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): **January 1, 2019**

OPHTHOTECH CORPORATION
(Exact Name of Registrant as Specified in its Charter)

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

One Penn Plaza, 35th Floor
New York, NY 10119
(Address of Principal Executive Offices) (Zip Code)

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

Not Applicable
(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 (*see* General Instruction A.2. below):

- 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))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company o

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

Forward-Looking Statements

This Form 8-K and Exhibit 99.1 attached hereto contain forward-looking statements of Ophthotech Corporation ("Ophthotech" or the "Company") that involve substantial risks and uncertainties. Any statements in this Form 8-K and Exhibit 99.1 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 Form 8-K and Exhibit 99.1, 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 research and development activities, the potential utility of its product candidates, and the potential for its business development strategy, including its collaborative gene therapy research programs and any potential in-license or acquisition opportunities. Such forward-looking statements involve substantial risks and uncertainties that could cause Ophthotech's preclinical and 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 the conduct and design of research and development programs and clinical trials, availability of data from these programs, 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 Form 8-K. 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.

Item 2.02 Results of Operations and Financial Condition

Although it has not finalized its full financial results for the fourth quarter and fiscal year ended December 31, 2018, the Company will announce during the 37th Annual J.P. Morgan Healthcare Conference, which begins on January 7, 2019, that it expects to report that it had approximately $131 million in cash and cash equivalents as of December 31, 2018.

The information contained in this Item 2.02 of Form 8-K is unaudited and preliminary, and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2018 and its results of operations for the three months and year ended December 31, 2018. The audit of the Company's financial statements for the year ended December 31, 2018 is ongoing and could result in changes to the information set forth above. The Company anticipates making a public announcement of its results of operations for the fourth quarter and fiscal year ended December 31, 2018 on or about February 27, 2019.

Item 5.02 Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.

On January 1, 2019, the Board of Directors (the "Board") of the Company increased the size of the Board from eight to nine members and elected Calvin (Cal) W. Roberts, M.D., as a director of the Company. Dr. Roberts was appointed as a Class II director and will serve in accordance with the Amended and Restated Bylaws of the Company until the 2021 annual meeting of stockholders and thereafter until his successor is duly elected and qualified or until his earlier death, resignation or removal.

In accordance with the Company's director compensation policy (the "Policy"), Dr. Roberts will receive (i) annual cash compensation of $45,000 for his service as a director, and (ii) reimbursement for reasonable travel and other expenses incurred in connection with attending meetings of the Board and committees thereof. In addition, in accordance with the Policy, Dr. Roberts was granted a stock option to purchase up to 32,000 shares of the Company's common stock at a per share exercise price of $1.20, which was the closing price of the Company's common stock on Monday, December 31, 2018, the trading day immediately preceding the grant date of Tuesday,

January 1, 2019. The option will vest monthly in equal amounts over a three-year period following the date of grant, subject to continued service with the Company.

Item 7.01 Regulation FD Disclosure.

The Company's Chief Executive Officer and President, Glenn Sblendorio, will be presenting on January 10, 2019 at the 37th Annual J.P. Morgan Healthcare Conference. The slides to be used during Mr. Sblendorio's presentation are attached hereto as Exhibit 99.1 and the information contained therein is incorporated herein by reference.

The information in Exhibit 99.1 of this Form 8-K 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 8.01 Other Events

2018 Year End Cash and Cash Equivalents

The information in Item 2.02 of this Form 8-K is incorporated by reference.

Update on Zimura Clinical Trials

As of the close of business on January 4, 2019, the Company enrolled a total of 57 patients for its OPH2005 randomized, double-masked, sham controlled, multi-center Phase 2b clinical trial evaluating the safety and efficacy of Zimura® (avacincaptad pegol) monotherapy for the treatment of autosomal recessive Stargardt disease (the "STGD1 Trial"). The Company plans to complete enrollment for the STGD1 Trial in the first quarter of 2019 and expects to enroll a total of 60 to 65 patients. The Company expects that initial, top-line data from this clinical trial will be available in the second half of 2020.

The Company has decided to discontinue its OPH2006 randomized, dose-ranging, open-label Phase 2a clinical trial of Zimura in combination with the anti-vascular endothelial growth factor agent Eylea® for the treatment of idiopathic polypoidal choroidal vasculopathy, or IPCV, in patients who have not responded to Eylea monotherapy. To date no patients have been enrolled in this clinical trial.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

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

99.1	Ophthotech Corporation Presentation for the 37th Annual J.P. Morgan Healthcare Conference dated January 2019

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: January 7, 2019

By: /s/ David F. Carroll
David F. Carroll
Senior Vice President, Chief Financial Officer and Treasurer

Ophthotech

vision is our mission

37th Annual J.P. Morgan Healthcare Conference

Glenn P. Sblendorio
Chief Executive Officer and President

NASDAQ: OPHT

January 2019

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 research and development activities, the potential utility of its product candidates, its expectations with respect to the financial impacts and benefits to Ophthotech of the acquisition of Inception 4, and the potential for its business development strategy, including its collaborative gene therapy research programs and any potential in-license or acquisition opportunities. Such forward-looking statements involve substantial risks and uncertainties that could cause Ophthotech's preclinical and 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 the conduct and design of research and development programs and clinical trials, availability of data from these programs, 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 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.

A Leader in Developing Gene Therapy and Novel Therapeutics for the Treatment of Retinal Diseases

Well-Positioned as a Leader in Retinal Diseases

- **Diversified Portfolio in Gene Therapy and Therapeutics**
  - Completed three gene therapy deals in orphan retinal indications and engaged gene therapy manufacturing partner
  - Expanded therapeutic pipeline with acquisition of Versant Ventures' Inception 4
- **Expanded the Board of Directors by Adding Leading Industry Experts**
  - Adrienne L. Graves Ph.D., former CEO of Santen
  - Jane Pritchett Henderson, former CFO of Voyager Therapeutics
  - Calvin W. Roberts M.D., CMO, Eye Care at Bausch Health Companies
- **Expanded Expertise in Regulatory and Gene Therapy Manufacturing In-house and with Leading Consultants in Gene Therapy**
- **Strong Cash Position and Well-Capitalized**
  - ~$131 million in cash and cash equivalents as of 12/31/18[1]
  - Cash runway through 2021[2]
  - No debt (Novo A/S agreement ended; no cash impact)

1 Unaudited estimate
2 Based on current programs to next data read-outs; excludes any potential business development activities or any other changes to the Company's current R&D programs

Pipeline Strategy: Build Sustainable Long-term Growth

	Indication	Research	Pre-clin.	Phase 1	Phase 2	Phase 3	Status / Planned Milestones
Therapeutics	**GA secondary to Dry AMD** Zimura monotherapy						• Phase 2b: top-line data **4Q 2019**
	Stargardt Disease (STGD1) Zimura monotherapy						• Phase 2b: top-line data **2H 2020**
	GA secondary to Dry AMD HtrA1 Inhibitor						• IND filing: late **2020**
Gene Therapy	**RHO-adRP** AAV vector						• Phase 1/2: initiate **2020**
	Best Disease AAV vector						• Phase 1/2: initiate **2021***
	LCA10 miniCEP290 AAV vector						• Ongoing*
	STGD1 miniABCA4 AAV vector						• Ongoing*
	AAV Gene Delivery Technology						• Ongoing*

* Option to in-license resulting IP

Gene Therapy for Retinal Diseases

Strategic Scientific Collaborations with Leading Academic Gene Therapy Centers

Penn
University of Pennsylvania

UF University of Florida

Ophthotech

University of Massachusetts Medical School
UMASS

Horae (红瑞) Gene Therapy Center

Gene Therapy Programs

Gene Therapy

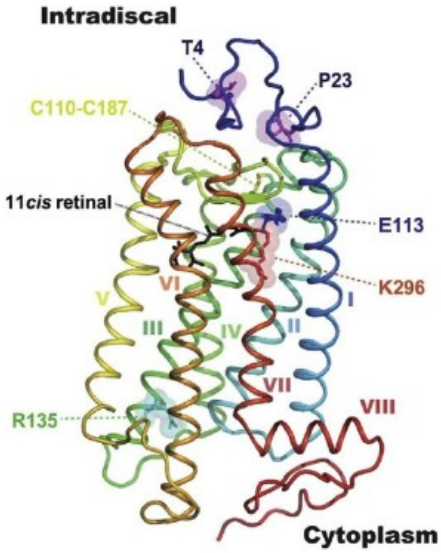
Indication	Research	Pre-clin.	Clinical	Status / Planned Milestones
RHO-adRP AAV vector				• Phase 1/2 initiate **2020**
Best Disease AAV vector				• Phase 1/2: initiate **2021**
LCA10 miniCEP290 AAV vector				• Ongoing
STGD1 miniABCA4 AAV vector				• Ongoing
AAV Gene Delivery Technology				• Ongoing

Rhodopsin-Mediated Autosomal Dominant Retinitis Pigmentosa

Rhodopsin-Mediated Autosomal Dominant Retinitis Pigmentosa

- Retinitis pigmentosa (RP): most prevalent inherited retinal dystrophy
- Bilateral degeneration of rod and cone photoreceptors that ultimately leads to night blindness and progressive visual impairment
- adRP: The most common autosomal dominant retinal disease
- More than 150 identified rhodopsin gene (*RHO)* mutations

Rhodopsin Molecule



Source: Prog Retin Eye Res 2018; 62:1-23.

RHO-adRP: Phase 1/2 Clinical Trial Planned to Initiate in 2020

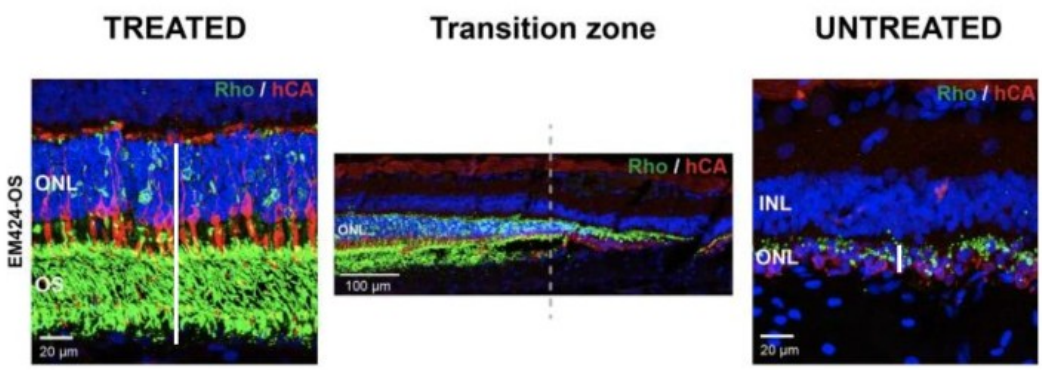
- **Mutation independent strategy**
- **Knockdown and replacement with a single AAV vector**
  - Suppression of endogenouns mutated toxic rhodopsin protein
  - Replace with healthy rhodopsin protein
- **Proof-of-concept in two animal models (canine and mouse)**
  - Long-term preservation of retinal structure and function
- **Clear path to IND submission**
  - Ongoing IND enabling and natural history studies
  - CMC strategy in place; engaged gene therapy manufacturing partner

Sources: PNAS September 4, 2018 115 (36) E8547-E8556 and HUMAN GENE THERAPY 23:356–366 (April 2012).

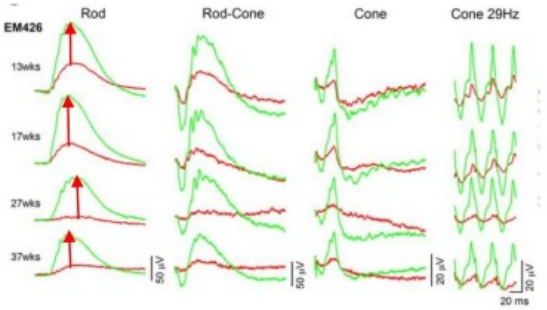
Proof-of-Concept:
Preservation of Retinal Structure and Function in *RHO* Mutant Dogs

scAAV2/5-H1p-*shRNA820* -hOP-*RHO820:* Rescued Outer Retina

Anatomy



Function (ERG)



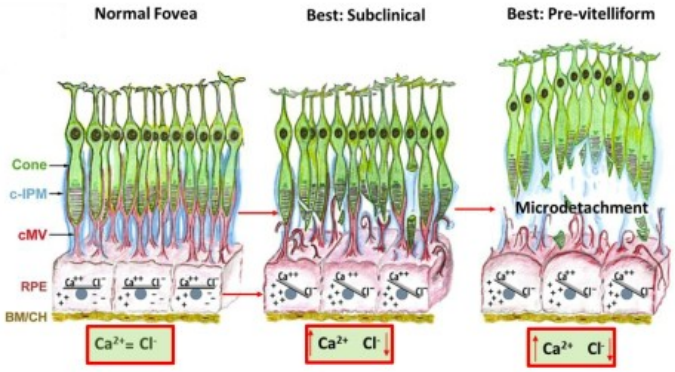
Green: scAAV2/5-RHO820 -shRNA820
RED: Balanced Salt Solution (BSS)

Source: PNAS September 4, 2018 115 (36) E8547-E8556

Best Vitelliform Macular Dystrophy
BVMD/Best Disease

Best Disease (Best Vitelliform Macular Dystrophy)

- An orphan inherited retinal disease caused by mutations in the *BEST1* gene, generally affecting both eyes
- Best1 protein helps regulate chloride ion traffic in retinal cells
- Formation of egg yolk-like vitelliform lesion in macular region leads to macular atrophy and permanent loss of central vision

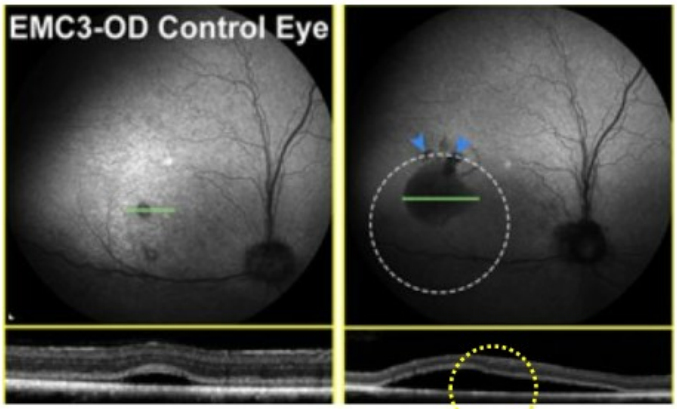


Sources: Proceedings of the National Academy of Sciences Mar 2018, 115 (12) E2839-E2848; 10.1073/pnas.1720662115
Progress in Retinal and Eye Research 58 (2017) 70-88
Ophthotech

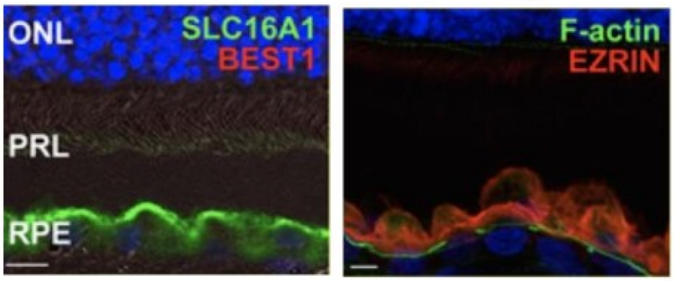
AAV2-*hBest1* Gene Therapy: Resolution of Retinal Microdetachment

Control Injection with BSS

Before injection **BSS: 87 wks post inj**

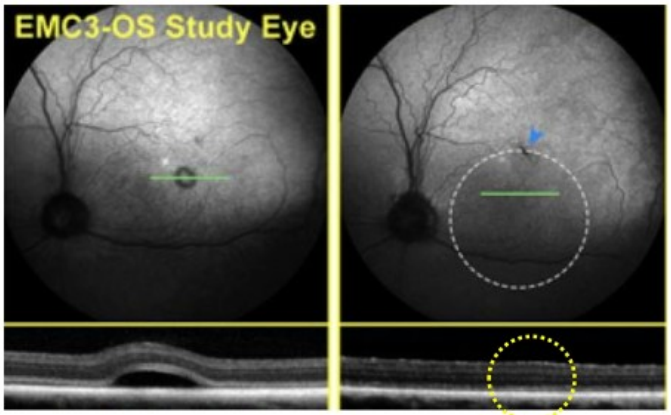


BSS: 103 wks post inj

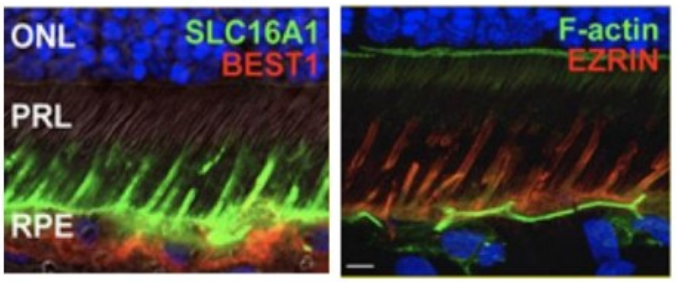


AAV Therapy with Human Transgene

Before injection **AAV: 103 wks post inj**



AAV: 103 wks post inj



Source: Proc Natl Acad Sci U S A. 2018 Mar 20;115(12):E2839-E2848.

BEST1: Phase 1/2 Clinical Trial Planned to Initiate by 2021

- **Proof of concept established in naturally occurring canine models**
- **IND enabling studies planned**
- **Natural history studies planned**
- **CMC strategy in place; engaging gene therapy manufacturing partner**

Minigene Programs: Engineer AAV-amenable Genes to Encode Functionally Optimized Proteins

- **Leber Congenital Amaurosis (LCA10): miniCEP290**
- **Autosomal Recessive Stargardt Disease: miniABCA4**

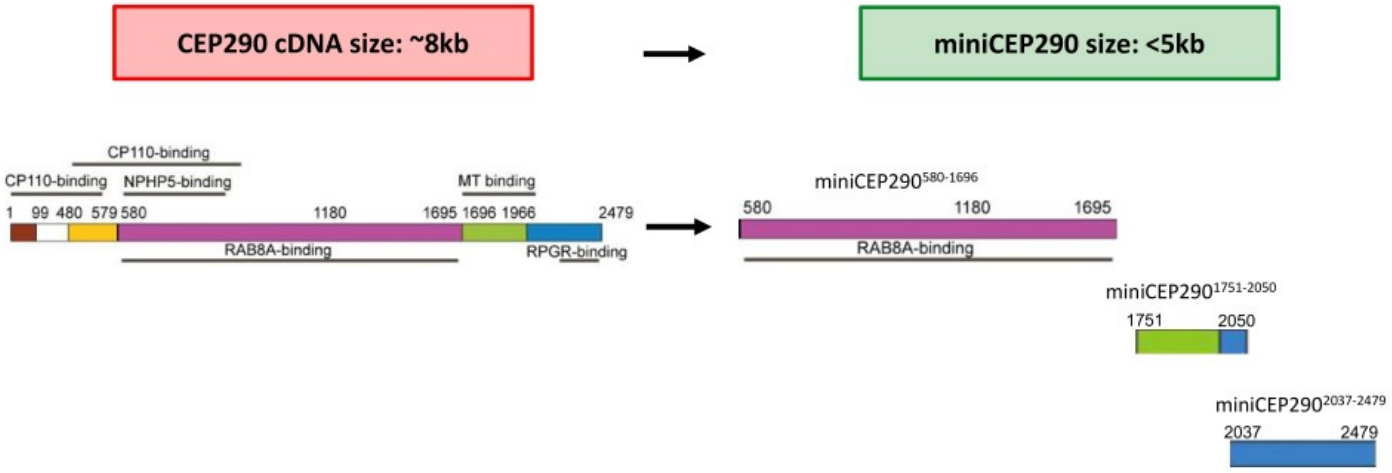
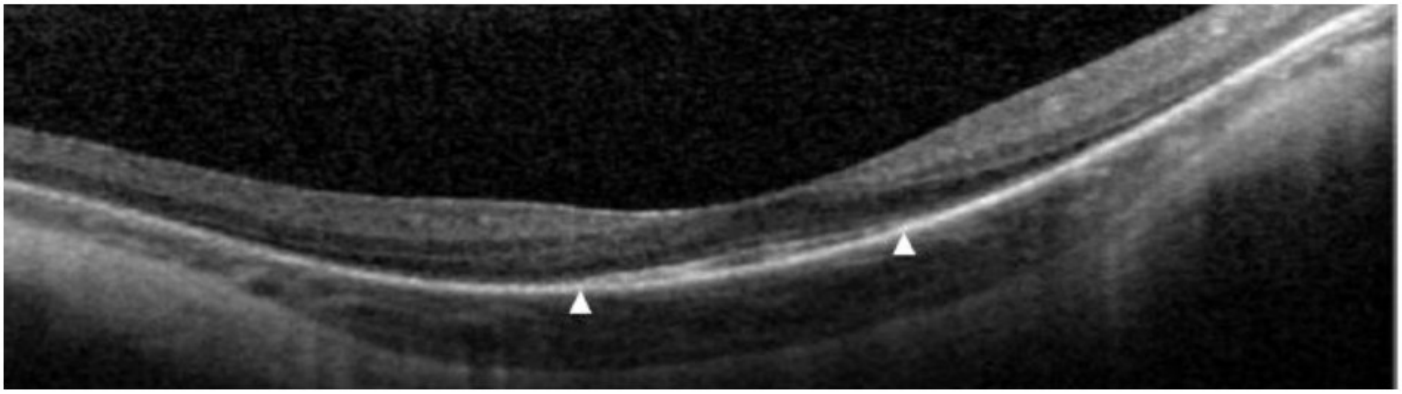


Illustration purposes only

Source: Ophthalmology 2018 Feb 2. pii: S0161-6420(17)32529-0. doi: 10.1016/j.ophtha.2017.12.013.

Leber Congenital Amaurosis (CEP290)

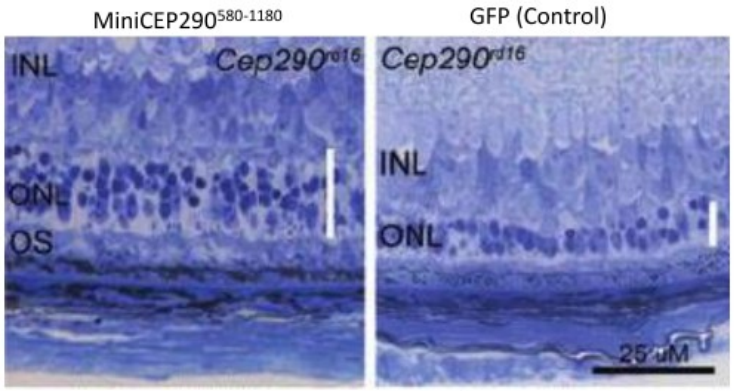
- CEP290 mutations: one of the most common causes of LCA
- Early onset vision loss
- Preserved outer retinal structure in the foveal area
- Extinguished Electroretinogram



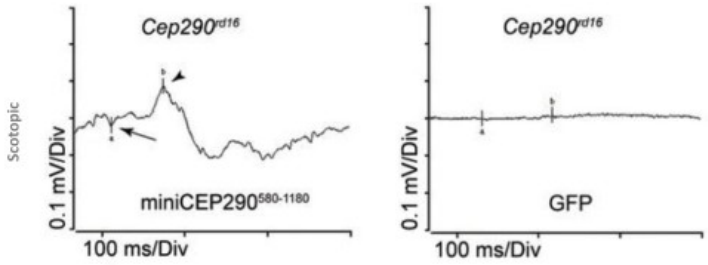
Source: Ophthalmology 2018 Feb 2. pii: S0161-6420(17)32529-0. doi: 10.1016/j.ophtha.2017.12.013.

LCA10: MiniCEP290 Improves Anatomy and Function

MiniCEP290$^{580\text{-}1180}$ Rescued Photoreceptor



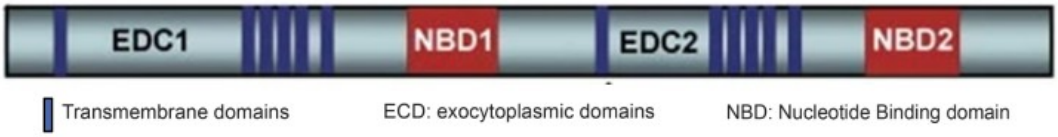
8 x 10^9 vg/µl into the subretinal space of *Cep290^{rd16}* mouse pups (P0/P1), Week 3



Source: Hum Gene Ther. 2018 Jan;29(1):42-50.

Stargardt: miniABCA4

ABCA4 cDNA size: ~7kb and contains multiple functional elements



Generate MiniABCA4 Fragments

↓

Clone into AAV Vectors

↓

Demonstrate *In Vitro* Protein Expression

↓

Proof of Concept *(in vivo)*

Illustration purposes only

Therapeutics Program for Retinal Diseases

Novel Therapeutic Programs

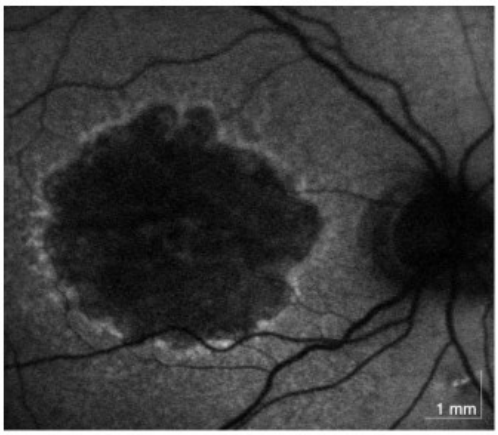
Therapeutics

Indication	Research	Pre-clin.	Phase 1	Phase 2	Phase 3	Status / Planned Milestones
GA secondary to Dry AMD Zimura monotherapy						• Phase 2b ongoing • Top-line data **4Q 2019**
Stargardt Disease (STGD1) Zimura monotherapy						• Phase 2b ongoing • Top-line data **2H 2020**
GA secondary to Dry AMD HtrA1 Inhibitor						• IND filing: late **2020**

* Option to in-license resulting IP

Zimura®, C5 Complement Inhibitor

Geographic Atrophy (GA) Secondary to Dry Age-Related Macular Degeneration



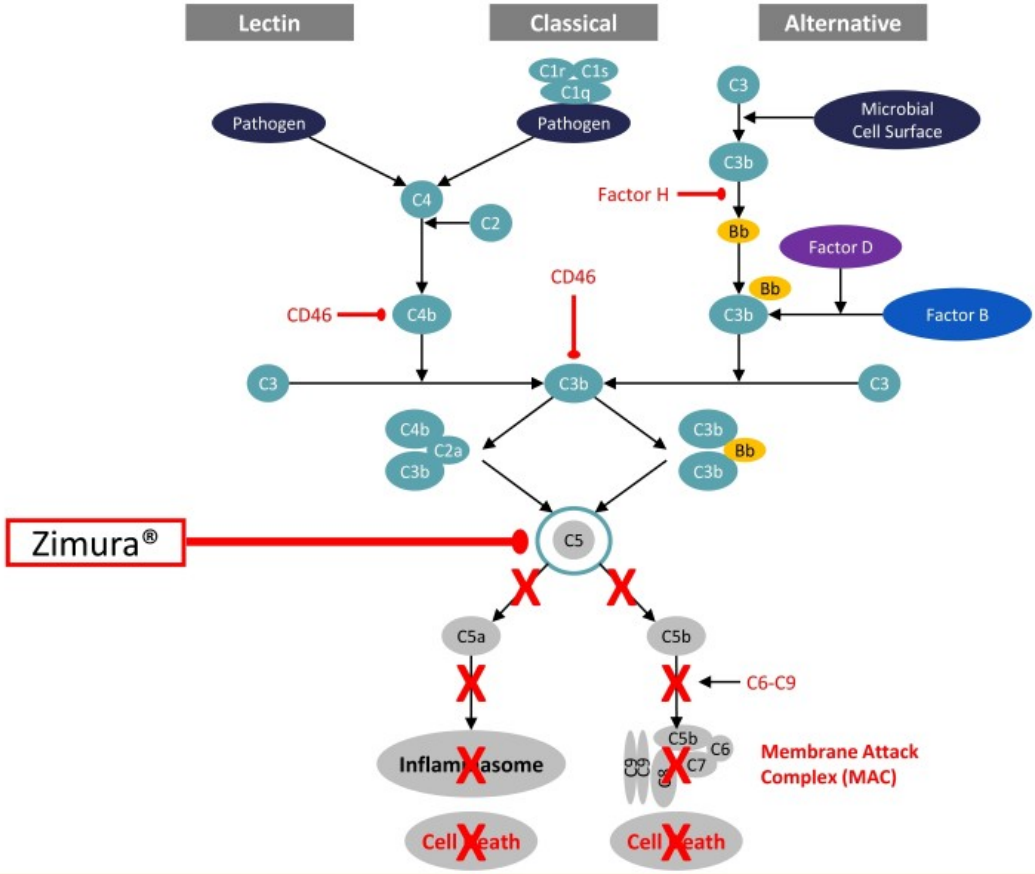
Geographic Atrophy Secondary to Dry AMD

- Major market opportunity – Unmet medical need
  - No FDA/EMA approved treatment options
  - Multi-billion dollar opportunity
  - Growing market size as population ages longer

- Role of complement in dry AMD
  - Genetic link between complement and AMD
  - With aging, complement deposition increases and leads to the formation of inflammasomes and accumulation of Membrane Attack Complex (MAC)
  - Inflammasome formation and MAC accumulation lead to retinal pigment epithelial (RPE) cell death and loss of vision

Sources:
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. Science. 2005 Apr 15;308(5720):385-389; 419-421; 421-424.

Zimura Blocks C5 Cleavage:
Potential to Block Inflammasome Formation & MAC Accumulation → Cell Death

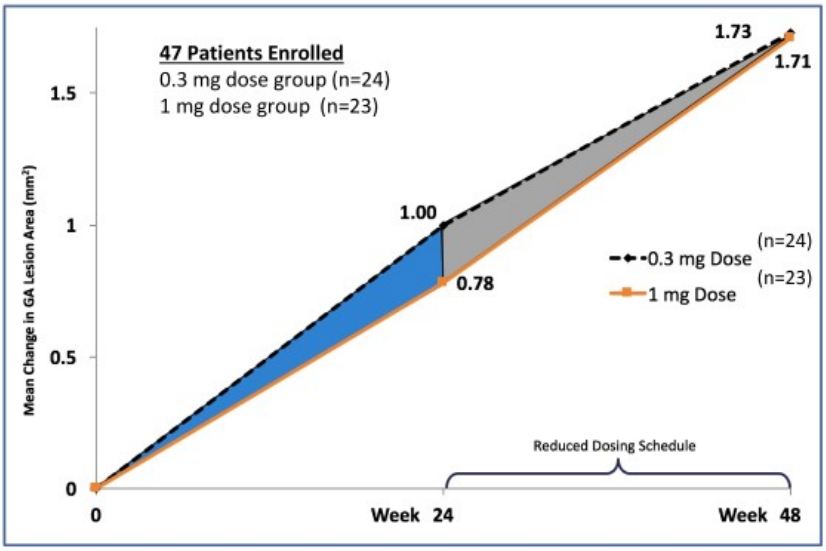


Source: OPHT internal

Dry AMD (GA): Zimura Phase 1/2a– 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)

- **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

Geographic Atrophy: Zimura Phase 2b - Ongoing

- Phase 2b, randomized, double-masked, sham-controlled clinical trial
- Cohorts:
  - Zimura: 3 dose levels
  - Sham
- 286 subjects enrolled; 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
- **Top-line data expected in 4Q 2019**

Zimura, C5 Complement Inhibitor

Autosomal Recessive Stargardt Disease
(Orphan Indication)

Autosomal Recessive Stargardt Disease (STDG1)

- Orphan disease – High unmet medical need
  - No FDA or EMA approved treatment available
- Progressive damage to the macula and retina caused by mutations in the *ABCA4* gene
- *ABCA4* gene makes a protein that normally helps clear away visual cycle byproducts inside retinal cells
- Lack of this protein leads to the accumulation of waste and complement activation leading to retinal cell death and loss of vision

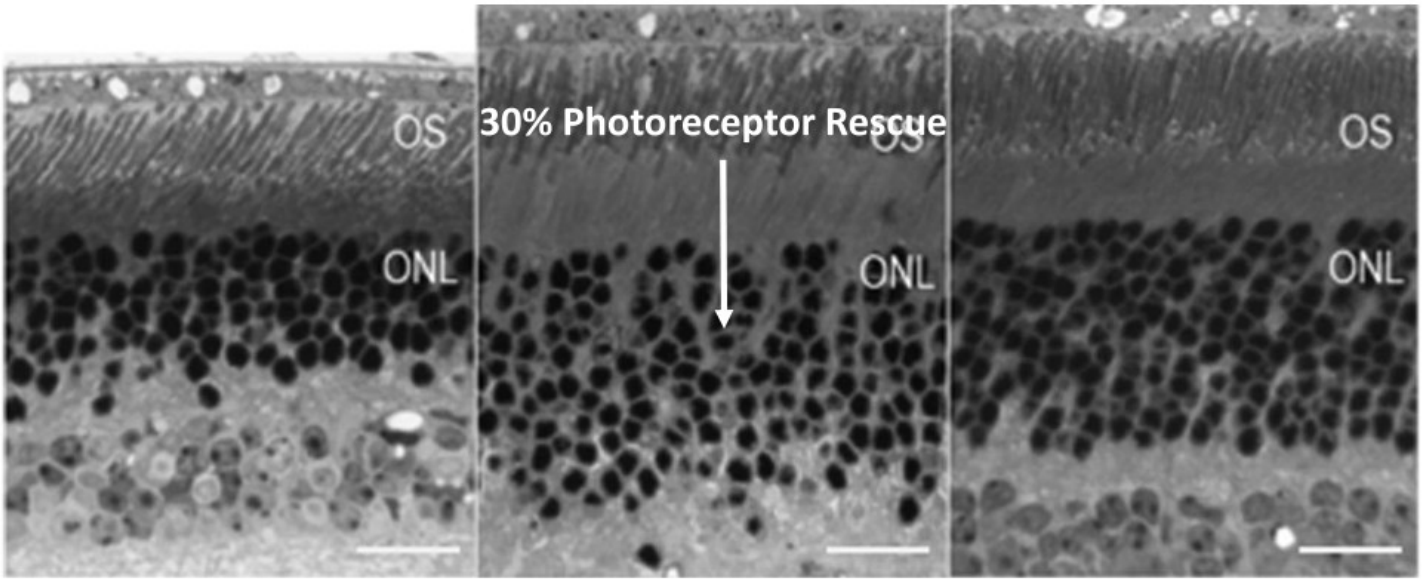
Sources: The Journal of Biological Chemistry. 2011; 286(21): 18593–18601. Proc Natl Acad Sci U S A. 2017; 114(15):3987-3992. Invest Ophthalmol Vis Sci. 2013;54:2669-2677.

Complement Inhibition Rescues Photoreceptors

Abca4$^{-/-}$

AAV-Null | **AAV-CRRY**

BALB/c
Uninjected Control



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.

Stargardt Disease : Zimura Phase 2b – Ongoing

- Phase 2b Clinical Trial
  - Randomized, double masked, sham controlled clinical trial
  - Enrollment to complete in Q1 2019 with ~65 subjects
  - Two arms:
    - Zimura
    - Sham
  - 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 2H 2020**

- Foundation Fighting Blindness
  - Access to FFB's publicly available ProgStar natural history study
  - Patient registry access to facilitate recruitment

FOUNDATION FIGHTING BLINDNESS
CLINICAL RESEARCH INSTITUTE

HtrA1 Inhibitor Therapeutic Program

(Acquisition of Inception 4 from Versant Ventures)

HtrA1 Inhibitor Program

- Acquired Inception 4, a privately held company backed by Versant Ventures
  - Gained worldwide development and commercial rights to HtrA1 inhibitor program to treat age-related retinal diseases
  - OPHT obtained ~$6.1 million in cash
  - Versant a major new investor in OPHT (~5.2 million shares)
  - Versant to identify additional opportunities to potentially expand Ophthotech pipeline

HtrA1 Inhibitor Program:
Small Molecules with High Affinity and Specificity for HtrA1

- **Strong genetic link between HtrA1 & AMD:**
  - Homozygotes have ~8.2 fold increased risk
- **AMD patients overexpress HtrA1:**
  - Increased intracellular expression of HtrA1 inside the RPE cells of AMD patients
  - Increased HtrA1 staining in a majority of drusen of AMD patients' donor eyes
  - Increased HtrA1 protein level in aqueous humor of wet AMD patients
- **Overexpression of HtrA1 protein contributes to AMD:**
  - Damages the extracellular matrix and Bruch's membrane
  - Alters and disrupts RPE cells
  - Upregulates complement
  - Leads to drusen formation
  - Interferes with RPE cell function and secondarily impacting photoreceptors

Sources: Human Molecular Genetics, 2005: 14, 3227–3236. Arch Ophthalmol. 2007;125:55-62. Aging Cell. 2018 May 5:e12710. doi: 10.1111/acel.12710. [Epub ahead of print]. Investigative Ophthalmology & Visual Science January 2017, Vol.58, 162-167. EBioMedicine 27 (2018) 258–274. Science 2006; 314 (5801), 992-993. Cell Cycle 6:9, 1122-1125, 1 May 2007]. Scientific Reports | 7: 14804 | DOI:10.1038. Invest Ophthalmol Vis Sci. 2010;51:3379–3386. PLoS One. 2011;6(8):e22959. doi: 10.1371/journal.pone.0022959. Invest Ophthalmol Vis Sci. 2010;51:3379–3386.

Pipeline Strategy: Build Sustainable Long-term Growth

	Indication	Research	Pre-clin.	Phase 1	Phase 2	Phase 3	Status / Planned Milestones
Therapeutics	**GA secondary to Dry AMD** Zimura monotherapy						• Phase 2b: top-line data **4Q 2019**
	Stargardt Disease (STGD1) Zimura monotherapy						• Phase 2b: top-line data **2H 2020**
	GA secondary to Dry AMD HtrA1 Inhibitor						• IND filing: late **2020**
Gene Therapy	**RHO-adRP** AAV vector						• Phase 1/2: initiate **2020**
	Best Disease AAV vector						• Phase 1/2: initiate **2021***
	LCA10 miniCEP290 AAV vector						• Ongoing*
	STGD1 miniABCA4 AAV vector						• Ongoing*
	AAV Gene Delivery Technology						• Ongoing*

* Option to in-license resulting IP

Business Development and Pipeline Expansion Strategy

Modality agnostic; key focus on gene therapy opportunities

- Compelling science
- Unmet medical need
- Meaningful commercial opportunity

Focused on Execution and Value Creation

- **Diversified Portfolio in Gene Therapy and Therapeutics**
  - Stargardt disease Zimura Phase 2b enrollment completion (Q1 '19)
  - Engaged third party gene therapy manufacturer (Q1 '19)
  - Minigene data (mid-year '19)
  - Dry AMD Zimura Phase 2b data (Q4 '19)
- **Operations and Expertise; Lean and Efficient Execution**
  - Expanded the Board of Directors by adding leading industry experts
  - Expanded expertise in regulatory and gene therapy manufacturing in-house and with leading consultants in gene therapy
  - Infrastructure set at ~35 employees for 2019
- **Strong Cash Position and Well-Capitalized**
  - ~$131 million in cash and cash equivalents as of 12/31/18[1]
  - Cash Runway through 2021[2]
  - No debt (Novo A/S agreement ended; no cash impact)

1 Unaudited estimate
2 Based on current programs to next data read-outs; excludes any potential business development activities or any other changes to the Company's current R&D programs