OPHTHOTECH

36th Annual J.P. Morgan Healthcare Conference

Glenn Sblendorio, Chief Executive Officer and President

NASDAQ: OPHT

January 2018

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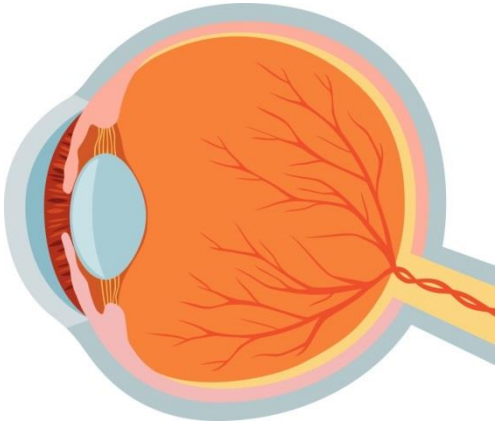
Ophthalmology: Age-related and Orphan Indications

Science Driven and Retina Focused

- **Deep Expertise in Ophthalmic Drug Development**
  - Multiple retina specialists
  - Strong global KOL network to facilitate clinical execution
  - Highly experienced clinical development team
- **Current Clinical Programs**
  - **Age-related**
    - Clinical trials in wet and dry AMD currently ongoing
    - Multi-billion dollar market opportunities
  - **Orphan**
    - Significant unmet medical need
    - Multiple programs ongoing or planned, led by a program in autosomal recessive Stargardt disease
- **Business Development Strategy**
  - Orphan ophthalmic and retinal diseases with therapeutic and gene therapy solutions
- **Strong Cash Position**
  - ~$167 million in cash and cash equivalents as of 12/31/17[1]

[1] Unaudited estimate

Value Creation: Multiple Track Strategy



Age-related Diseases
Multiple opportunities in large markets where medical need remains for patients

Orphan Diseases
Focus on underserved patients with the potential for an accelerated path to market

Business Development
Disciplined approach to evaluation of therapeutic and gene therapy solutions to ophthalmic diseases

Diversified Pipeline in Age-related and Orphan Diseases

Zimura® (Complement C5 inhibitor)

Dry AMD (GA)	• Phase 2b trial ongoing (monotherapy) • ~ 200 Patients / Primary endpoint at Month 12 • Top-line data expected in 2H/2019
Wet AMD	• Phase 2a trial ongoing (in combination with anti-VEGF) • ~ 60 Patients / 6 month study • Top-line data expected in late 2018
Autosomal Recessive Stargardt Disease (Orphan)	• Phase 2b trial ongoing (monotherapy) • ~ 120 Patients / Primary endpoint at Month 18 • Top-line data expected in 2020
Idiopathic Polypoidal Choroidal Vasculopathy (IPCV)	• Phase 2a trial ongoing (in combination with anti-VEGF) • ~ 20 Patients / 9 month study • Top-line data expected in 2H/2019
Intermediate/Posterior Uveitis (Orphan)	• Phase 2a trial planning to initiate in 2018 (monotherapy)

Multiple Catalysts Near-term and Beyond

Zimura (Complement C5 inhibitor)

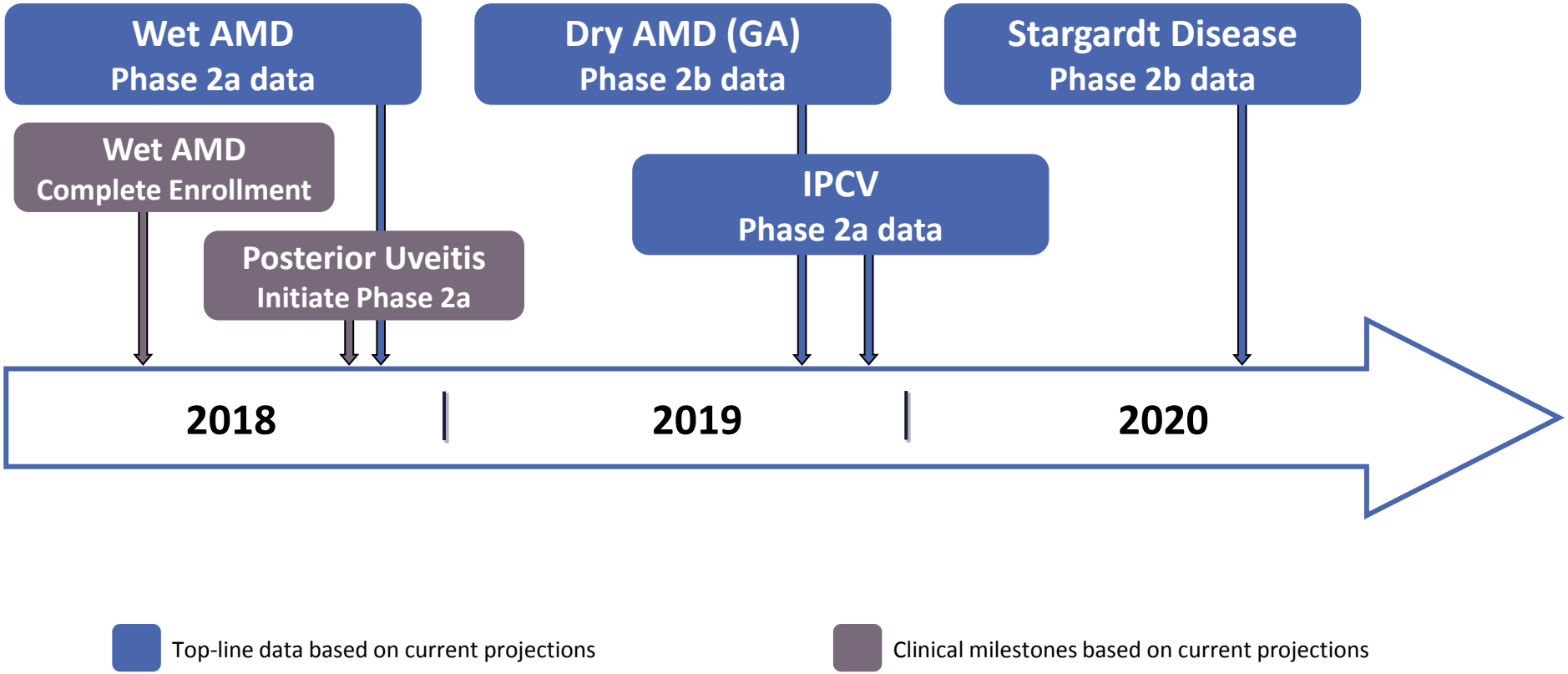


Diagram is for visual purposes only and is not intended to indicate specific timing of expected events

Zimura, C5 Complement Inhibitor

Geographic Atrophy Secondary to Dry AMD

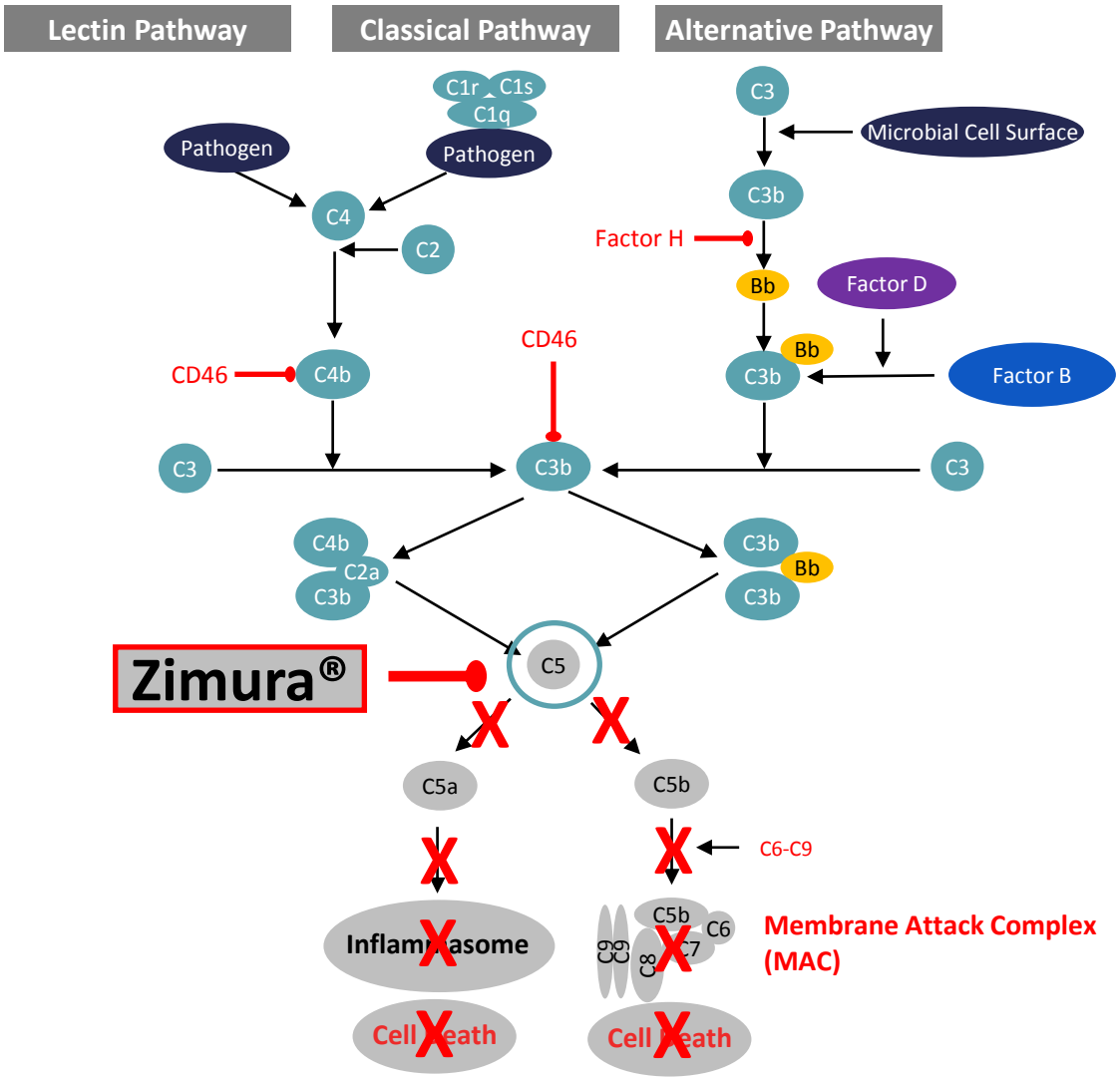
Development of Zimura for Geographic Atrophy Secondary to Dry AMD

- Major unmet medical need
  - Large market with no approved treatment options available
- Role of complement in dry AMD[1]
  - Complement deposition increases with aging
  - Complement activation leads to the formation and accumulation of inflammasomes and Membrane Attack Complex (MAC)
  - Inflammasomes and MAC lead to retinal pigment epithelial (RPE) cell death
  - RPE degeneration leads to photoreceptor cell death and loss of vision

[1] The Journal of Biological Chemistry Vol. 290, NO. 52, pp. 31189–31198, December 25, 2015. Invest Ophthalmol Vis Sci. 2013;54:110–120. J Immunol. 2015; 195:3382-3389. Med Sci Monit, 2010; 16(1): BR17-23. Am J Ophthalmol 2002;134:411–431. Proc Natl Acad Sci USA. 2005, 102(20), 7053-7054.

Zimura - Complement C5 Inhibitor

Inhibition of C5 prevents the formation of C5a and C5b-9, regardless of complement pathway

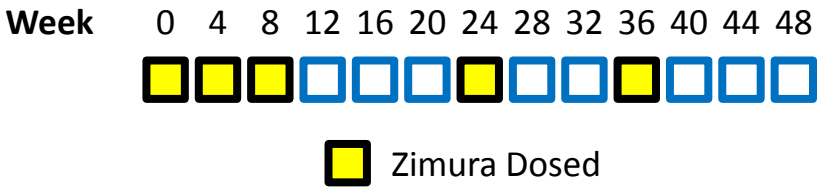


Source: OPHT internal

Zimura Phase 1/2a Dry AMD (GA) – Completed*

Study Design

Intravitreal Zimura was administered for a maximum of 5 injections at one of two dose levels (0.3 mg/eye or 1mg/eye)



47 Patients Enrolled

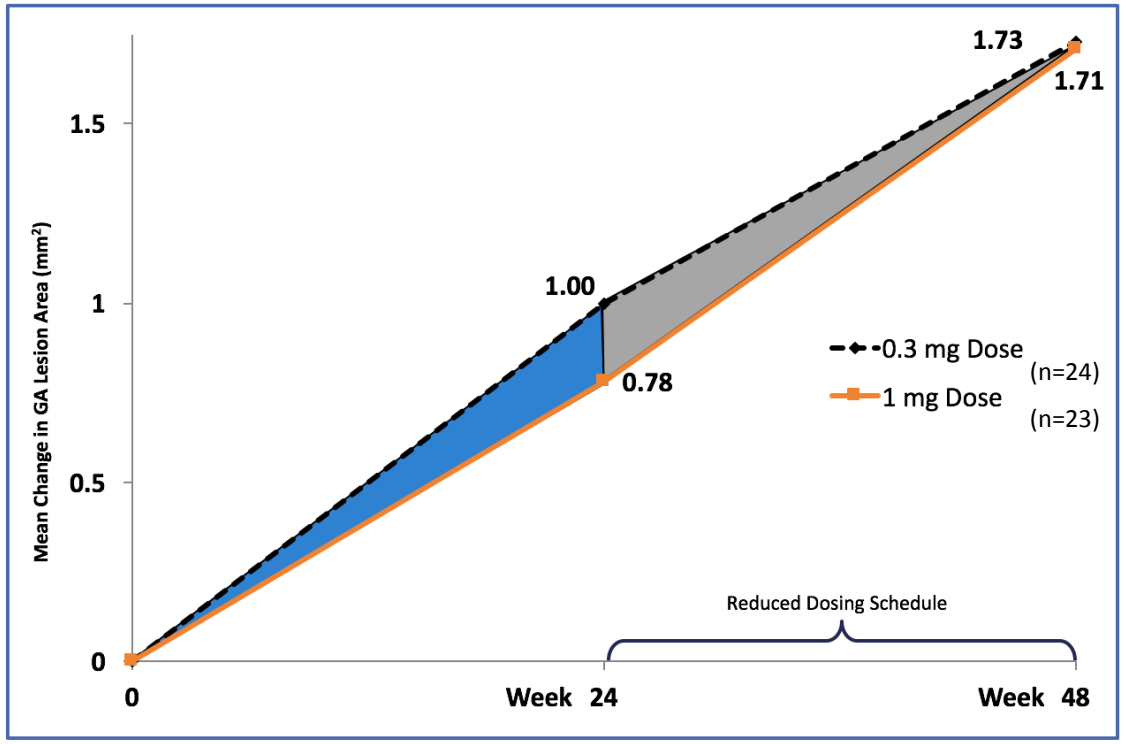
0.3 mg dose group (n=24)

1 mg dose group (n=23)

*Uncontrolled safety trial; small sample size

Zimura Phase 1/2a Dry AMD (GA) – Completed*

- **Potential efficacy signal(s)**
  - Presence of a dose-response trend with “on-off effect”
- **Safety**
  - No Zimura related adverse events
  - Zero incidence of wet AMD in eyes treated with Zimura



*Uncontrolled safety trial; small sample size

Zimura Phase 2b Dry AMD (GA) Clinical Trial – Ongoing

- Phase 2b, randomized, double masked, sham controlled clinical trial
- Study recently amended to accelerate anticipated timeline to obtain data
- ~ 200 subjects will be treated with monthly study treatment (Zimura or Sham) for 18 months
- Primary Efficacy Endpoint
  - Mean rate of change in GA over 12 months measured by fundus autofluorescence (FAF) at three time points

Top-line data expected in 2H 2019

Zimura, C5 Complement Inhibitor

Wet Age-Related Macular Degeneration

Current Standard of Care – Anti-VEGF Monotherapy

Unmet Need

"The processes responsible for the decrease in vision in CATT and other studies are multiple, but seem to be related to an increase in the proportion patients with an abnormally thin retina (< 120 µm), an increase in prevalence of geographic atrophy, . . . "

"These data highlight the need for agents that can prevent or minimize geographic atrophy . . . "

Source: Ophthalmology 2016;123:1751-1761

Development of Zimura for Wet AMD

- Unmet medical need remains– major market opportunity

- Anti-VEGF monotherapy:
  - Shown to reach a ceiling effect
  - Majority of patients do not reach a visual acuity of 20/40 or better
  - In the real world most patients lose vision over time

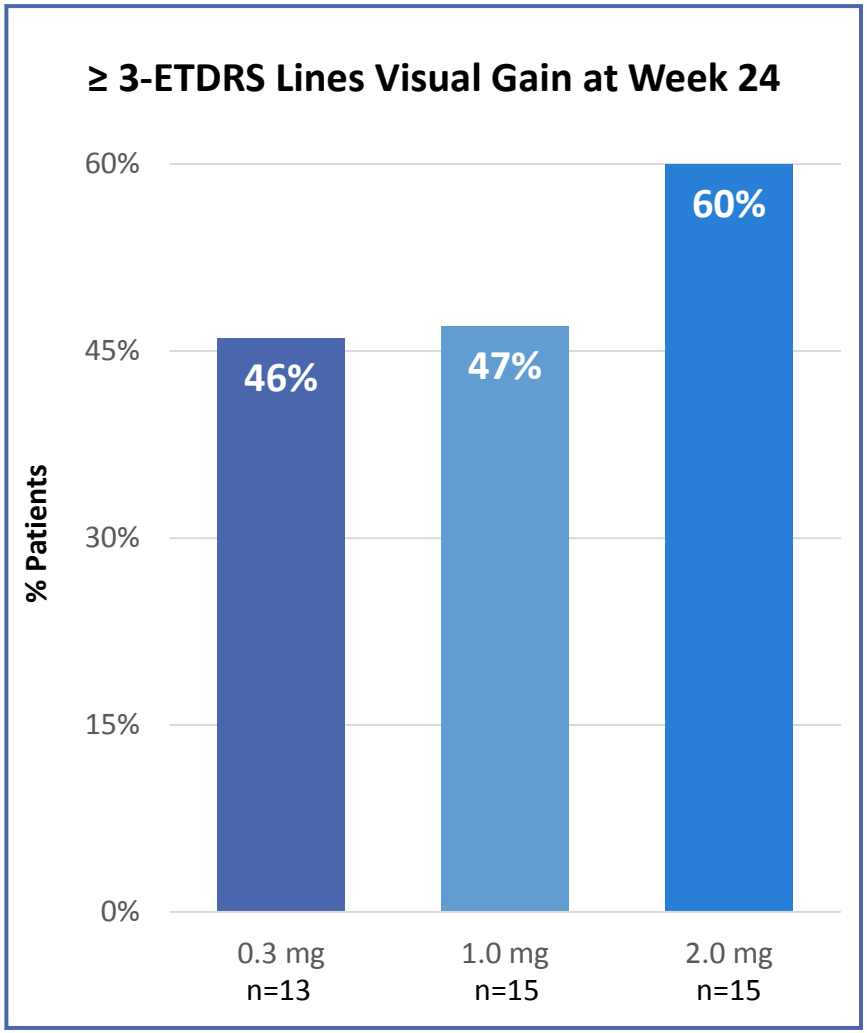
- Role of Complement in Wet AMD[1]
  - VEGF Increases Complement Factor H (CFH) (regulator of complement activation)
  - CFH decreases complement activation
  - Anti-VEGF Increases complement activation
  - Patients receiving anti-VEGF monotherapy may develop geographic atrophy[2]

- Adding Zimura to anti-VEGF therapy may improve the efficacy and safety of anti-VEGF

[1] J Clin Invest. 2017;127(1):199-214
[2] Ophthalmology 2014; 121:150-161.

Zimura Phase 1/2a Wet AMD – Completed*

- Included:
  - Treatment-naïve patients
  - All CNV subtypes
  - Patients receiving six monthly doses of Zimura in combination with Lucentis® 0.5mg
- Safety:
  - All doses well tolerated; no safety concerns were identified



*Uncontrolled safety trial; small sample size; subgroup analysis

Zimura Wet AMD Clinical Trial – Ongoing

- Phase 2a open label clinical trial
- N = ~ 60 subjects
- Objectives:
  - To assess the safety of intravitreal Zimura administered in combination with Lucentis® 0.5 mg in treatment naïve subjects with wet AMD
  - Dose ranging
  - Validate results from previously completed Phase 1/2a
- Duration: 6 months

Top-line data expected in late 2018

Zimura, C5 Complement Inhibitor

Autosomal Recessive Stargardt Disease (STGD1)

(Orphan Indication)

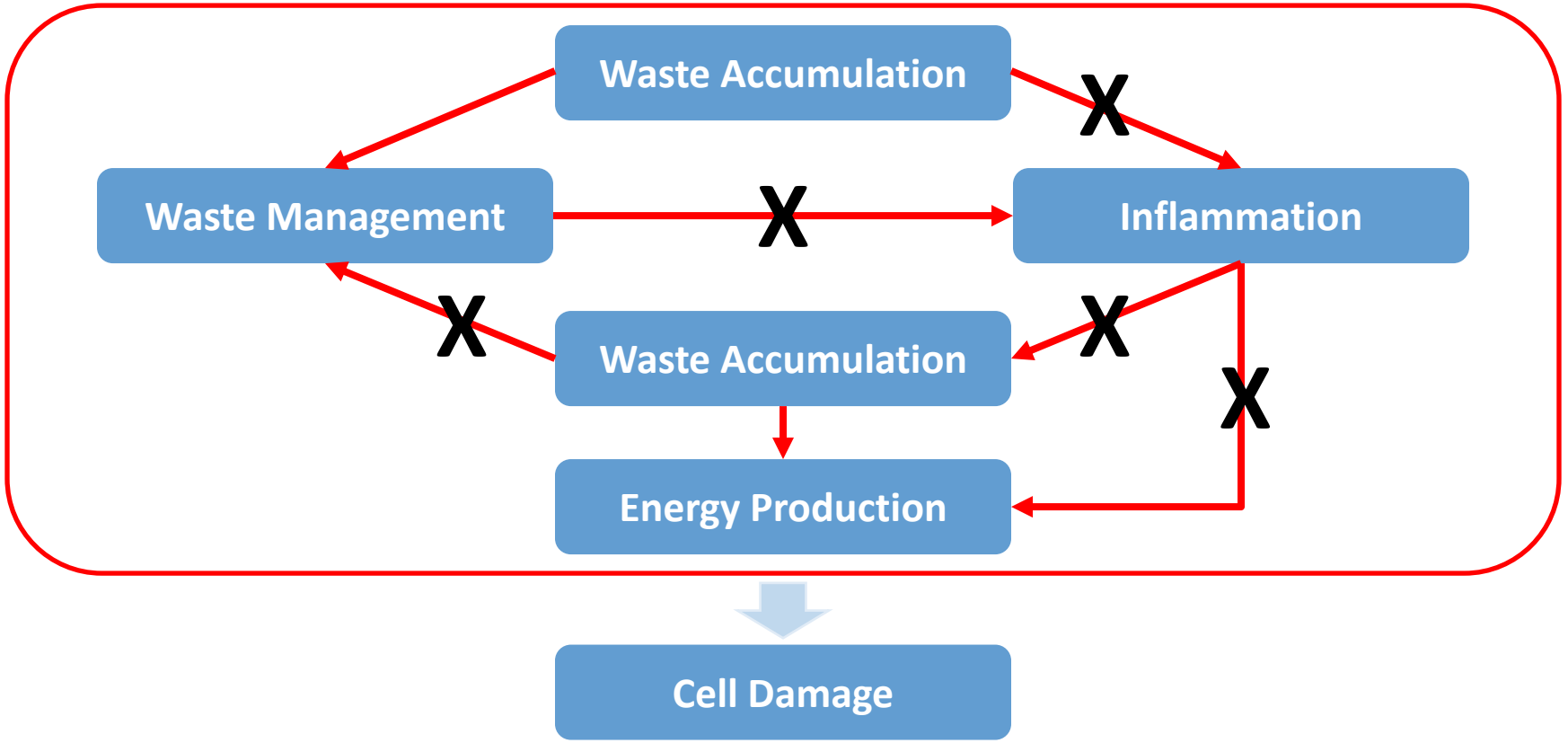
Development of Zimura in Autosomal Recessive Stargardt Disease

- High unmet medical need – Orphan disease
  - No FDA or EMA approved treatment available
- Role of Complement in Stargardt Disease[1]
  - Bisretinoids (visual cycle waste) activate complement
  - Complement inhibition rescues photoreceptor cells in a Stargardt animal model
  - Anti-C5 improved RPE cell viability in bisretinoid/complement cell culture model

[1] The Journal of Biological Chemistry. 2011; 286(21): 18593–18601. Proc Natl Acad Sci U S A. 2017; 114(15):3987-3992. Invest Ophthalmol Vis Sci. 2013;54:2669-2677

ABCA4 Gene Mutation (Autosomal Recessive Stargardt, STGD1): Waste Accumulation X Inflammation

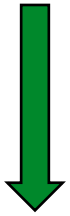
Complement inhibition may potentially lead to healthier RPE cells = Better ability to process and recycle the waste and therefore slow down the progression of Stargardt disease [(1)]



(1) Sources: FASEB J. 2004 Mar;18(3):562-4. Graefe's Arch Clin Exp Ophthalmol (2002) 240:983-988. The Journal of Biological Chemistry. 2011; 286(21): 18593–18601. Proc Natl Acad Sci U S A. 2017; 114(15):3987-3992. Invest Ophthalmol Vis Sci. 2013;54:2669-2677

Stargardt Albino Abca4$^{-/-}$ Mice: Complement Inhibition Rescues Photoreceptors

Expression of Complement Inhibitory Protein (CRRY)



Normalized Complement Activity

~2 fold decrease in bisretinoid accumulation

~30% increase in the number of photoreceptor nuclei

PNAS

Complement modulation in the retinal pigment epithelium rescues photoreceptor degeneration in a mouse model of Stargardt disease

Tamara L. Lenis[a,b,1], Shanta Sarfare[a,b,1,2], Zhichun Jiang[a,b], Marcia B. Lloyd[a,b], Dean Bok[a,b], and Roxana A. Radu[a,b,3]

Source: Proc Natl Acad Sci U S A. 2017; 114(15):3987-3992.

Zimura Stargardt Disease (STGD1) Clinical Trial - Initiated

- Phase 2b, randomized, double masked, sham controlled clinical trial
- N = ~ 120 subjects
- Duration of treatment: 18 months
- Primary Endpoint: Mean rate of change in the area of ellipsoid zone defect measured by en face SD-OCT

Top-line data expected in 2020

OPHT / Foundation Fighting Blindness

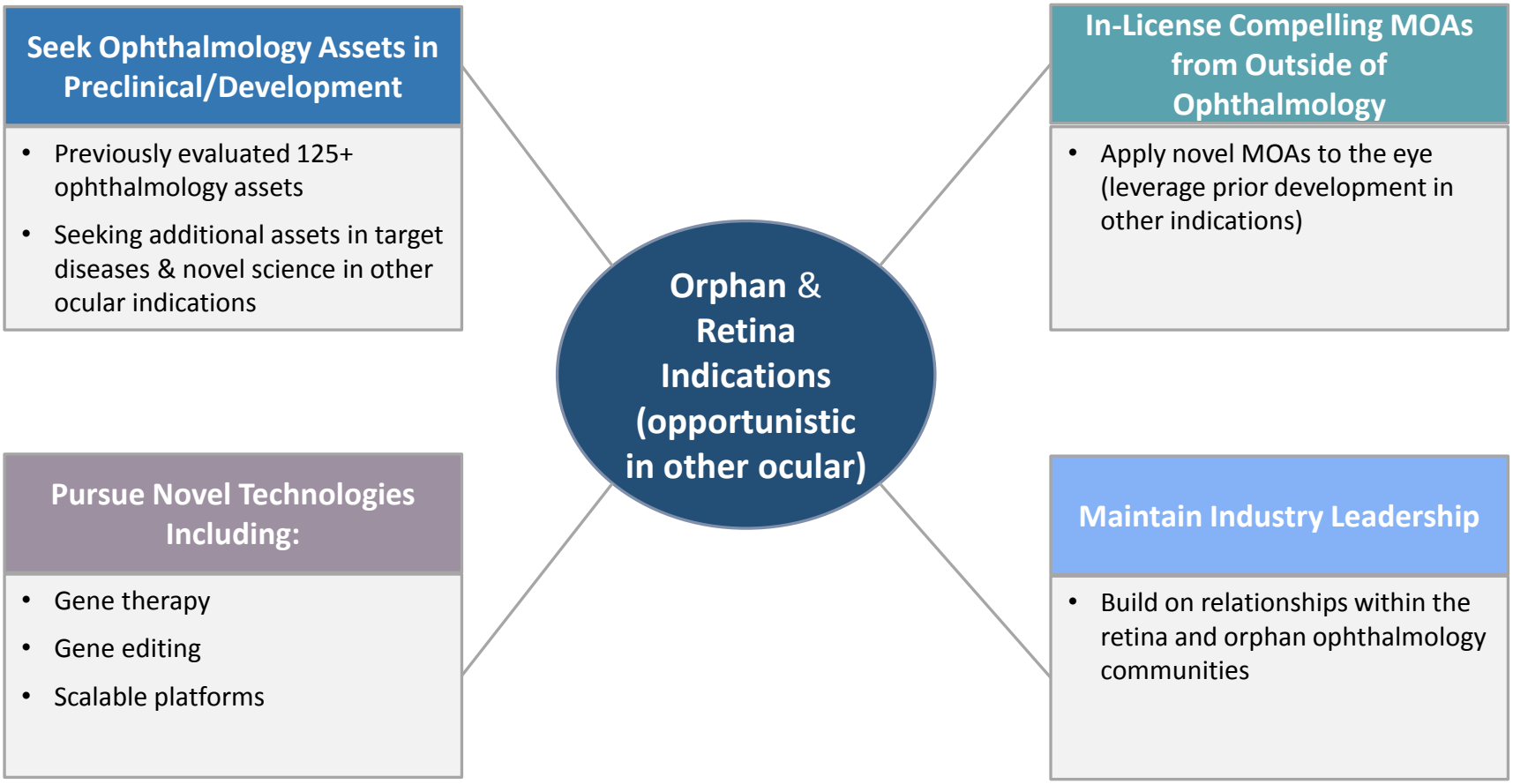
- OPHT agreement with Foundation Fighting Blindness (FFB)
  - Highly-distinguished organization recognized for its scientific commitment to orphan inherited retinal diseases
  - Established network of scientists and a robust patient registry

- Access to FFB's publicly available **ProgStar** study
  - Largest Natural History Study of Stargardt Disease
  - Data leveraged for Zimura Stargardt study design

FOUNDATION FIGHTING BLINDNESS
CLINICAL RESEARCH INSTITUTE

Pipeline Expansion Strategy

Become a leader in the development of novel therapeutics for age-related and orphan diseases of the eye



Financial Highlights

Strong Cash Position

- ~$167 million in cash and cash equivalents as of 12/31/17[1]
- External costs to bring Zimura programs to next phase of development expected to range between $25 million and $35 million[2]
- Cash corporate overhead expenses expected to average less than $2 million per month and continues to decline[2, 3]

[1]Unaudited estimate
[2]Guidance as of 11/8/17 and excludes any potential business development activities or any other changes to the Company's current clinical development programs
[3]Cash corporate overhead expenses consist of cash expenditures for employees and external G&A expenses

Executing on Strategic Plan: Age-related and Orphan Ophthalmic Indications

Zimura

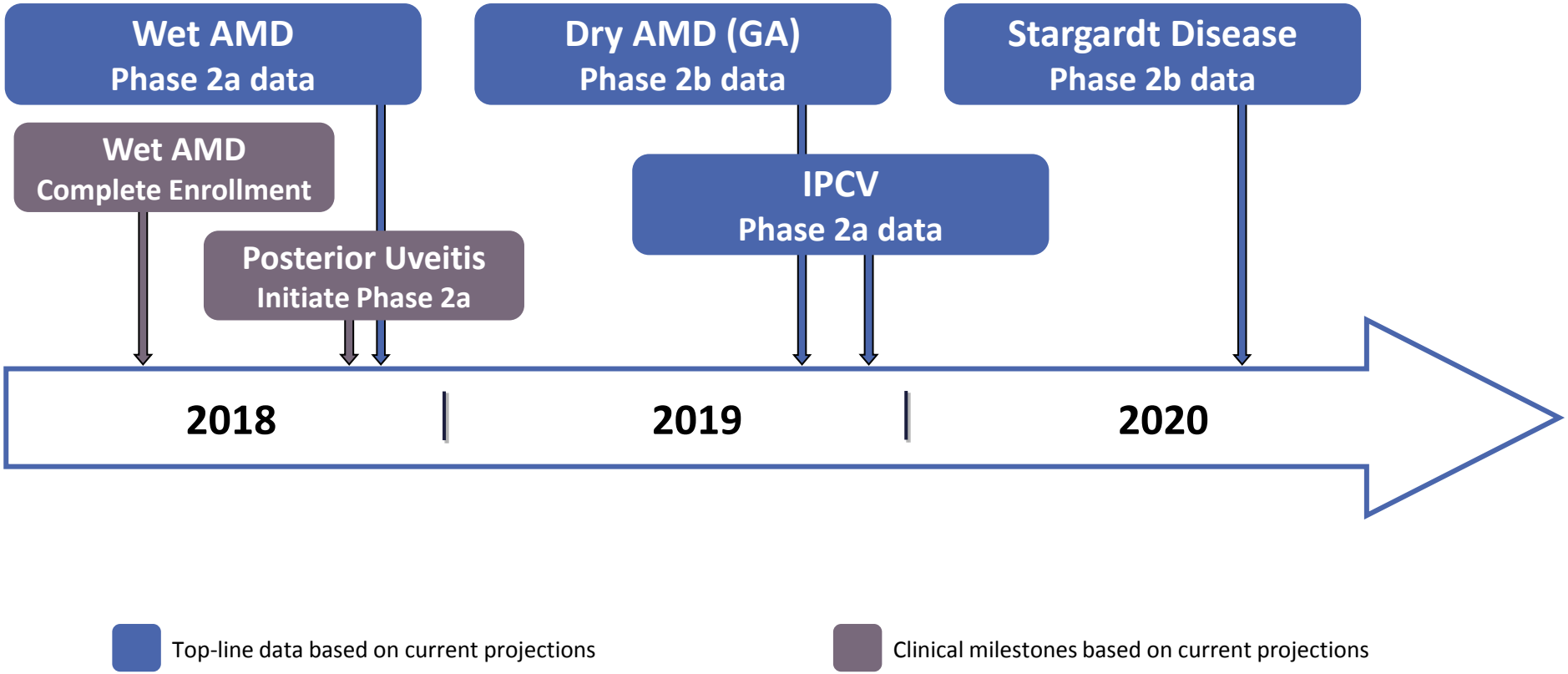
✓ **Wet AMD**	*Phase 2a ongoing*
✓ **Dry AMD**	*Phase 2b ongoing*
✓ **Stargardt Disease**	*Phase 2b ongoing*
✓ **IPCV**	*Phase 2a ongoing*
■ **Posterior Uveitis**	*Phase 2a to initiate in 2018*

Business Development

- Orphan ophthalmic and retinal diseases with therapeutic and/or gene therapy solutions

Multiple Catalysts Near-term and Beyond

Zimura (Complement C5 inhibitor)



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