OPHTHOTECH

Corporate Overview

NASDAQ: OPHT

November 2017

Forward-looking statements

Any statements in this presentation about Ophthotech's future expectations, plans and prospects constitute forward-looking statements for purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995. Forward-looking statements include any statements about Ophthotech's strategy, future operations and future expectations and plans and prospects for Ophthotech, and any 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. In this presentation, Ophthotech's forward looking statements include statements about the implementation of its strategic plan, Ophthotech's projected use of cash and cash balances, the timing, progress and results of clinical trials and other development activities, and the potential for its business development strategy, including any potential in-license or acquisition opportunities. Such forward-looking statements involve substantial risks and uncertainties that could cause Ophthotech'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 initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory matters and negotiation and consummation of in-license and/or acquisition transactions, need for additional financing and other factors discussed in the "Risk Factors" section contained in the quarterly and annual reports that Ophthotech files with the Securities and Exchange Commission. Any forward-looking statements represent Ophthotech's views only as of the date of this presentation. Ophthotech anticipates that subsequent events and developments will cause its views to change. While Ophthotech may elect to update these forward-looking statements at some point in the future, Ophthotech specifically disclaims any obligation to do so except as required by law.

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 and retina indications; opportunistic in other ocular diseases
- **Strong Cash Position**
  - Expect 2017 year-end cash balance to range between $155 million to $165 million*

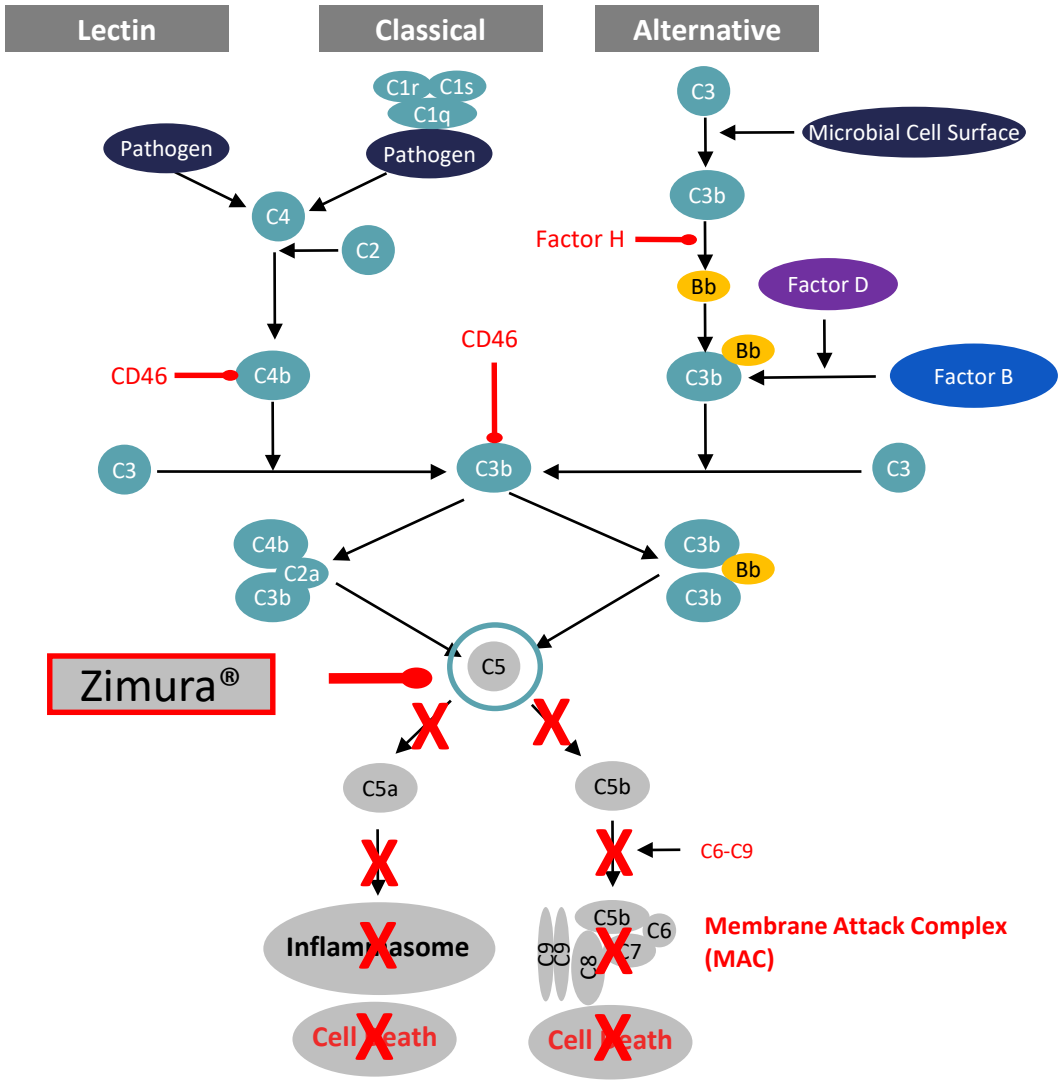
* Excluding any potential business development activities or any other changes to the Company's current clinical development programs

Diversified Pipeline in Ophthalmology

Zimura (Complement C5 inhibitor)

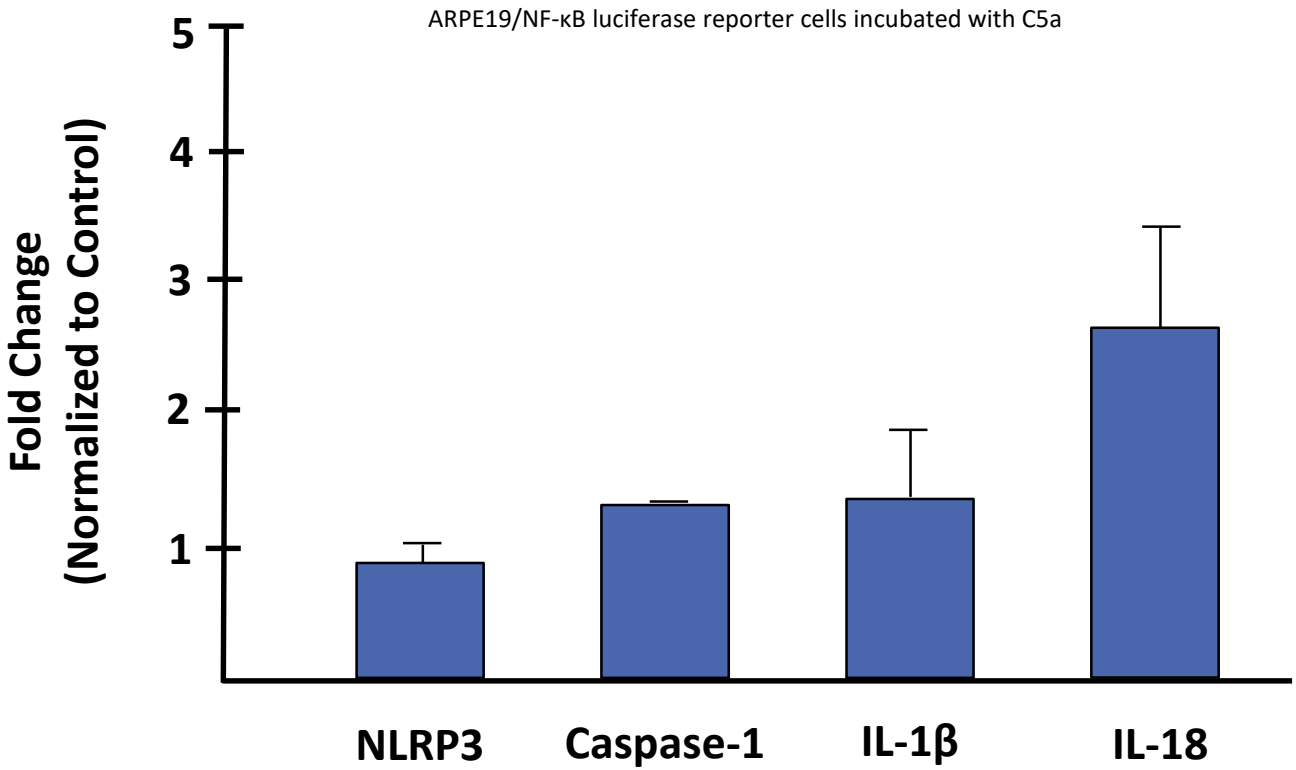
Dry AMD (GA)	• Phase 2b trial ongoing • ~ 200 Patients / Primary endpoint at Month 12 • Expected top-line data in 2H/2019
Wet AMD	• Phase 2a trial ongoing • ~ 60 Patients / 6 month study • Expected top-line data in late 2018
Autosomal Recessive Stargardt Disease (Orphan)	• Phase 2b to initiate by end of 2017 • ~ 120 Patients / Primary endpoint at Month 18 • Expected top-line data in 2020
Idiopathic Polypoidal Choroidal Vasculopathy (IPCV)	• Phase 2a to initiate by end of 2017 • ~ 20 Patients / 9 month study • Expected top-line data in 2H/2019
Intermediate/Posterior Uveitis (Orphan)	• Phase 2a planning to initiate in 2018

Complement Pathway: Inflammasome & MAC → Cell Death



Source: OPHT internal

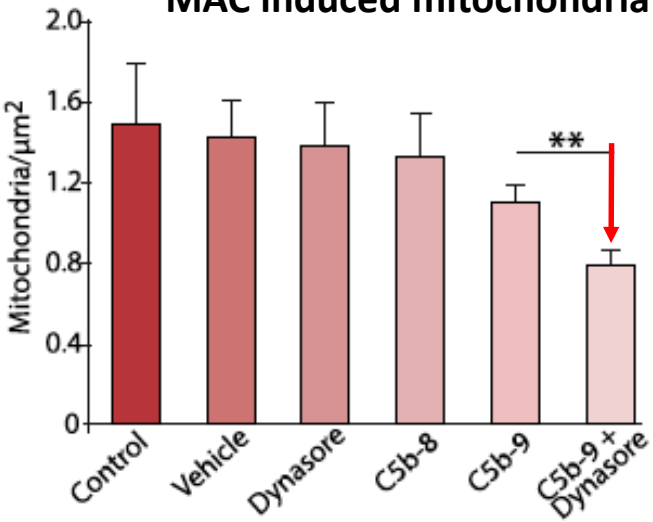
C5a Upregulates Inflammasome Related Genes → Cell Death

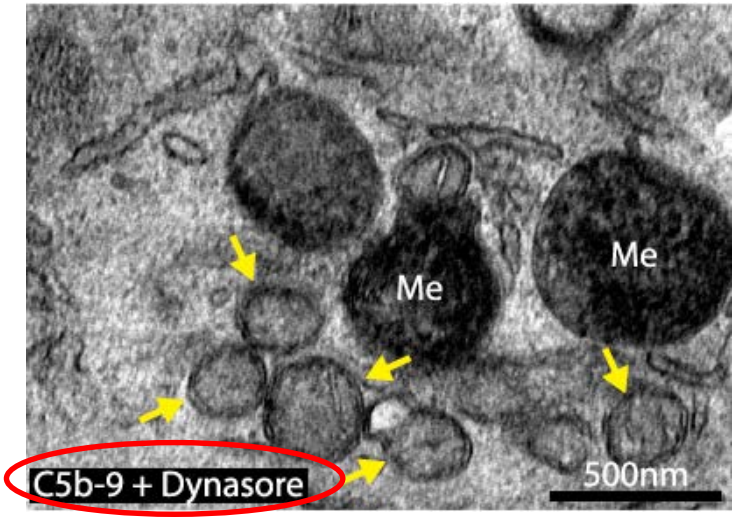


Source: Br J Ophthalmol. 2016 May; 100(5): 713–718.

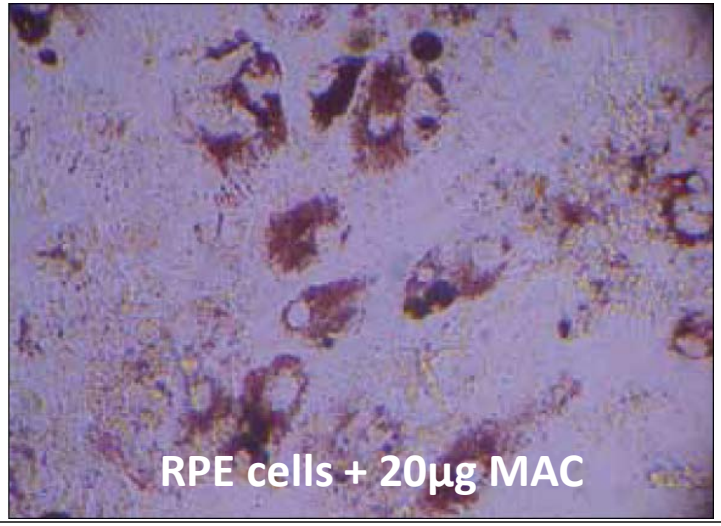
MAC Accumulation: Mitochondrial Damage & RPE Cell Death

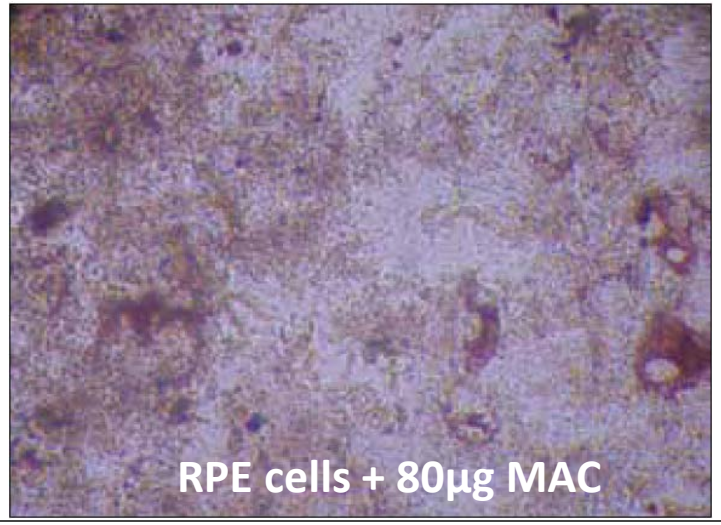
MAC induced mitochondria damage: Fewer and smaller/rounder than typical





Increased MAC Concentration leads to RPE cell lysis (cell death)





Sources: J Immunol. 2015; 195:3382-3389.
Med Sci Monit, 2010; 16(1): BR17-23.

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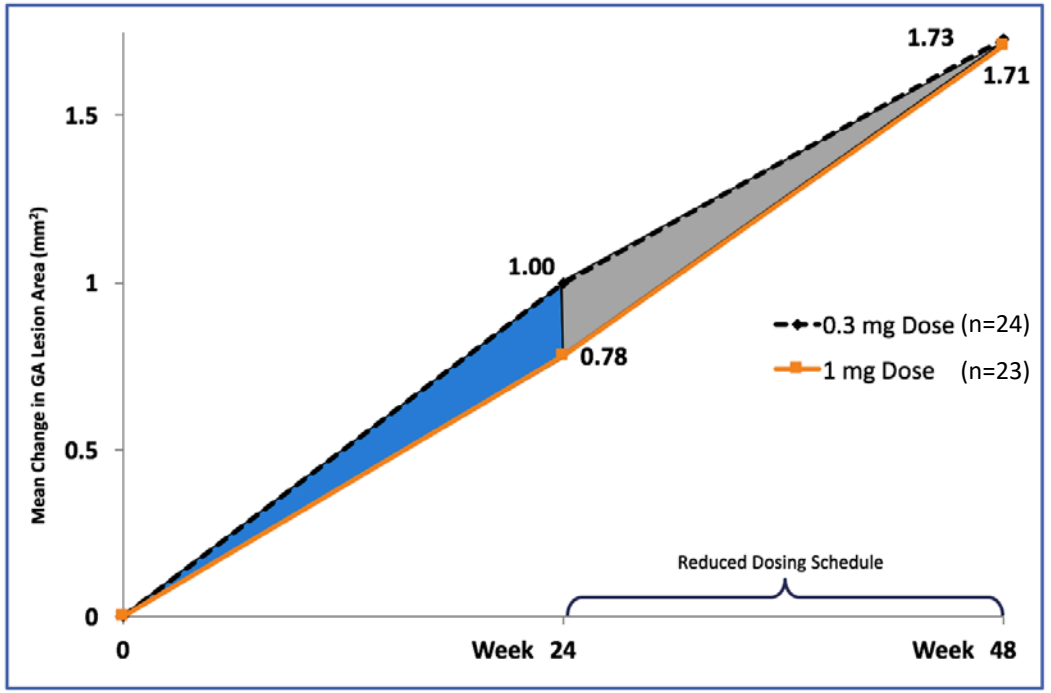
Development of Zimura in GA Secondary to Dry AMD

Geographic Atrophy, a disease characterized by retinal cell death, leading to central vision loss

- An unmet medical need - large market with no FDA/EMA approved treatment
- Phase 1/2a clinical trial completed
- Randomized, double masked, sham controlled Phase 2b clinical trial of monotherapy Zimura ongoing:
  - ~ 200 patients will be treated monthly
  - Primary efficacy endpoint: Mean rate of change in GA over 12 months

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

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



*Uncontrolled safety trial; small sample size

Development of Zimura in Wet AMD

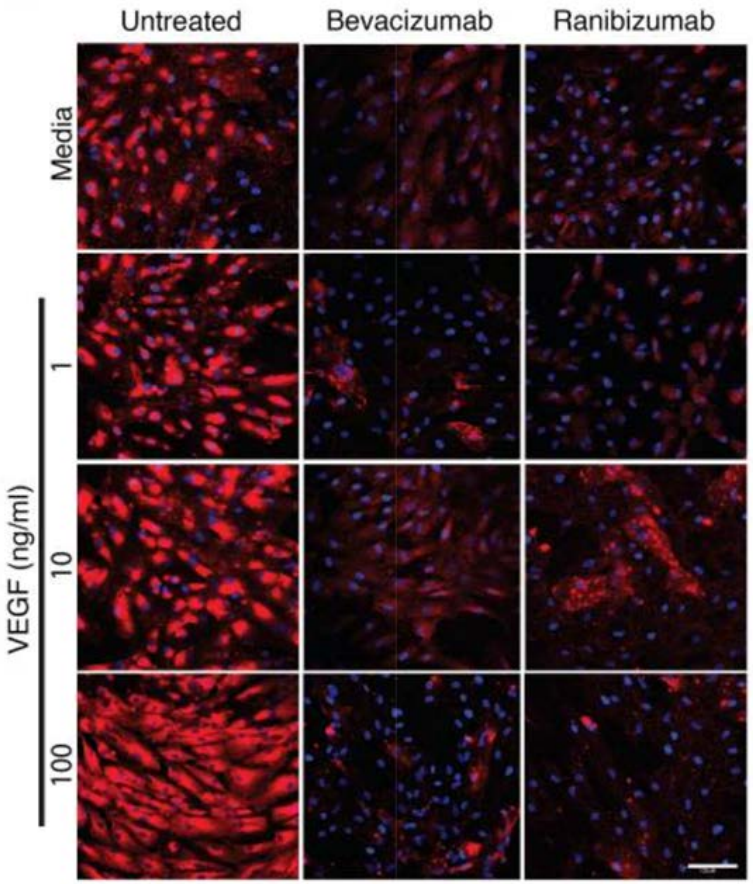
Characterized by abnormal blood vessel growth into the retina, leading to central vision loss

- Unmet medical need – major market opportunity
- Anti-VEGF monotherapy
  - Shown to reach a ceiling effect
  - In the real world most patients lose vision over time
  - Patients may develop geographic atrophy: 20% at two years and ~ 38% at 5 years[1]
- VEGF upregulates complement factor H, which is a complement inhibitory factor (Scripps Laboratories in San Diego)[2]
- Phase 1/2a clinical trial completed
- Phase 2a trial ongoing:
  - Dose ranging in combination with anti-VEGF
  - ~60 treatment-naïve patients to be treated for 6 months

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

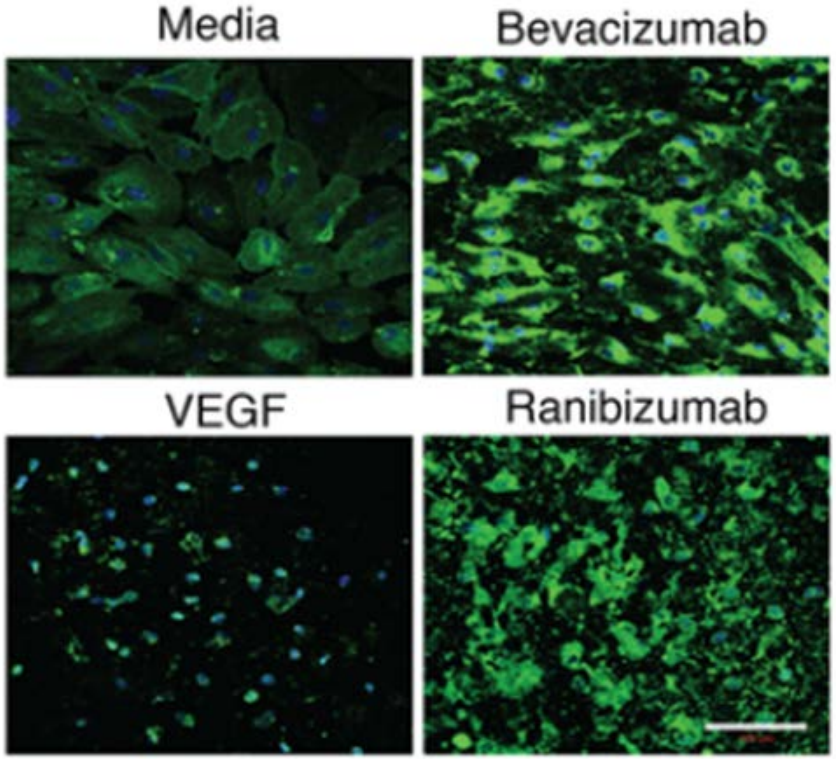
Anti-VEGF Decreases CFH Protein

RPE cells incubated with VEGF showed a dose-dependent increase in CFH protein



Red: CFH protein

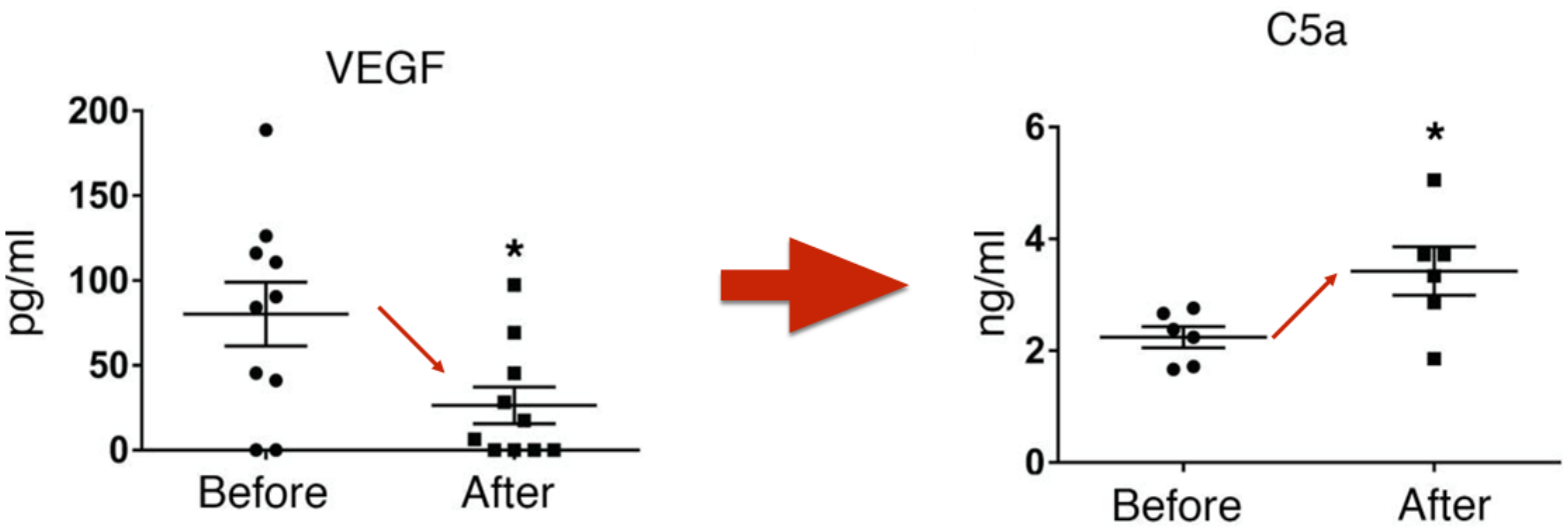
Anti-VEGF increases complement deposition in RPE cells



Green: Complement deposition

J Clin Invest. 2017;127(1):199-214

Intravitreal Anti-VEGF Treatment Results in Complement Activation

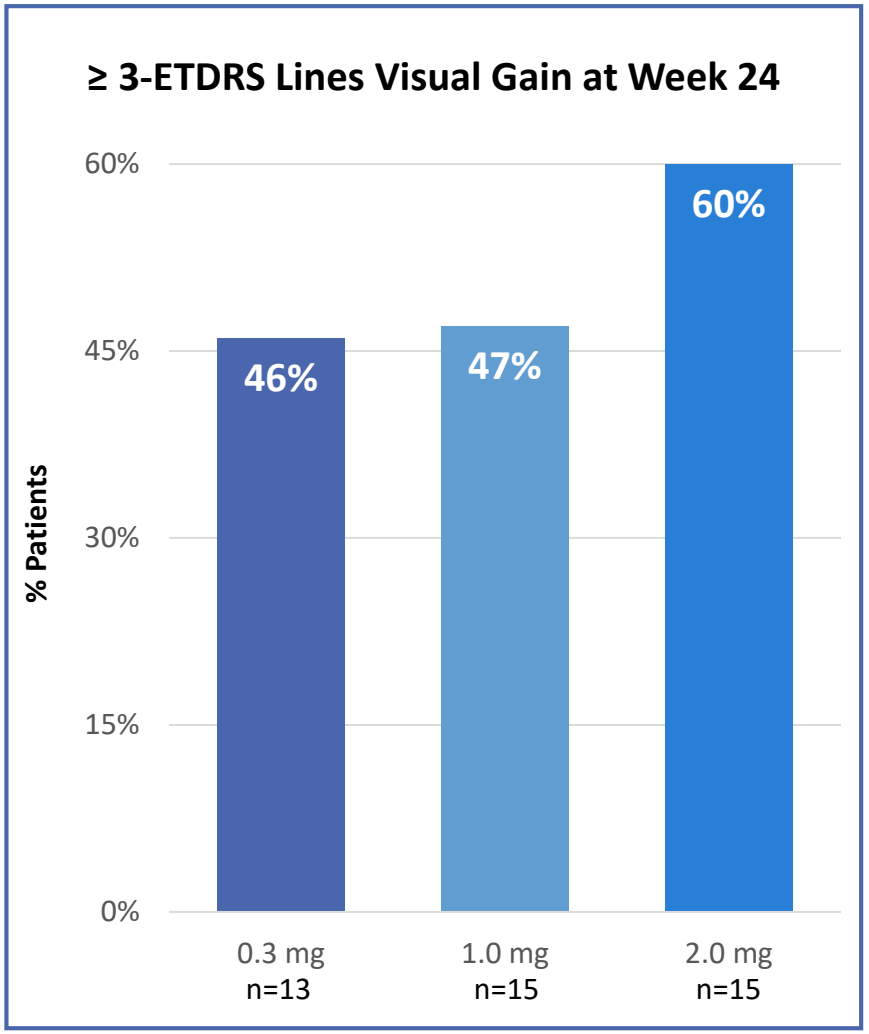


Aqueous humor from 10 wet AMD patient eyes was sampled before and 48 hours after a single intravitreal bevacizumab injection

J Clin Invest. 2017;127(1):199-214

Zimura Phase 1/2a Wet AMD – Completed*

- Inclusion:
  - Treatment Naïve subjects
  - All CNV subtypes
- Design:
  - 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

Development of Zimura in Autosomal Recessive Stargardt Disease

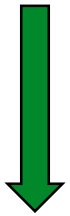
A devastating inherited orphan retinal disease causing vision loss during childhood/adolescence

- High unmet medical need with no FDA/EMA approved treatment
- Scientific evidence[1]
  - Bisretinoids (visual cycle waste) activate complement and prevent its clearance
  - Complement inhibition rescues photoreceptor cells in a Stargardt animal model
  - Anti-C5 improved RPE cell viability in bisretinoid/complement cell culture model
- Orphan disease
  - Potential for priority review voucher
  - Seven year exclusivity in US and 10 years in EU
- Phase 2b, randomized, double masked, sham controlled clinical trial to initiate by end of 2017
  - ~ 120 patients / Primary endpoint at Month 18
  - Primary endpoint: Mean rate of change in the area of ellipsoid zone defect

Sources: 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

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

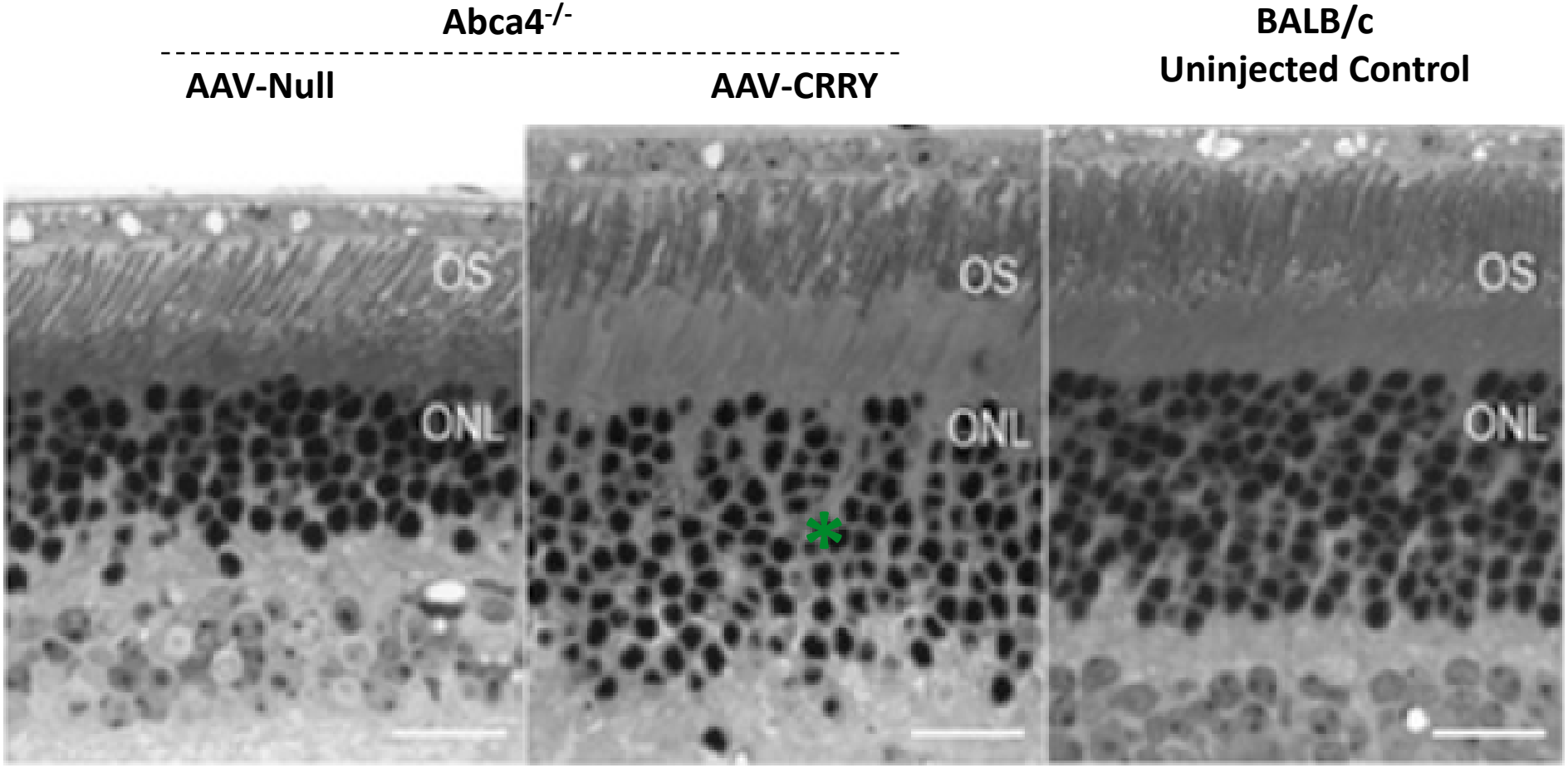
PNAS

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.

Complement Inhibition Rescues Photoreceptors



Representative retinal images from 1 Year old Albino Abca4$^{-/-}$ or BALB/c Mice

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

Ophthalmology: Age-related and Orphan Indications

Strategic Plan

✓ Wet AMD – Zimura	*Phase 2a ongoing*
✓ Dry AMD – Zimura	*Phase 2b ongoing*
❑ Stargardt Disease – Zimura	*Phase 2b to initiate by end of 2017*
❑ IPCV – Zimura	*Phase 2a to initiate by end of 2017*
❑ Posterior Uveitis – Zimura	*Phase 2a to initiate in 2018*
➢ Business Development	*Orphan & Retina indications* *Opportunistic in other ocular diseases* *Ongoing*