UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **July 26, 2017**

OPHTHOTECH CORPORATION

(Exact Name of Registrant as Specified in Charter)

Delaware	**001-36080**	**20-8185347**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

One Penn Plaza, 19th Floor
New York, New York 10119
(Address of Principal Executive Offices) (Zip Code)

Registrant's telephone number, including area code: **(212) 845-8200**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 2.02. Results of Operations and Financial Condition.

On July 26, 2017, Ophthotech Corporation announced its financial results for the quarter ended June 30, 2017. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 7.01. Regulation FD.

On July 26, 2017, Ophthotech Corporation issued a press release announcing updates to its business plan as part of its ongoing strategic review. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference.

On July 26, 2017, Ophthotech Corporation posted an investor presentation to its website at http://investors.ophthotech.com/events.cfm. A copy of the investor presentation is furnished as Exhibit 99.3 to this Current Report on Form 8-K and is incorporated herein by reference.

The information in this Form 8-K (including Exhibits 99.1, 99.2 and 99.3) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. The furnishing of this information hereby shall not be deemed an admission as to the materiality of any such information.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits:

The following Exhibit relating to Item 2.02 shall be deemed to be furnished, and not filed:

99.1 Press Release dated July 26, 2017.

The following Exhibits relating to Item 7.01 shall be deemed to be furnished, and not filed:

99.2 Press Release dated July 26, 2017; and
99.3 Ophthotech Corporation Investor Presentation dated July 2017.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

OPHTHOTECH CORPORATION

Date: July 26, 2017

By: /s/ Barbara A. Wood

Barbara A. Wood
Senior Vice President, General Counsel and Secretary

EXHIBIT INDEX

Exhibit No.	Description
99.1	Press Release dated July 26, 2017
99.2	Press Release dated July 26, 2017
99.3	Ophthotech Corporation Investor Presentation dated July 2017

Ophthotech Reports Second Quarter 2017 Financial and Operating Results

- Conference Call and Webcast Today, July 26, 2017, at 8:00 a.m. ET –

NEW YORK, NY, July 26, 2017 – Ophthotech Corporation (Nasdaq: OPHT) today announced financial and operating results for the second quarter ended June 30, 2017 and provided a business update on its strategic plan.

The Company also announced today that it is pursuing a strategy to leverage its clinical experience and retina expertise to identify and develop therapies to treat multiple orphan ophthalmic diseases for which there are limited or no treatment options available. In parallel, the Company is continuing its on-going age-related retinal programs and its business development efforts to obtain rights to additional products, product candidates and technologies to treat ophthalmic diseases, particularly those of the back of the eye. Please refer to Ophthotech's press release issued earlier today and the call-in and webcast information below for a discussion of the Company's financial and operating results and a business update.

"We believe that with our strategic plan we will be well positioned as a company with multiple ongoing or planned clinical programs in both orphan retinal diseases as well as in back of the eye indications," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "We are also continuing our business development efforts with the goal of broadening and advancing our pipeline. We are committed to developing treatments for patients with devastating ophthalmic diseases and to maximizing value for our shareholders."

Initial, top-line data from the Company's Fovista® OPH1004 trial, its remaining Phase 3 clinical trial, are expected in the third quarter of 2017. The Company believes the failure of two previous Phase 3 Fovista clinical trials and the failure of a competitor's Phase 2 clinical trial investigating the combination of a PDGF inhibitor and a VEGF inhibitor may be indicative of a low likelihood of success for OPH1004. The Company expects that its strategy for the Fovista® development program for the treatment of wet AMD will be primarily determined by the data from OPH1004, and in the context of the negative data from the Company's previous Phase 3 Fovista clinical trials.

Second Quarter 2017 Financial Highlights

- **Cash Position:** As of June 30, 2017, the Company had $196.4 million in cash, cash equivalents, and marketable securities. The Company expects a 2017 year end cash balance of between $145 million and $160 million, excluding any potential business development activities, and after accounting for the approximately $20 million to $35 million that remains committed to implementing a reduction in personnel, the winding-down of the Phase 3 Fovista® in combination with Lucentis® clinical trials, the termination of the Fovista Expansion Studies, and obtaining initial, top-line data for OPH1004.

- **Revenues:** Collaboration revenue was $1.7 million for the quarter ended June 30, 2017, compared to $28.2 million for the same period in 2016. In June 2016, the Company

earned $28.2 million from the achievement of the final enrollment based clinical milestone under the Company's licensing and commercialization agreement with Novartis Pharma AG. For the six months ended June 30, 2017, collaboration revenue was $3.3 million, compared to $43.9 million for the same period in 2016. Collaboration revenue decreased in both the quarter and six months ended June 30, 2017 due to lower revenue from clinical drug supply shipments and due to the inclusion of the final enrollment-based clinical milestone under the Company's agreement with Novartis Pharma AG in 2016.

- **R&D Expenses:** Research and development expenses were $15.7 million for the quarter ended June 30, 2017, compared to $48.3 million for the same period in 2016. For the quarter ended June 30, 2017, research and development expenses include approximately $1.1 million in costs related to the Company's previously announced reduction in personnel. For the six months ended June 30, 2017, research and development expenses were $47.6 million, compared to $86 million for the same period in 2016. For the six months ended June 30, 2017, research and development expenses include approximately $5.9 million in costs related to the Company's previously announced reduction in personnel. Research and development expenses decreased in both the quarter and six months ended June 30, 2017 primarily due to a decrease in expenses related to the Company's Fovista® Phase 3 clinical program, including manufacturing activities.

- **G&A Expenses:** General and administrative expenses were $8.6 million for the quarter ended June 30, 2017, compared to $10.5 million for the same period in 2016. For the quarter ended June 30, 2017, general and administrative expenses include approximately $0.7 million in costs related to the Company's previously announced reduction in personnel. For the six months ended June 30, 2017, general and administrative expenses were $21.7 million, compared to $25.2 million for the same period in 2016. For the six months ended June 30, 2017, general and administrative expenses include approximately $4.6 million in costs related to the Company's previously announced reduction in personnel and its termination of facilities leases. General and administrative expenses decreased in both the quarter and six months ended June 30, 2017 primarily due to a decrease in costs to support the Company's operations and infrastructure.

- **Net Loss:** The Company reported a net loss for the quarter ended June 30, 2017 of $22.2 million, or ($0.62) per diluted share, compared to a net loss of $29.9 million, or ($0.85) per diluted share, for the same period in 2016. For the six months ended June 30, 2017, the Company reported a net loss of $65.3 million, or ($1.82) per diluted share, compared to a net loss of $66.2 million, or ($1.88) per diluted share, for the same period in 2016.

Conference Call/Web Cast Information

Ophthotech will host a conference call/webcast to discuss the Company's financial and operating results and provide a business update. The call is scheduled for July 26, 2017 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 888-280-4443 (USA) or 719-457-2603 (International), passcode 8248330. A live, listen-only audio webcast of the conference call can be accessed on the Investor Relations section of the Ophthotech website at: www.ophthotech.com. A replay will be available approximately two hours following the live call for two weeks. The replay number is 888-203-1112 (USA Toll Free), passcode 8248330. A supplemental slide presentation is available in the "Investor" section of the Ophthotech website prior to the start of the call / webcast.

About Ophthotech Corporation
Ophthotech is a biopharmaceutical company specializing in the development of novel therapeutics for diseases of the eye. For more information, please visit www.ophthotech.com.

Forward-looking Statements
Any statements in this press release 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 press release, 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, the potential utility or commercialization of any of Ophthotech's product candidates and 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, need for additional financing and negotiation and consummation of in-license and/or acquisition transactions 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 press release. 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.

OPHT-G

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alex.vanrees@smithsolve.com

Ophthotech Corporation
Selected Financial Data (unaudited)
(in thousands, except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Statements of Operations Data:				
Collaboration revenue	$ 1,661	$ 28,198	$ 3,323	$ 43,919
Operating expenses:				
Research and development	15,657	48,262	47,636	86,032
General and administrative	8,552	10,489	21,711	25,185
Total operating expenses	24,209	58,751	69,347	111,217
Loss from operations	(22,548)	(30,553)	(66,024)	(67,298)
Interest income	344	446	722	892
Other loss	(1)	(98)	(22)	(68)
Loss before income tax provision	(22,205)	(30,205)	(65,324)	(66,474)
Income tax provision (benefit)	(1)	(260)	2	(228)
Net loss	$ (22,204)	$ (29,945)	$ (65,326)	$ (66,246)
Net loss per common share:				
Basic and diluted	$ (0.62)	$ (0.85)	$ (1.82)	$ (1.88)
Weighted average common shares outstanding:				
Basic and diluted	35,858	35,392	35,831	35,324

	June 30, 2017	December 31, 2016
	(in thousands)	
Balance Sheets Data:		
Cash, cash equivalents, and marketable securities	$ 196,442	$ 289,278
Total assets	201,788	299,630
Deferred revenue	206,653	209,976
Royalty purchase liability	125,000	125,000
Total liabilities	350,608	394,248
Additional paid-in capital	515,615	504,517
Accumulated deficit	(664,285)	(598,959)
Total stockholders' deficit	$ (148,820)	$ (94,618)

Ophthotech Expands Focus with Development for Ophthalmic Orphan Diseases

(Conference Call Scheduled for today, July 26, 2017 at 8:00 a.m. ET)

-Stargardt Disease Clinical Trial Planned to Start Before the End of this Year-

-Focus on Multiple Orphan Programs in Retinal Diseases and Continue Age-related Retinal Programs -

-Business Development Efforts Ongoing -

NEW YORK, NY July 26, 2017 – Ophthotech Corporation (Nasdaq: OPHT) today announced that the Company is pursuing a strategy to leverage its clinical experience and retina expertise to identify and develop therapies to treat multiple orphan ophthalmic diseases for which there are limited or no treatment options available. In parallel, the Company continues its ongoing age-related retinal programs and its business development efforts to obtain rights to additional products, product candidates and technologies to treat ophthalmic diseases, particularly those of the back of the eye. Call-in and webcast information is provided below for a discussion of the Company's financial and operating results and a business update.

- Ophthotech's orphan ophthalmic disease strategy will be led by a randomized, controlled clinical trial assessing the efficacy and safety of Zimura® (avacincaptad pegol), the Company's C5 complement inhibitor, for Stargardt disease, a devastating inherited retinal orphan disease causing vision loss during childhood or adolescence for which patients have no approved treatment. This trial is scheduled to start by the end of this year.

- The Company is continuing its programs in age-related eye diseases, including the planned initiation of a Phase 2a clinical trial of Zimura® in combination with anti-VEGF therapy for wet age-related macular degeneration (AMD) and a Phase 2a clinical trial of Zimura® in combination with anti-VEGF therapy for idiopathic polypoidal choroidal vasculopathy. Both of these trials are scheduled to start by the end of this year.

- The Company's Phase 2/3 clinical trial of Zimura® as a monotherapy for the treatment of geographic atrophy, a form of dry AMD, is ongoing. The Company has maintained a limited number of trial sites for this study and will re-assess its strategy for this study following results of a competitor's complement trial for geographic atrophy, which are expected by year end.

- The National Eye Institute is leading a Phase 1/2 clinical trial of the Company's drug candidate, Fovista® (pegpleranib) in combination with anti-VEGF therapy for the treatment of retinal manifestations of the orphan disease Von Hippel-Lindau Syndrome.

- Ophthotech is also planning a Phase 2a clinical trial of Zimura® for intermediate/posterior non-infectious uveitis, a rare inflammatory disease of the back of the eye, and a potential pre-clinical program with Fovista® for retinoblastoma, a rare cancer of the eye in children. These studies are planned to start in 2018.

"We are excited to move the Company forward with a goal of becoming a leader in the development and commercialization of ophthalmic therapeutics for orphan diseases and for larger indications in the back of the eye, such as age-related retinal diseases," stated Glenn P. Sblendorio, Chief Executive Officer and President of Ophthotech. "We believe that we will be well positioned as a company with multiple shots on goal to bring ophthalmic therapeutics to market. We are also continuing our business development efforts with the goal of broadening and advancing our pipeline. We are committed to developing treatments for patients with devastating ophthalmic diseases and to maximizing value for our shareholders."

Supporting the Company's strategy for the development of Zimura® in Stargardt disease is a recently published independent, peer-reviewed paper in the prestigious journal of *Proceedings of National Academy of Science* (PNAS) from a world-class laboratory at the University of California, Los Angeles (UCLA) that highlights the potential role of complement inhibition in addressing the urgent unmet medical need in Stargardt disease. Additionally, independent literature also supports the scientific evidence for the potential role of complement and specifically the membrane attack complex (MAC) in this disease. The clinical safety data for Zimura® from the Company's completed early stage age-related macular degeneration trials provide a basis to proceed directly to a randomized, controlled clinical trial to assess the safety and efficacy of Zimura® in Stargardt disease.

The Company also announced that it has entered into an agreement with the Foundation Fighting Blindness (FFB). FFB is a highly-distinguished organization recognized for its scientific commitment to orphan inherited retinal degenerative diseases with an established network of scientists and a robust patient registry. Ophthotech has engaged FFB to provide the Company with information from its publicly available ProgStar study, the largest natural history study on Stargardt disease to date, which Ophthotech plans to use in the design of its planned clinical trial of Zimura® for Stargardt disease, and to potentially assist with the Company's other orphan degenerative retinal programs.

"We commend Ophthotech for recognizing the underserved patients afflicted with Stargardt disease for whom currently there is no available FDA approved treatment option," stated Patricia Zilliox, Ph.D., FFB's Clinical Research Institute Chief Drug Development Officer. "We

are delighted and honored to team up with Ophthotech thereby complementing their expertise in ophthalmic drug development with our experience in studying Stargardt disease."

"We are fortunate to have the opportunity to work closely with the Foundation Fighting Blindness," stated Kourous A. Rezaei, M.D., Senior Vice President of Medical Strategy. "We also intend to work closely with the FDA over the next few months to discuss the regulatory pathway for our Zimura® Stargardt program."

The Company also announced changes to its wet AMD program for Zimura®. The Company believes that supplementing anti-VEGF therapy with an anti-complement such as Zimura® in wet AMD may have the potential to further enhance the efficacy of anti-VEGF monotherapy and decrease unwanted side effects in wet AMD from anti-VEGF drugs. A recent peer reviewed publication from the *Journal of Clinical Investigation* from the prestigious Scripps Research Institute citing the role of anti-VEGF therapy in complement activation supports this thesis. Due to a new study design and updated enrollment criteria, the Company will cease enrollment in its current Phase 2a clinical trial of Zimura® in wet AMD, and initiate a new Phase 2a clinical trial to assess whether it can replicate findings from its previous Phase 1/2a clinical trial. The Company will be assessing a range of dosing regimens before committing to a larger and more costly trial. This trial is scheduled to initiate before the end of the year.

"The opportunities to develop orphan drugs for ophthalmic diseases along with some intriguing new developments regarding the role of complement in anti-VEGF therapy allow us to focus our resources and efforts on science-driven solutions in addressing the unmet need in ophthalmic diseases," stated Mr. Sblendorio. "In addition, we have reviewed a large number of assets and technology platforms over the past few months and are actively continuing to review, in a prudent manner, assets or technology platforms which would fit into our strategic goals in addition to other compelling ophthalmology opportunities."

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OPHTHOTECH

Corporate Overview

NASDAQ: OPHT

July 2017

Forward-looking statements

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Strategic Objective

Science driven, retina focused company with multiple programs concentrated in orphan and age-related indications

Timeline/Planned Milestones on the Near Horizon

2017

- ✓ **Set Strategic Plan**
- ☐ **Stargardt Disease – Zimura**
  Initiate program — *By end of this year*
- ☐ **Wet AMD – Zimura**
  Initiate Phase 2a trial — *By end of this year*
- ☐ **IPCV – Zimura**
  Initiate Phase 2a trial — *By end of this year*
- ☐ **Dry AMD – Zimura**
  Phase 2/3 trial ongoing — *Strategic decision (by end of this year)*

2018

- ☐ **Posterior Uveitis – Zimura** — *Planning Phase 2a program*
- ☐ **Von Hippel Lindau – Fovista** — *Initial data*
- ☐ **Retinoblastoma – Fovista** — *Planning pre-clinical program*

Multiple Shots on Goal

Zimura (Complement C5 inhibitor)

Stargardt Disease (Orphan)	• Plan to initiate program by end of this year • Engaged with Foundation Fighting Blindness (ProgStar study data)
Wet AMD	• Plan to initiate Phase 2a trial by end of this year • Assessing a range of dosing regimens in combination with anti-VEGF
Idiopathic Polypoidal Choroidal Vasculopathy (IPCV)	• Plan to initiate Phase 2a trial by the end of this year
Dry AMD (GA)	• Phase 2/3 monotherapy trial currently ongoing • Awaiting results of competitor for strategic decision making
Intermediate/Posterior Uveitis (Orphan)	• Planning Phase 2a trial to initiate in 2018

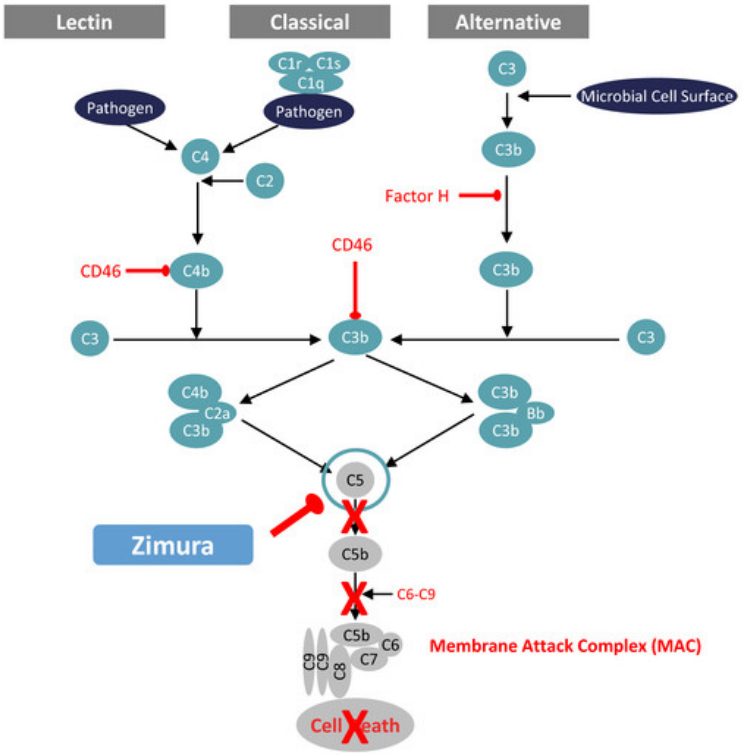
Fovista (anti-PDGF)

Von Hippel-Lindau Syndrome (Orphan)	• Phase 1/2 trial is ongoing • Collaboration with the National Eye Institute
Retinoblastoma (Orphan)	• Planning pre-clinical program

Zimura, C5 Complement Inhibitor

Ophthalmic Orphan and Age-Related Eye Diseases

Complement Pathway



Source: OPHT internal

Zimura, C5 Complement Inhibitor

Stargardt Disease
(Orphan Indication)

Rationale for Development of Zimura in Stargardt

A devastating inherited retinal orphan disease that causes vision loss during childhood/adolescence with no FDA approved treatment

- High unmet medical need
  - Estimated prevalence (US) of ~32,000 - 41,000 patients[(1)]
  - No FDA approved treatment available
- Scientific evidence[(2)]
  - 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
- Orphan disease
  - May have potential for priority review voucher
  - Seven year exclusivity
- OPHT plans to initiate program by the end of this year
  - Randomized, control clinical trial
  - Identified and engaged potential trial sites

Sources:
1) Blacharski PA . Fundus flavimaculatus. In: Newsome DA ed. Retinal Dystrophies and Degenerations. New York: Raven Press; 1988:135–159.
2) 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

OPHT Engages Foundation Fighting Blindness

- OPHT entered into an 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

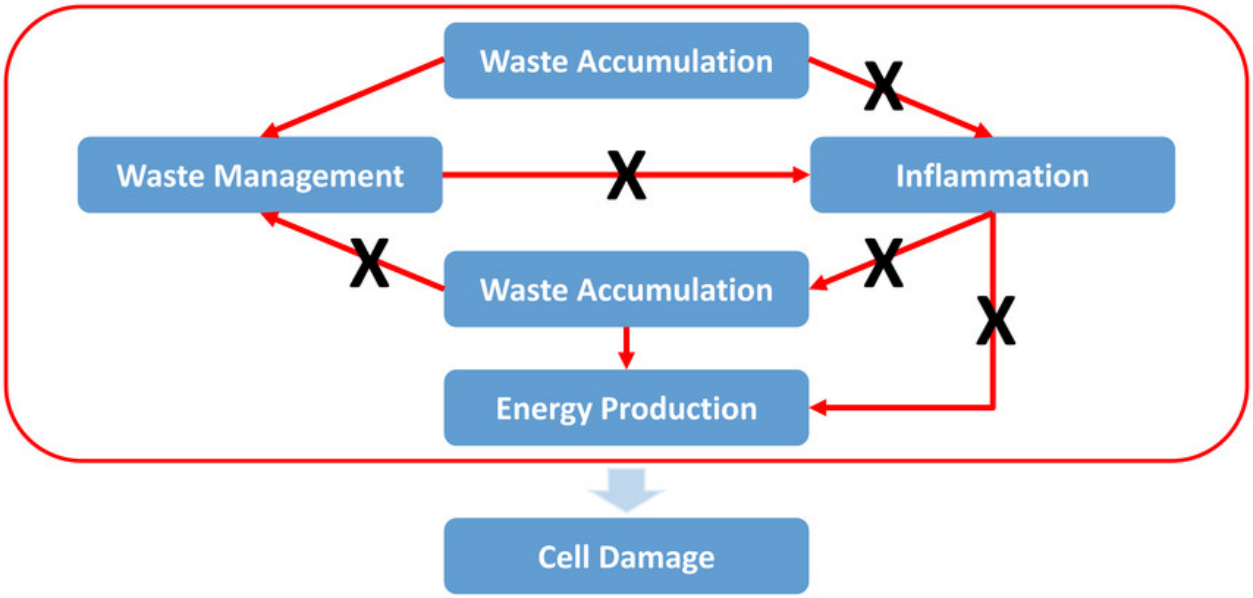
- OPHT gains access to FFB's publicly available **ProgStar** study
  - Largest Natural History Study of Stargardt Disease
  - OPHT plans to leverage information in the design of Zimura Stargardt study

FOUNDATION FIGHTING BLINDNESS
CLINICAL RESEARCH INSTITUTE

Gene Mutation: Waste Accumulation —X→ Inflammation

Zimura®

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

Autosomal Recessive Stargardt Disease (STGD1): Disease Overview

Progressive damage to the macula and retina caused by mutations in the ABCA4 gene

- ABCA4 gene makes a protein that normally helps clear away vitamin A byproducts inside photoreceptors
- Lack of the needed protein leads to the accumulation of bisretinoids/lipofuscin/A2E ("wear and tear" pigment: waste)
- Accumulation of waste and associated inflammation (complement activation) in the RPE cells leads to the death of photoreceptors and loss of vision

Complement Inhibition Rescues Light Perceiving Cells

Expression of Complement Inhibitory Protein (CRRY)

Normalized Complement Activity

~2 fold decrease in bisretinoid accumulation

~30% increase in the number of photoreceptor nuclei

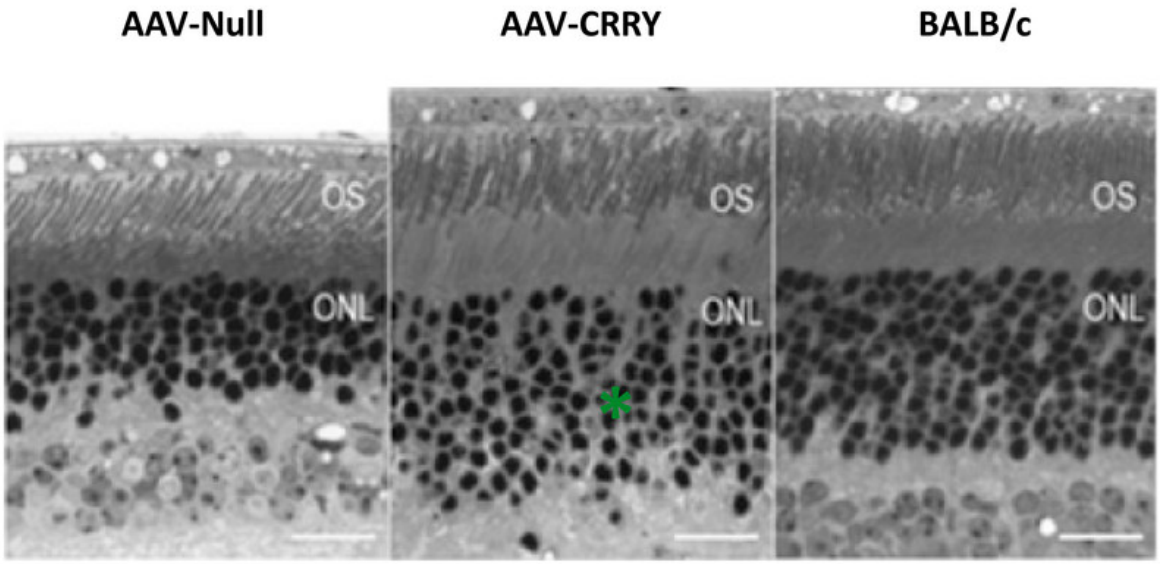
PNAS

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.

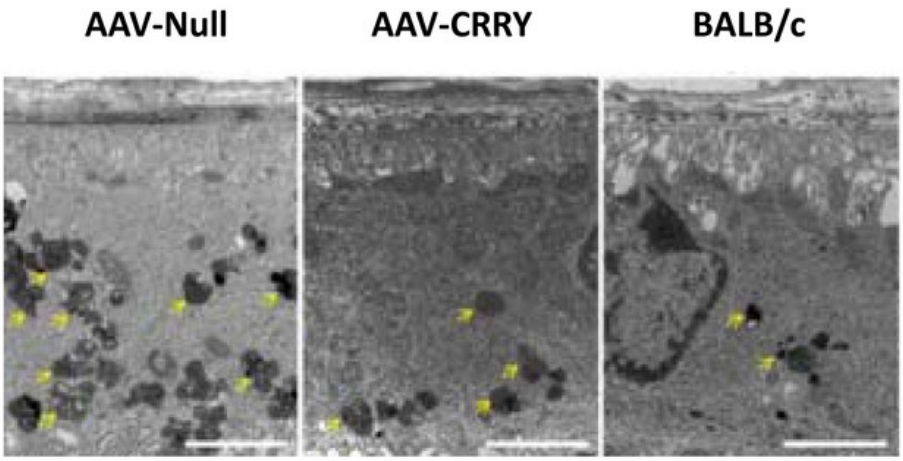
Decreased Complement Activity: Rescued Photoreceptors



1 Year old Albino Abca4$^{-/-}$ or BALB/c Mice

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

Decreased Complement Activity: Decreased Lipofuscin Accumulation

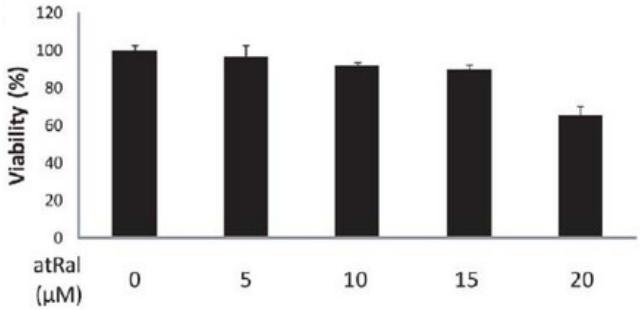


1 Year old Albino Abca4$^{-/-}$ or BALB/c Mice

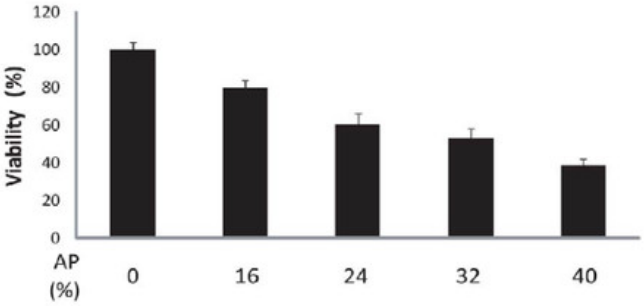
Source: Proc Natl Acad Sci U S A. 2017; 114(15):3987-3992.

Waste + Complement Activation: Significant Reduction in RPE Viability

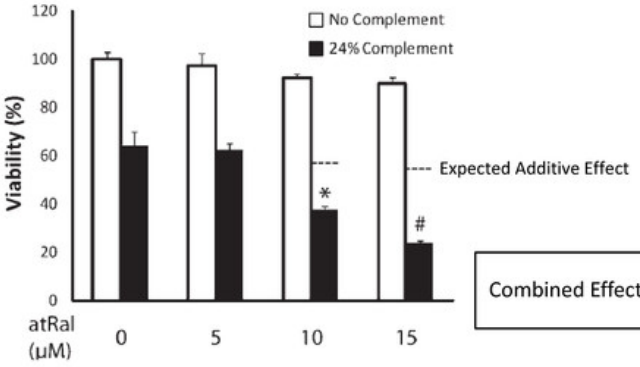
Waste: all-Trans-Retinal (atRal)



Complement Activation



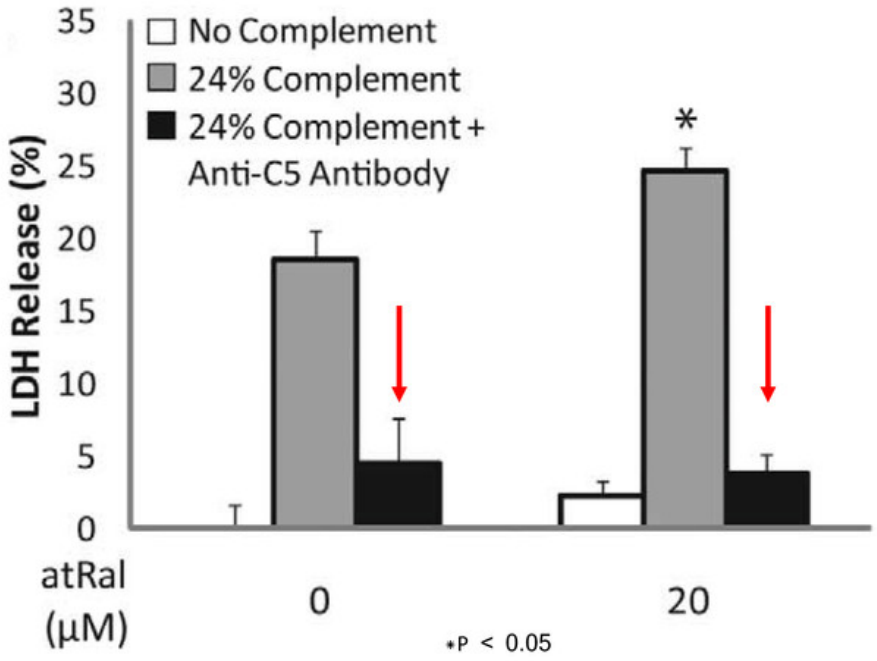
atRal + Complement



Combined Effect >> Expected Additive Effect

Source: Invest Ophthalmol Vis Sci. 2013;54:2669-2677

Anti-C5 Inhibits atRal/Complement Induced Cell Toxicity



Source: Invest Ophthalmol Vis Sci. 2013;54:2669-2677

Zimura, C5 Complement Inhibitor

Wet and Dry Age-Related Macular Degeneration

Rationale for Development of Zimura in Wet AMD

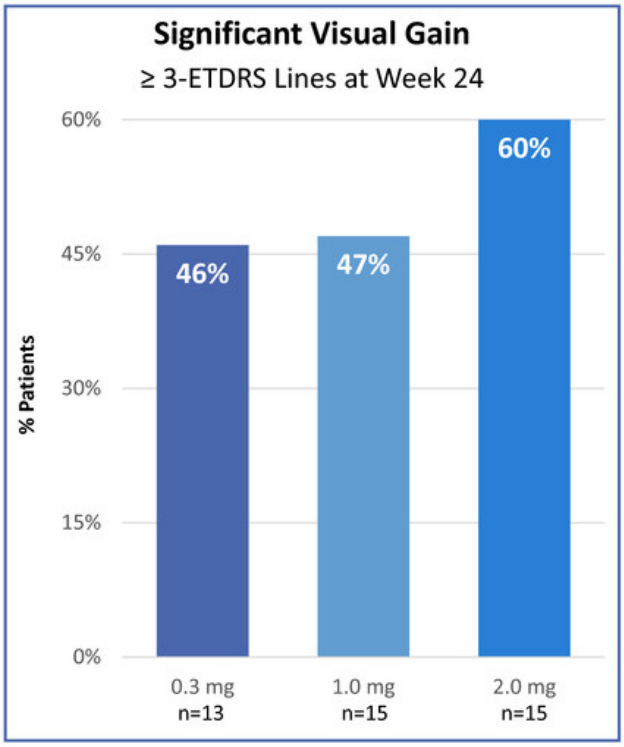
A disease characterized by abnormal neovascularization into the retina, which leads to central vision loss

- Unmet medical need – major market opportunity:
  - Anti-VEGF monotherapy
    - Shown to reach a ceiling effect
    - Majority of patients do not reach a visual acuity of > 20/40
    - In the real world most patients lose vision over time
- Patients receiving anti-VEGF monotherapy may develop geographic atrophy: 20% at two years and ~38% at 5 years have geographic atrophy (CATT study) [1]
- New developments in the role of complement in anti-VEGF therapy
  - VEGF upregulates complement factor H, which is a complement inhibitory factor (Research from Scripps Laboratories in San Diego) [2]
- Completed Zimura Phase 1/2a
- Phase 2a trial to initiate by the end of this year

Sources:
1) Ophthalmology 2014;121:150-161.
2) 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 0.5mg Lucentis
- Safety:
  - All doses well tolerated; no Adverse Events considered to be related to Zimura



Rationale for Development of Zimura in Dry AMD

Geographic Atrophy, a disease characterized by atrophy of the retina, which leads to central vision loss

- An unmet medical need – major market opportunity
- Phase 2/3 clinical trial of monotherapy Zimura is ongoing
- Strategic decision based on the competitor's study outcome available in the second half of 2017

Conclusion: Large market with no available treatment options supports a second to market therapy

Zimura, C5 Complement Inhibitor

Idiopathic Polypoidal Choroidal Vasculopathy (IPCV)

Rationale for Development of Zimura in Idiopathic Polypoidal Choroidal Vasculopathy

A retinal disease involving the choroidal vasculature characterized by the presence of polypoidal lesions, which leads to vision loss

- Unmet medical need
  - Estimated prevalence (U.S.) ~80,000 – 160,000 (1)
- Scientific rationale (2)
  - Response to anti-VEGF monotherapy may be limited
  - Anti-VEGF treatment may lead to complement activation
- Plan to initiate Phase 2a trial by the end of this year

Sources:
1) OPHT Estimate based on published data
2) J Clin Invest. 2017;127(1):199–214. Br J Ophthalmol, 2010: 94(3), 297-301. Br J Ophthalmol. 2008 Jan;92(1):70-3.

Zimura, C5 Complement Inhibitor

Non-Infectious Intermediate/Posterior Uveitis
(Orphan Indication)

Rationale for Development of Zimura in Uveitis

Non-infectious intermediate/posterior uveitis: A rare inflammatory disease of the back of the eye

- Unmet medical need
  - Need for effective intermediate/posterior uveitis treatments with minimal local and systemic adverse events
  - Estimated total Uveitis prevalence (U.S.)*: 38 in 100,000, or about ~120,000 (1)
- Scientific rationale (2)
  - Anti-C5, sCRRY inhibits uveitis in Experimental Autoimmune Uveitis (EAU) model
  - C5 knock-out mice had decreased uveitis severity compared with wild-type mice
  - C3aR/C5aR deficient mice resistant to EAU
  - Complement Factor B inhibition decreases EAU in mouse model
- Planning to initiate Phase 2a trial in 2018

Conclusion: Reduction of complement activity may lead to a decrease in inflammation

Sources:
1) Orphanet J Dis. 2012; 7: 57. Published online 2012 Aug 29. doi:10.1186/1750-1172-57
2) Clin Exp Immunol. 2010 Mar;159(3):303-14. Exp Eye Res. 2006 Mar;82(3):389-94. ARVO2017; program 536, Board #: B0091.Eur J Immunol. 2010 Oct;40(10):2870-81.

* Intermediate/posterior uveitis are forms of uveitis found in the vitreous and retina; prevalence for these forms are less than the total uveitis prevalence

Fovista, Anti-PDGF

Von Hippel-Lindau Syndrome
(Orphan Indication)
and
Retinoblastoma
(Orphan Indication)

Rationale for Development of Fovista in Von Hippel-Lindau Syndrome

An orphan disease where the ocular manifestation presents as retinal capillary hemangiomas that may leak, induce traction and cause vision loss

- Unmet medical need
  - No optimal treatment option available
  - Estimated Prevalence (U.S.): ~7,000 (1)

- Scientific rationale
  - PDGF and VEGF are involved in the proliferation of the capillary hemangioma (2)
  - Anti-VEGF + anti-PDGF combination therapy has the potential to slow down the progression of the disease

- Collaboration with National Eye Institute
  - Phase 1/2a trial ongoing

Sources:
1) Surv Ophthalmol. 2001 Sep-Oct;46(2):117-42.
2) Annu Rev Pathol. 2007;2:145-73.

Rationale for Development of Fovista in Retinoblastoma

An orphan cancer in children resulting from the growth of immature retinal cells leading to loss of vision, eye, and death

- Unmet medical need
  - No optimal treatment for metastatic tumor
  - Potential to be first to market to prevent or decrease the likelihood of metastasis
  - Estimated Prevalence (U.S.): 1 out of every 20,000 births per year /200 new cases a year [1]

- Scientific rationale
  - Retinoblastoma cell lines express PDGF receptor [2]
  - Planning pre-clinical program

Sources:
1) Int J Ophthalmol.2011; 4(1): 103–109. Published online 2011 Feb 18. doi: 10.3980/j.issn.2222-3959.2011.01.24
2) Eye. 2013; 27: 92-99

Diversified Ophthalmic Company

- Positioned to identify value driven solutions for ophthalmic diseases with significant unmet need
- Orphan Ophthalmology (3 current or planned clinical programs, 1 planned pre-clinical) led by a program for Stargardt Disease
  - Significant need to treat underserved patients with rare ophthalmic diseases
  - Potential for faster, less costly clinical trials
  - Limited product competition
  - Regulatory exclusivity for 7 years in US/10 years in EU
- Age-related Ophthalmology for wet and dry AMD currently ongoing
  - Multi-billion dollar market opportunities
- Continue business development strategy
  - Focus on retina & orphan; opportunistic in other ocular diseases
- Significant cash position
  - $196M in cash, cash equivalents and available for sales securities as of June 30, 2017
  - Expect 2017 year-end cash balance to range between $145 million to $160 million*
- Ophthalmic drug development expertise
  - Multiple retina specialists
  - Experienced clinical development team

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

Timeline/Planned Milestones on the Near Horizon

2017

✓ **Set Strategic Plan**	
❑ **Stargardt Disease – Zimura**	
Initiate program	*By end of this year*
❑ **Wet AMD – Zimura**	
Initiate Phase 2a trial	*By end of this year*
❑ **IPCV – Zimura**	
Initiate Phase 2a trial	*By end of this year*
❑ **Dry AMD – Zimura**	*Strategic decision*
Phase 2/3 trial ongoing	*(by end of this year)*

2018

❑ **Posterior Uveitis – Zimura**	*Initiate Phase 2a program*
❑ **Von Hippel Lindau – Fovista**	*Initial data*
❑ **Retinoblastoma – Fovista**	*Planning pre-clinical*