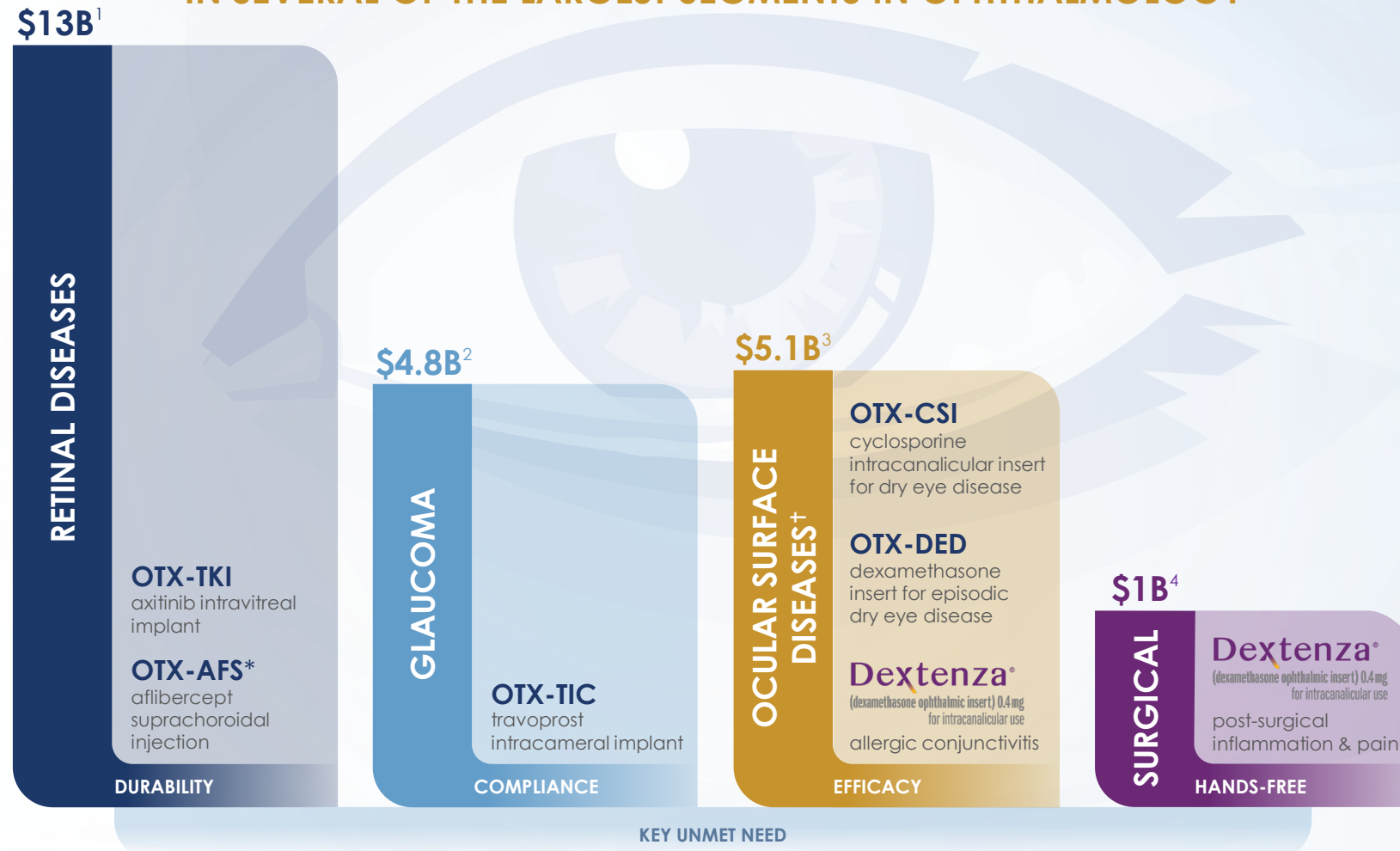
# FORWARD LOOKING STATEMENTS

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 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-DED for the treatment of temporary dry eye disease, OTX-CSI for the treatment of dry eye disease, 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-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 and other financial metrics of DEXTENZA; 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 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 contribution 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 outcome of the Company's ongoing legal proceedings, the severity and duration of the COVID-19 pandemic including its effect on the Company's and relevant regulatory authorities' operations, the 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.



# 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.

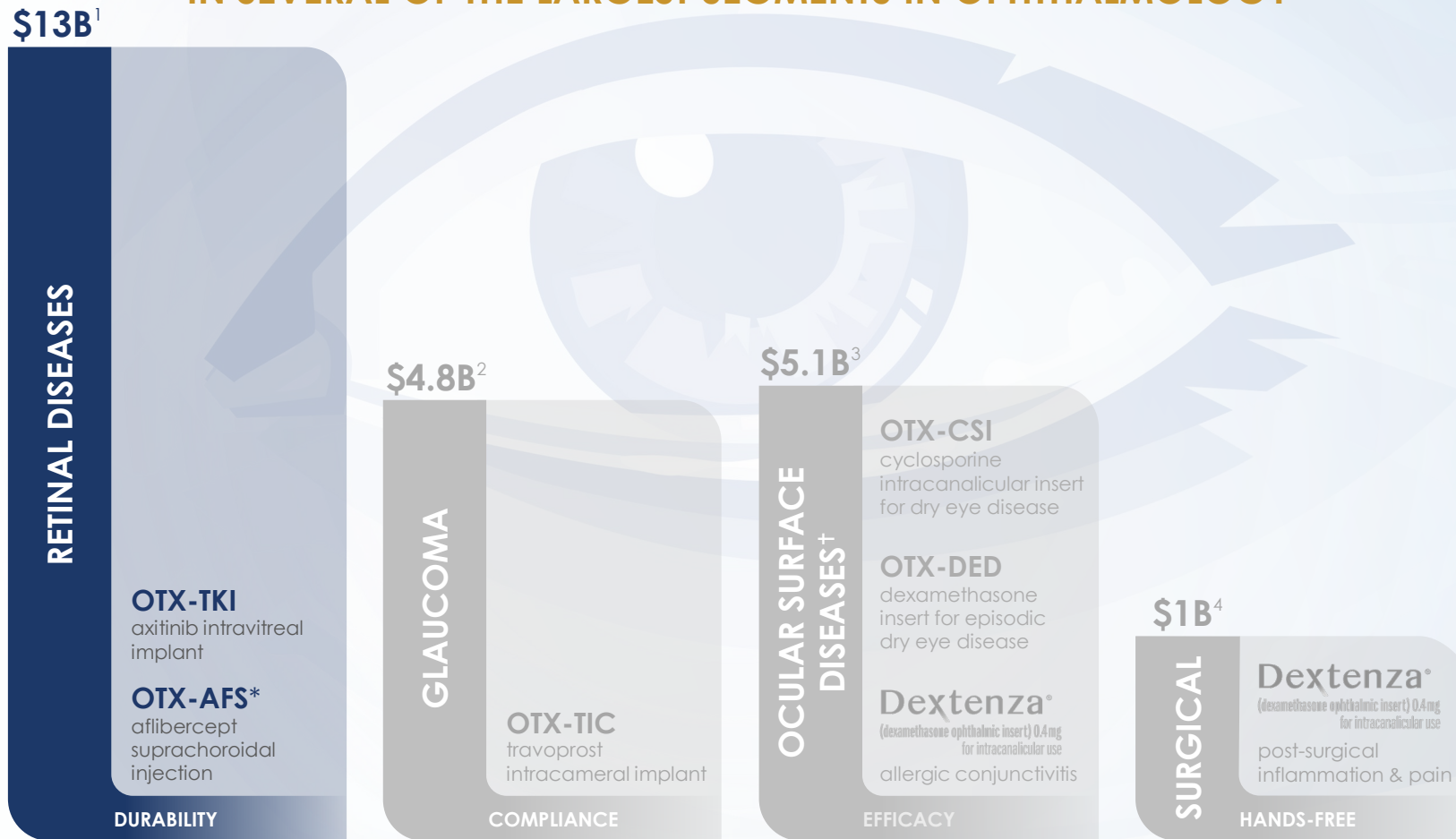
\* In collaboration with REGENERON; † Data shown here is only representative of Dry Eye and not other Ocular Surface Diseases

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

# TOTAL GLOBAL MARKETS

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# OTX-TKI (AXITINIB INTRAVITREAL IMPLANT)

## SUSTAINED RELEASE THERAPY FOR RETINAL DISEASES

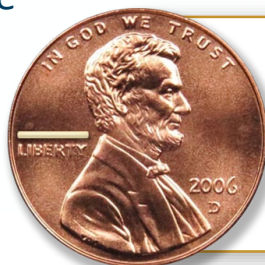
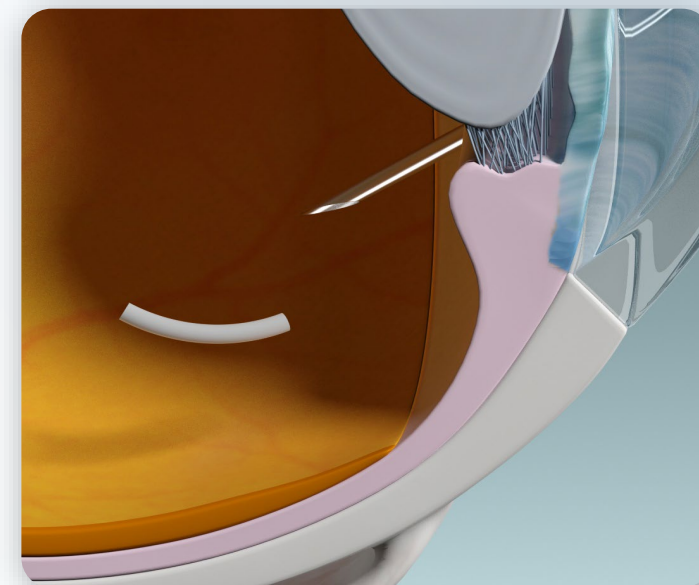
Plan to provide Phase 1 clinical update at AAO (Nov 2020) and file for US exploratory IND in 2020

### 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 with injections for patients<sup>3</sup>

### KEY PRODUCT ATTRIBUTES

- Targeting sustained release for 4.5-6 months
- Broader anti-angiogenic profile (small molecule)
- Small fiber (27-30G needle) with minimal/no visual impact
- Preservative-free



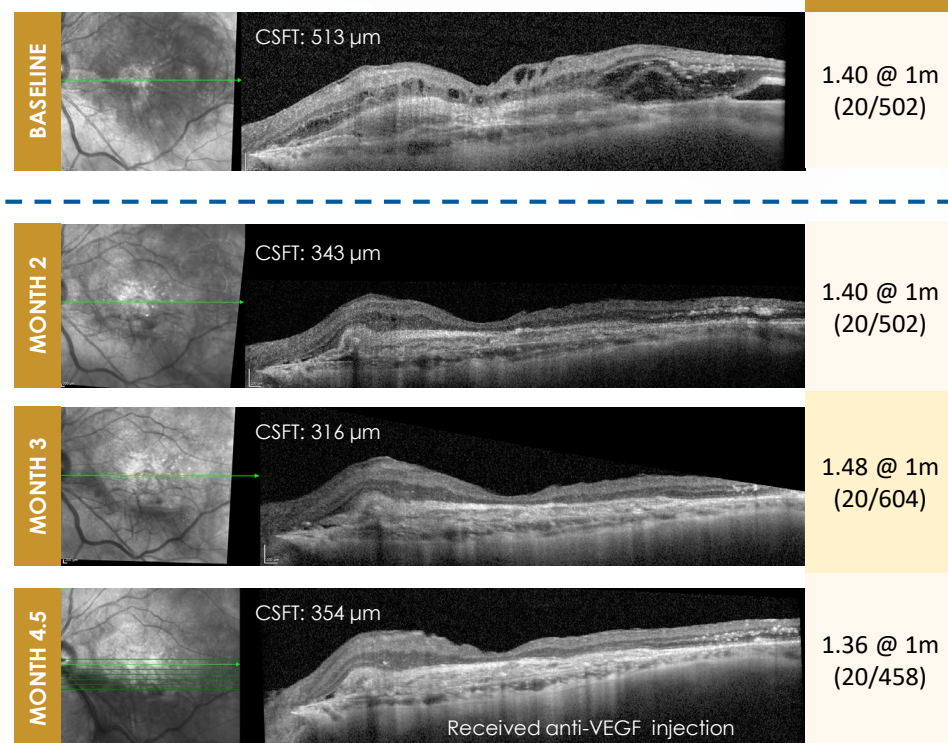
### ONGOING PHASE 1 CLINICAL TRIAL

- First (200µg) and second (400µg) cohorts fully enrolled
- Currently dosing third (600µg) cohort
- To date, observed to have a generally favorable safety profile

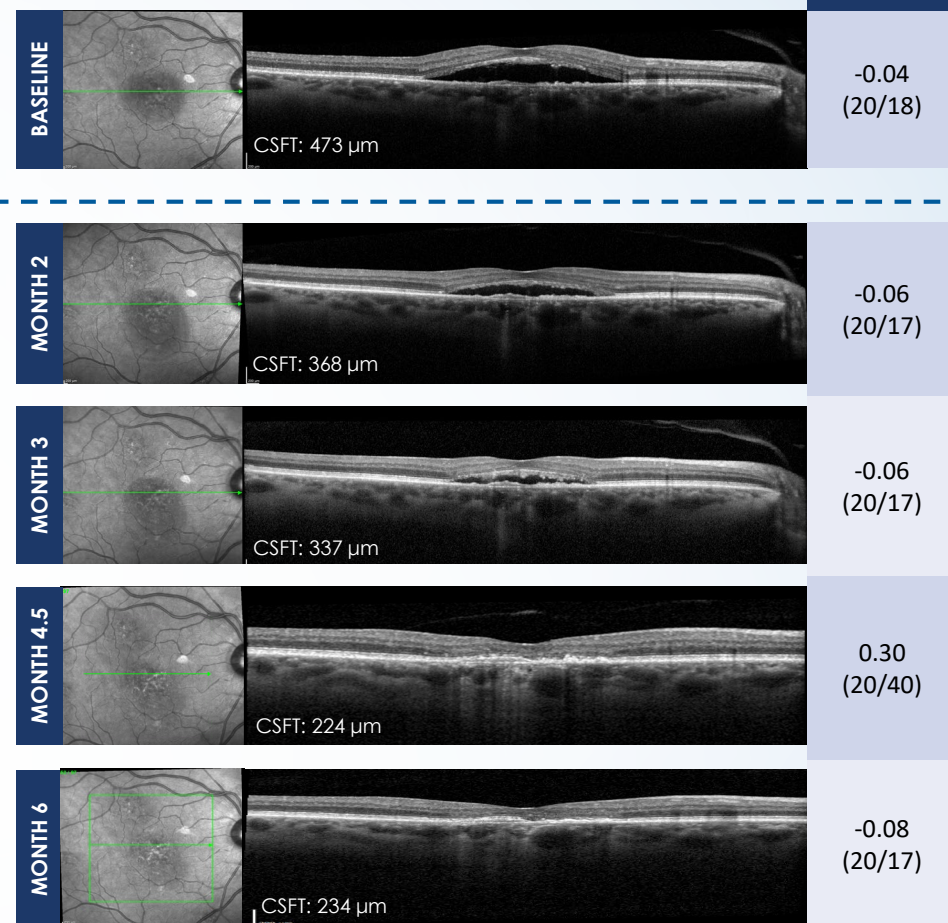
1. EYLEA Full Prescribing information 2019 2. Lucentis full Prescribing Information 2019 3. Bochot A, Fattal E. Liposomes for intravitreal drug delivery: a state of the art. *J Control Release.* 2012;161(2):628-634.

# PHASE 1 CLINICAL TRIAL, COHORT 2: SD-OCT EVALUATIONS

## Subject 1: Treatment Naïve (OS)



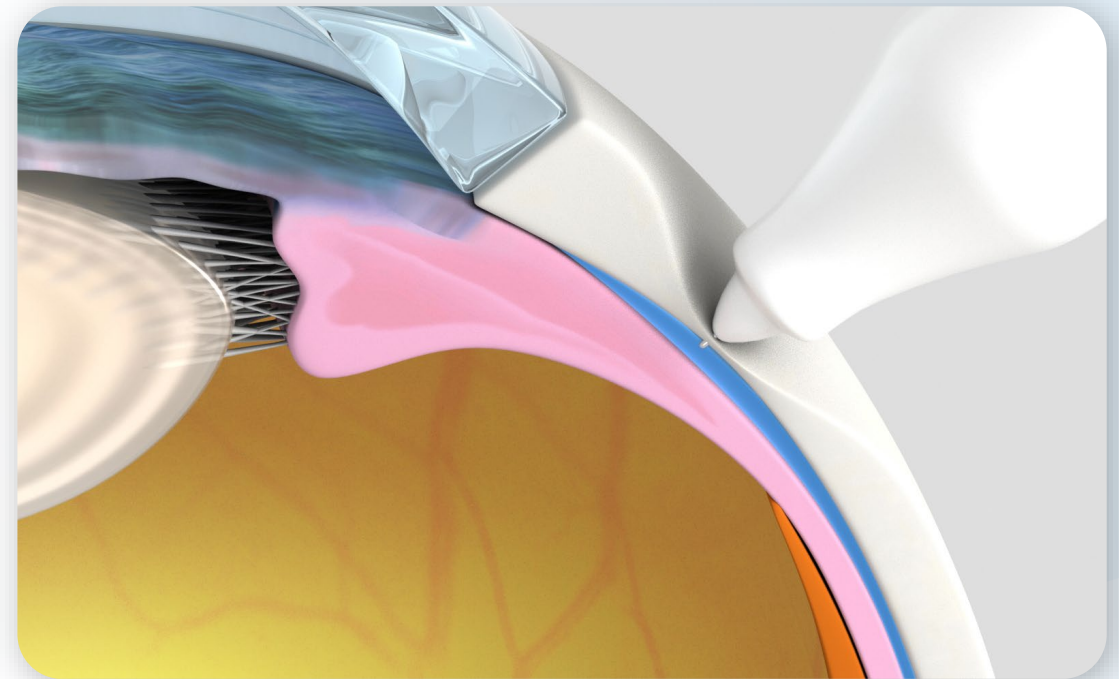
## Subject 2: History of EYLEA Q4 Weeks (OD)



**Preliminary biological signal of clinically-meaningful decrease in retinal fluid:**  
Some subjects showed a decrease in intraretinal and/or subretinal fluid by 2 months

**AMENDED AGREEMENT TO DEVELOP A NOVEL, SUSTAINED-RELEASE FORMULATION OF EYLEA<sup>®</sup> (AFLIBERCEPT)**

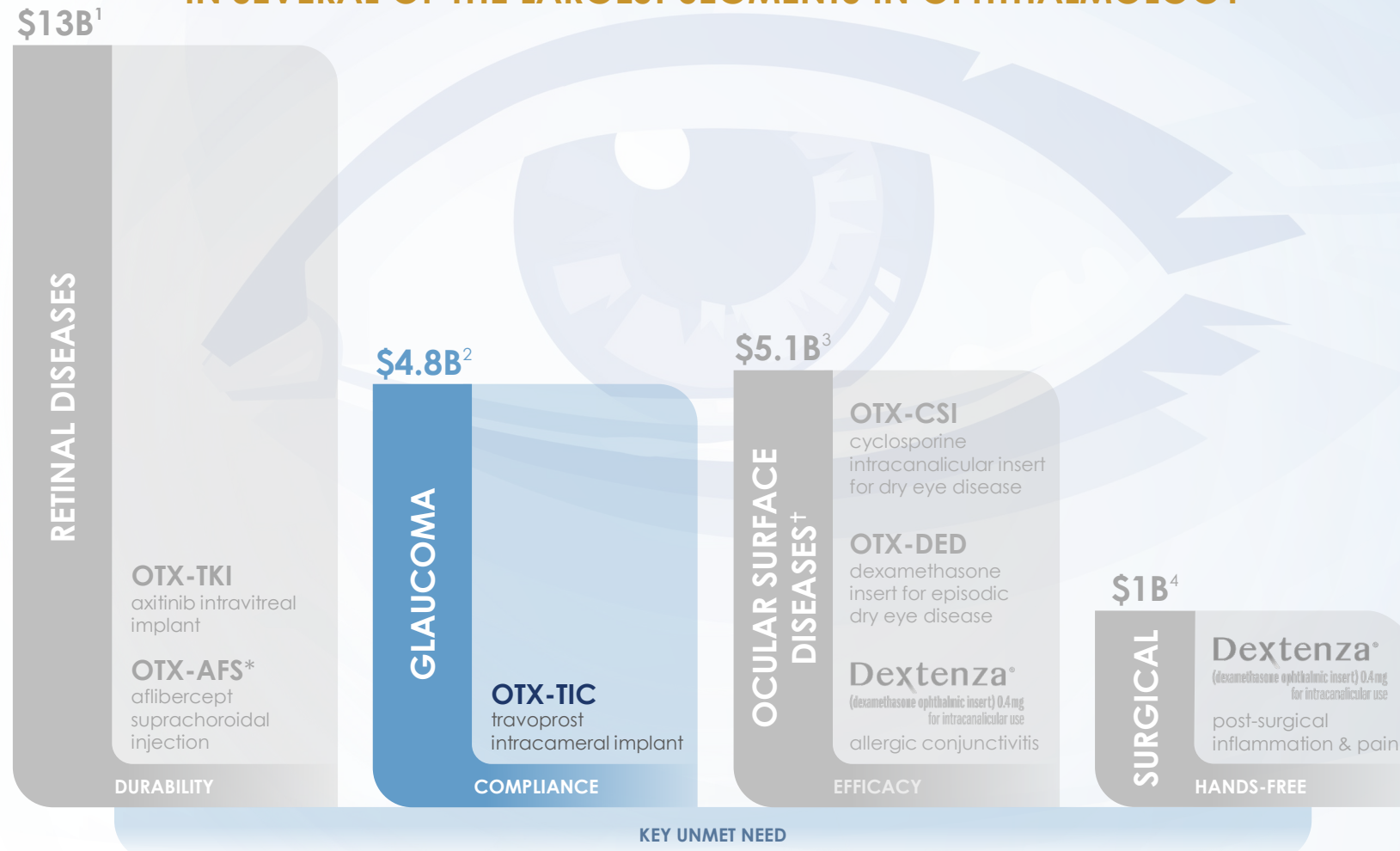
- **EYLEA is a vascular endothelial growth factor (VEGF) trap approved for the treatment of wet age-related macular degeneration (wet AMD) and other serious retinal diseases**
  - EYLEA is the global market leader with \$7.5 billion in revenue in 2019<sup>1</sup>
- **Evaluating opportunity to incorporate aflibercept with our 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 Therapeutix 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

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# OTX-TIC (TRAVOPROST INTRACAMERAL IMPLANT)

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

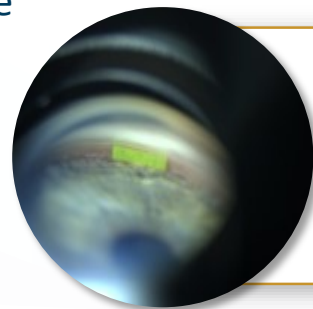
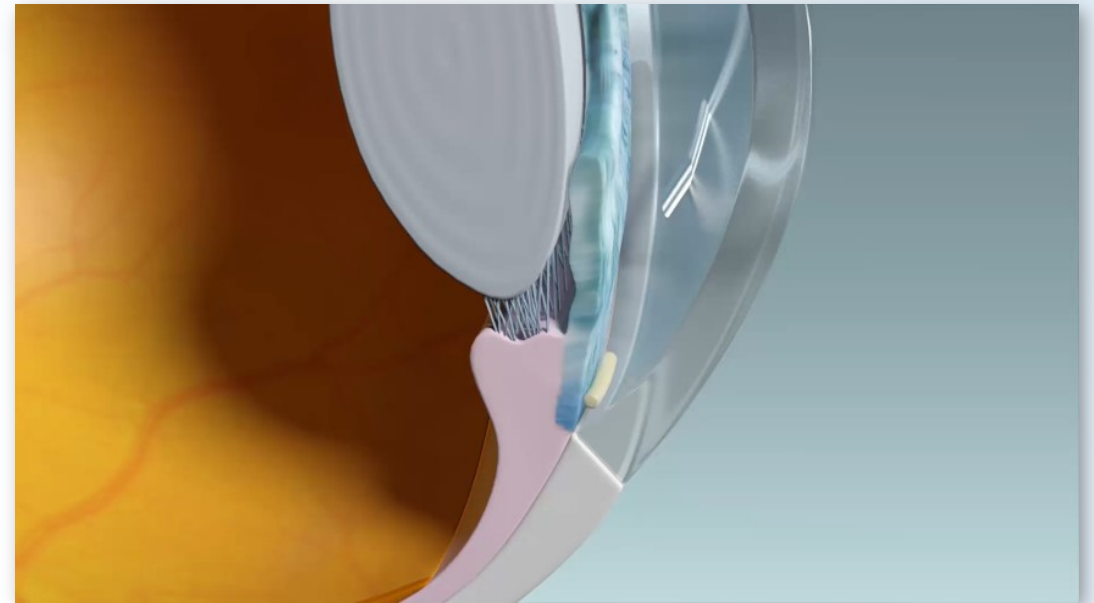
Plans to initiate Phase 2 clinical trial in first half 2021

### ISSUES WITH EXISTING TREATMENTS

- High rates of non-adherence to glaucoma therapies
- Poor adherence has 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 proprietary injector
- Resides in the iridocorneal angle
- Fully biodegradable
- Preservative-free



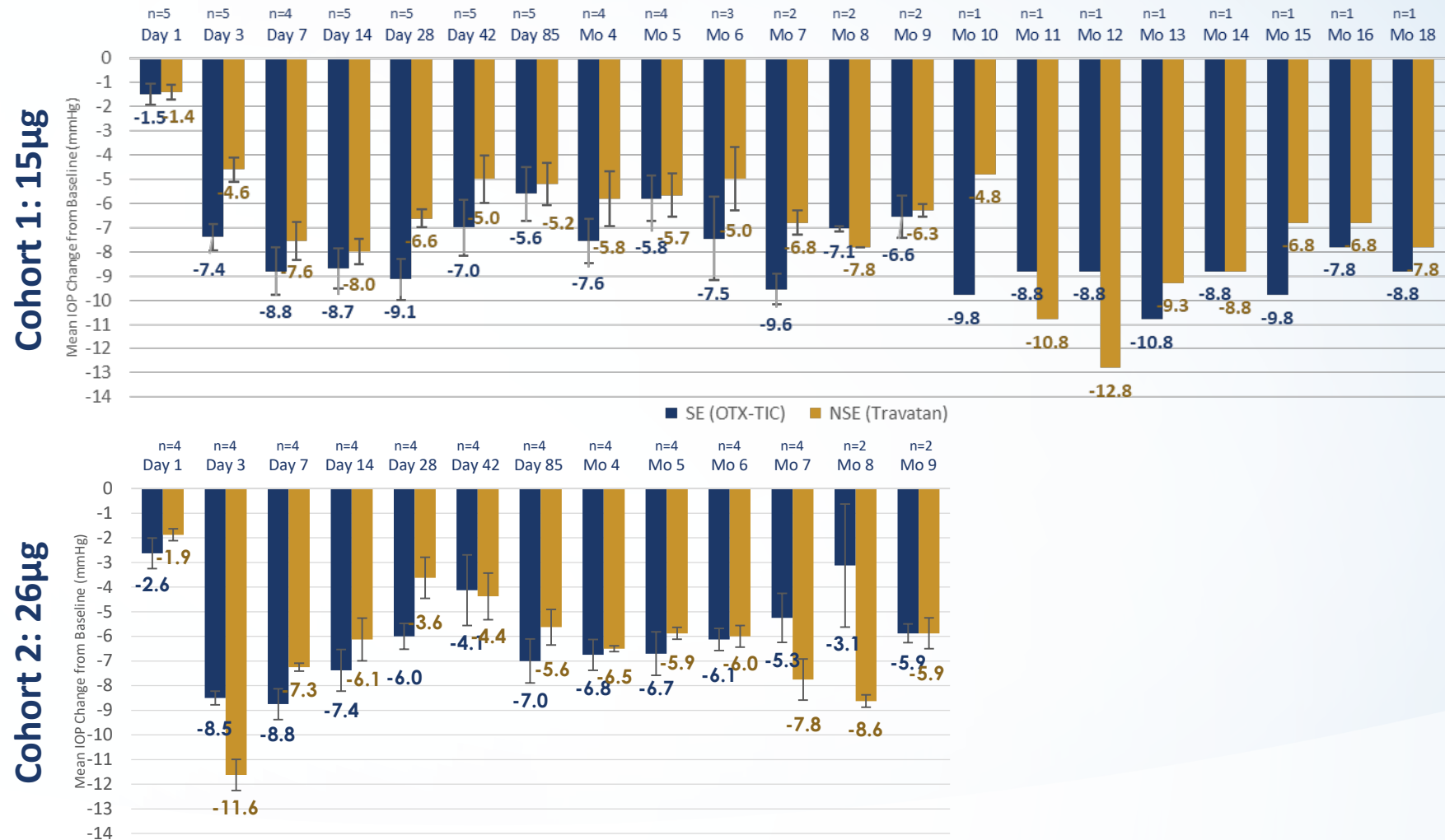
### ONGOING PH1 CLINICAL TRIAL

- Implant has biodegraded in most subjects by 6-7 months
- Implant has shown limited movement and was visible at all exams in all patients
- Endothelial cell counts and pachymetry assessments indicate no clinically meaningful changes from baseline

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.

# IOP DECREASE UP TO 7-10 MMHG RECORDED IN COHORTS 1 (UP TO 18 MONTHS) & 2 (UP TO 9 MONTHS)

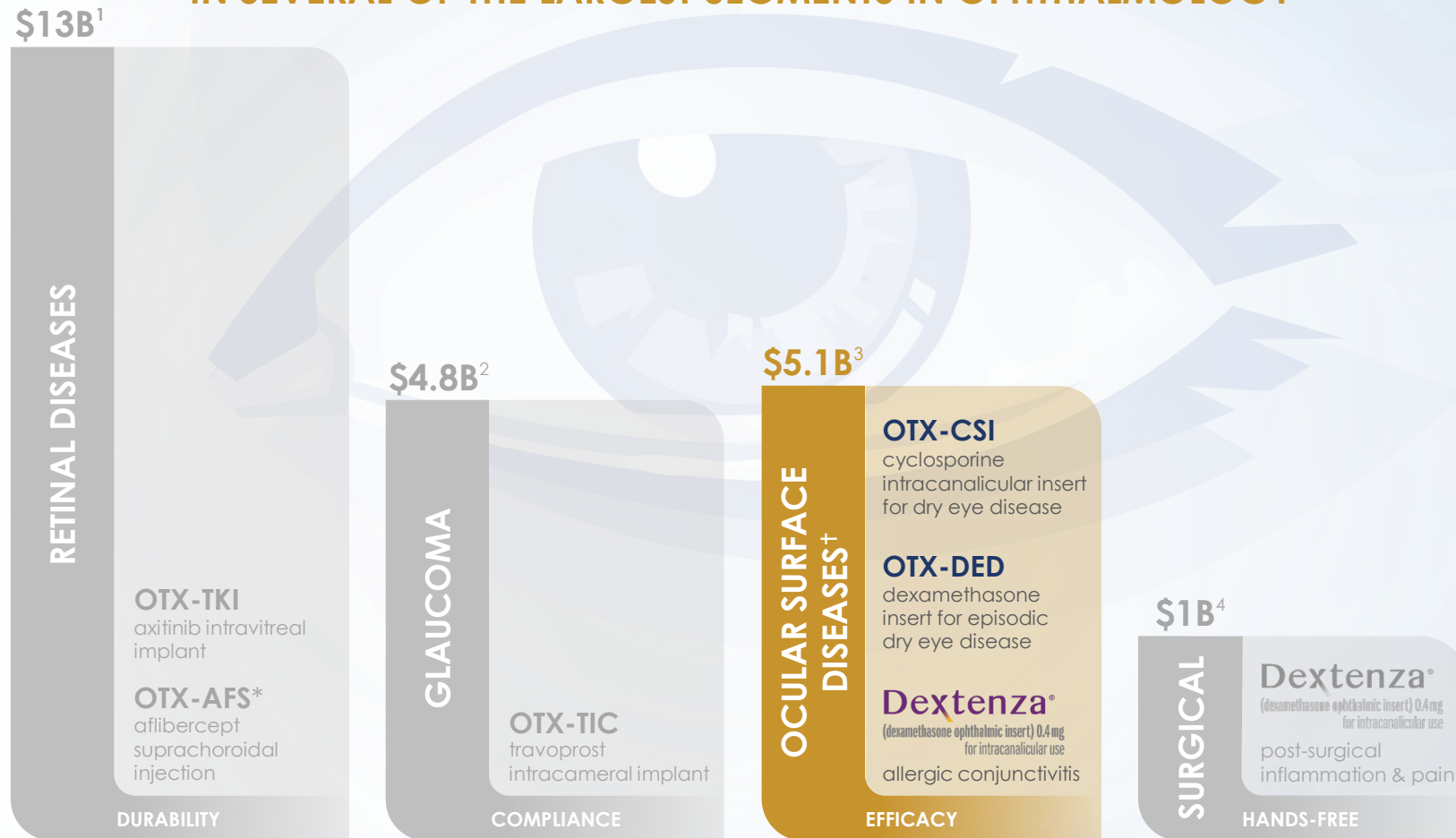
## DECREASED IOP AS EARLY AS TWO DAYS AFTER OTX-TIC IMPLANTATION



NB: Interim look; Unmonitored data \*If the study eye was given other IOP lowering medication, the IOP value was removed from the analysis. Data as of April 2020.

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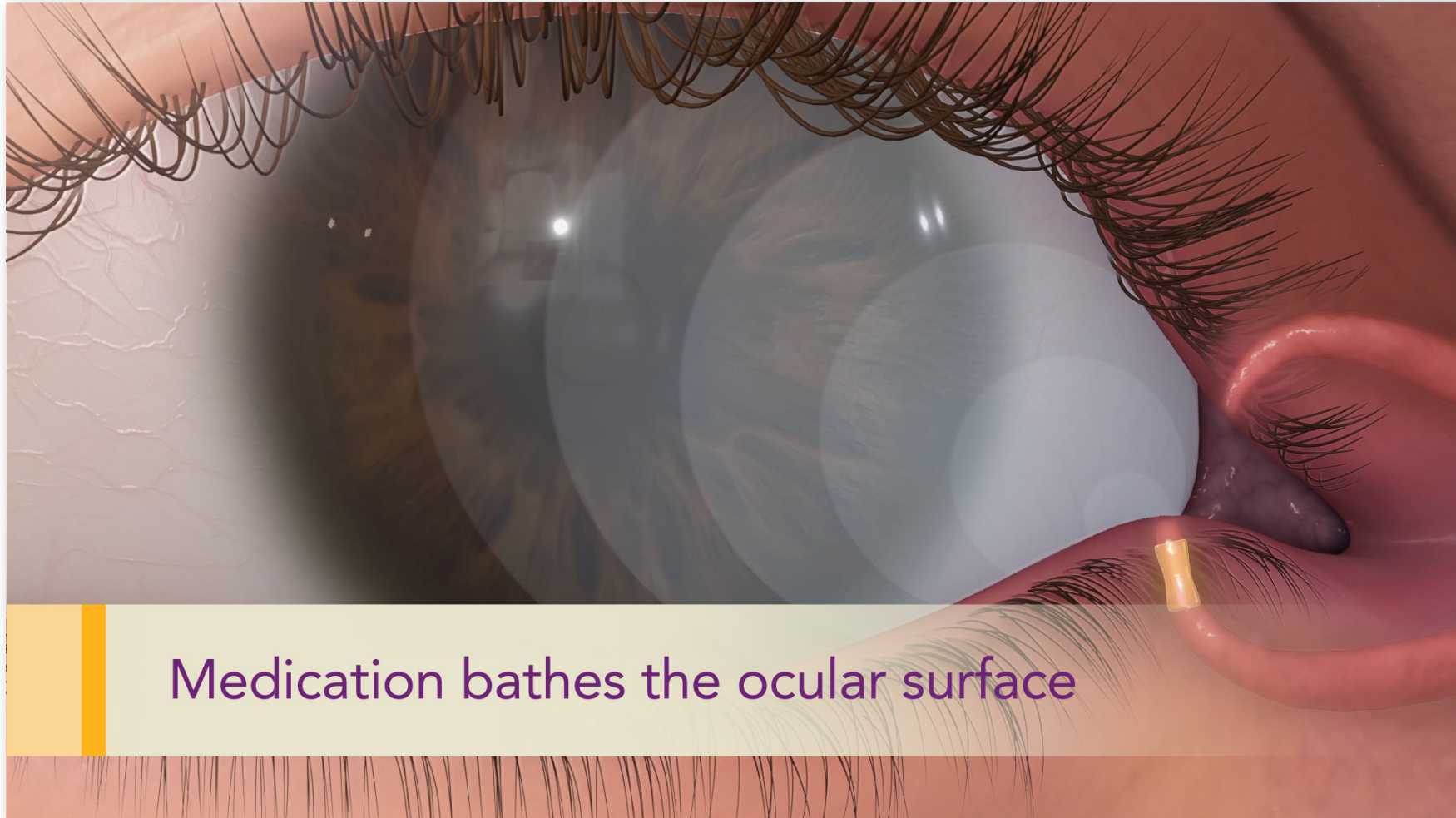
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# INTRACANALICULAR INSERTS

## AN INNOVATION IN DRUG DELIVERY TO THE OCULAR SURFACE



Medication bathes the ocular surface



# OTX-CSI (CYCLOSPORINE INTRACANALICULAR INSERT)

## SUSTAINED RELEASE THERAPY FOR DRY EYE DISEASE

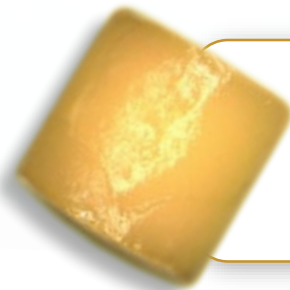
Plan to initiate Phase 2 clinical trial in 2020

### 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 effective therapy up to 12 weeks with a single insert
- Occludes the punctum
- Fully biodegradable insert



### ONGOING PHASE 1 CLINICAL TRIAL

First patient dosed in Phase 1 clinical trial in May 2020



# OTX-DED (DEXAMETHASONE INTRACANALICULAR INSERT)

## STERIODS ARE CURRENTLY USED TO TREAT EPISODIC DRY EYE

### ISSUES WITH EXISTING TREATMENTS

- Approved therapies for DED are known for slow onset of action and burning/stinging upon application
- Topical steroids (which are not FDA approved for DED) can be abused and contain preservatives causing ocular toxicity

Plan to submit Phase 2—enabling IND in 2020

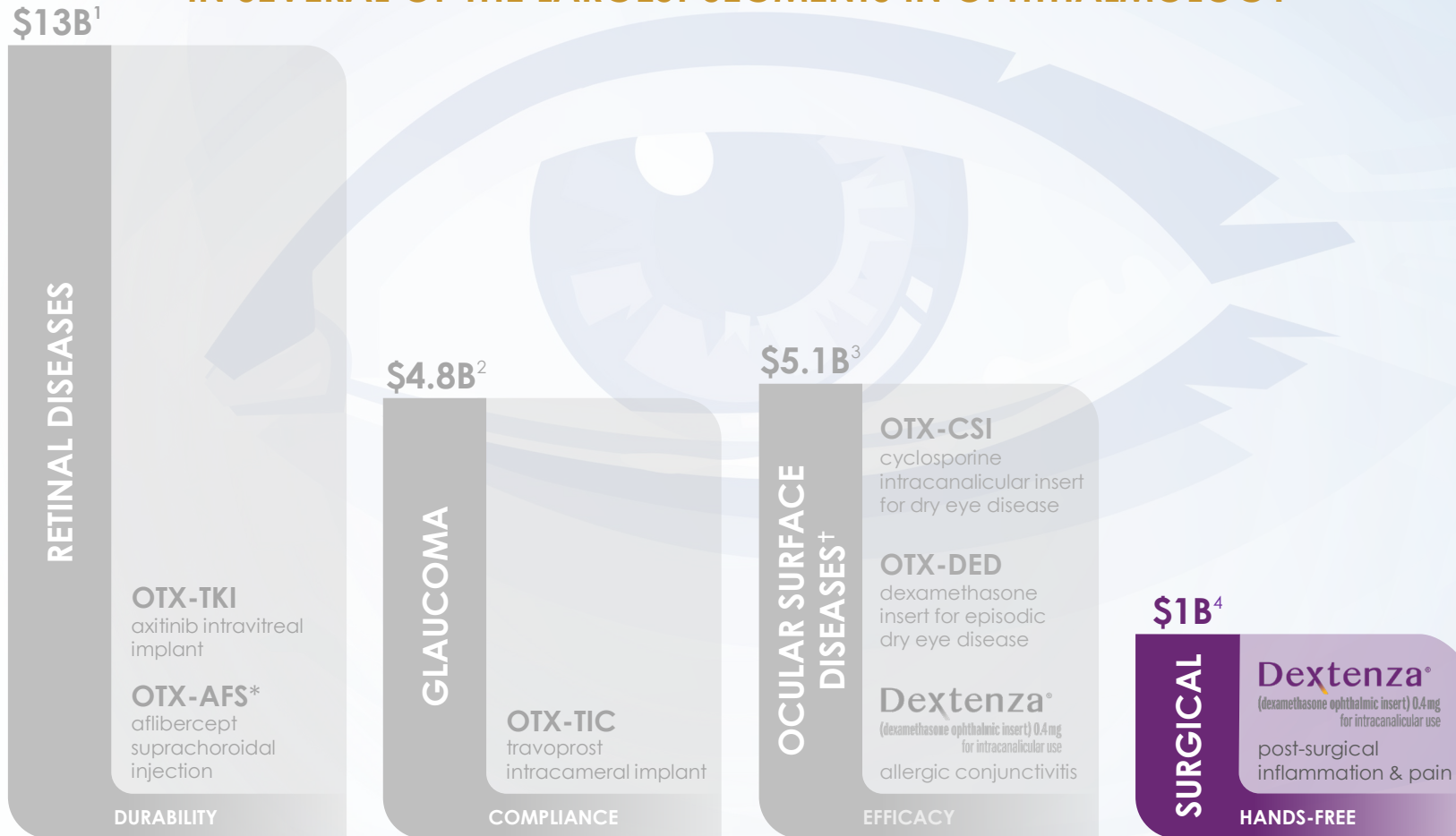
### KEY PRODUCT ATTRIBUTES

- Dexamethasone loaded in hydrogel
- Preservative-free
- Occludes the canaliculus providing more rapid onset of action
- Fully biodegradable insert
- Shorter duration and lower concentration of steroid release compared with DEXTENZA® (FDA approved dexamethasone intracanalicular insert for the treatment of inflammation and pain following ophthalmic surgery)
- Leverages strong safety profile of DEXTENZA®



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# DEXTENZA® (DEXAMETHASONE OPHTHALMIC INSERT)

## A HANDS-FREE ALTERNATIVE TO EYE DROPS

Provides a tapered delivery of preservative-free steroid for up to 30 days

**1**  
INNOVATIVE  
INSERT

VS

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

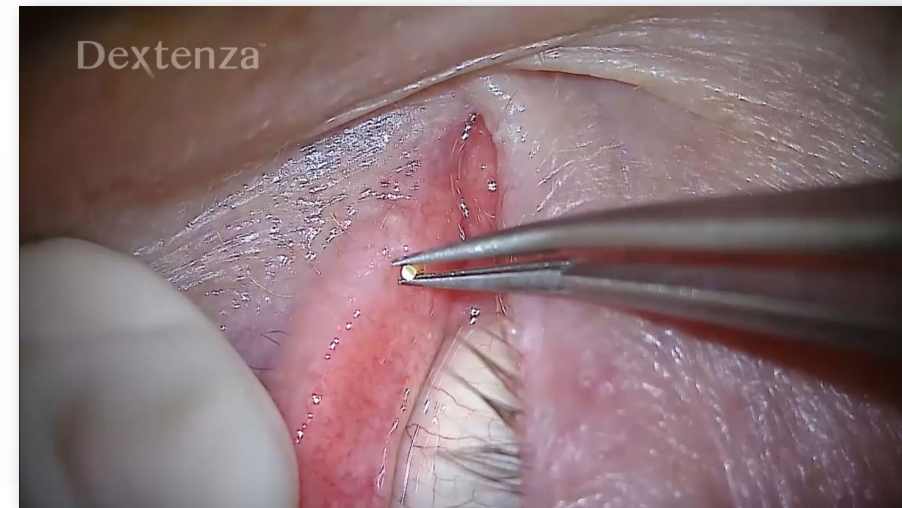
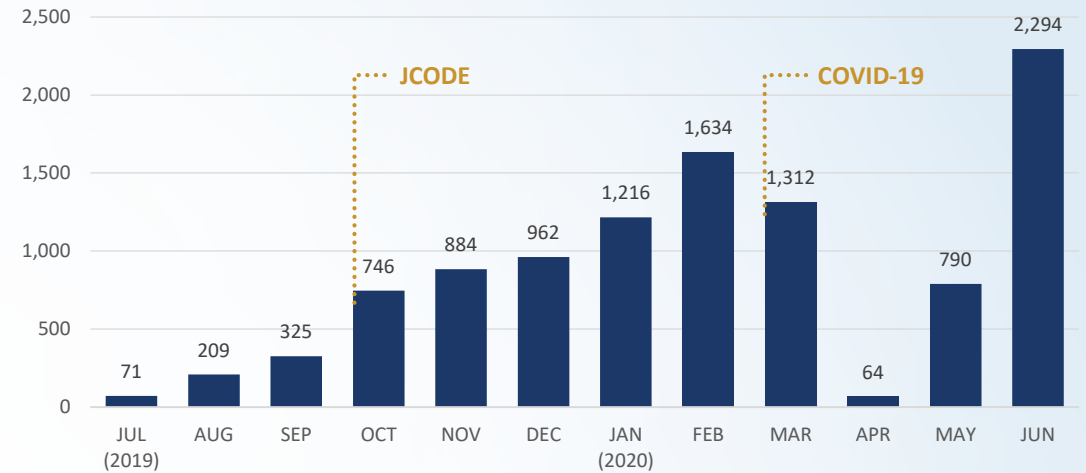
### REIMBURSEMENT CODING

- Product J-code 1096
- Procedure CPT code 0356T, Category 1 application filed
- Medicare administrative contractor (Novitas) issued coverage policy for 0356T of ~\$100 per insertion

### FDA APPROVED FOR THE TREATMENT OF INFLAMMATION AND PAIN FOLLOWING OPHTHALMIC SURGERY

Plan to file sNDA in Q4 to add the treatment of ocular itching associated with allergic conjunctivitis as an additional indication for DEXTENZA

BILLABLE INSERTS SINCE LAUNCH

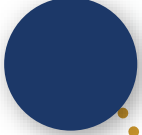
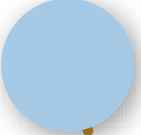
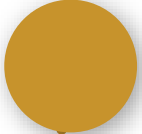
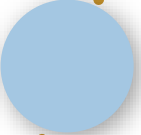







# ANTICIPATED NEAR-TERM MILESTONES

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-  **OTX-TKI (wet AMD)** – Plan to provide Phase 1 clinical update at AAO (Nov 2020) and file for US exploratory IND in 2020
-  **OTX-TIC (glaucoma)** – Plan to initiate Phase 2 clinical trial in first half 2021
-  **OTX-CSI (dry eye)** – Plan to initiate Phase 2 clinical trial in 2020
-  **OTX-DED (episodic dry eye)** – Plan to submit Phase 2 enabling IND in 2020
-  **DEXTENZA<sup>®</sup> (allergic conjunctivitis)** – Plan to file sNDA in 2020



# PIPELINE AT A GLANCE

PRODUCT/PROGRAM	DISEASE STATE	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	REGULATORY APPROVAL	
<b>WET AMD</b>							
<b>OTX-TKI</b> (axitinib intravitreal implant)	Wet AMD, DME and RVO <sup>†</sup>	▶					
<b>OTX-AFS</b> (afibercept suprachoroidal injection) In collaboration with <b>REGENERON</b>	Wet AMD, DME and RVO <sup>†</sup>	▶					
<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) 0.4 mg	Allergic conjunctivitis	▶					
<b>SURGICAL</b>							
<b>Dextenza®</b> (dexamethasone ophthalmic insert) 0.4 mg	Post-surgical ocular inflammation and pain	▶					◀

**NEW PRODUCT CANDIDATE**

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