vision is our mission
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 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 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 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.
Value Creation: Building a Leading Retina Company

Developing Transformative Gene Therapies and Novel Therapeutics for Retinal Diseases

- **Market:** Large and orphan indications
- **Pipeline:** Expansion through business development activities
- **Execution:** Unique in-house expertise in clinical development
- **Strong Cash Position:** Drive future growth
Multi-Modality Approach: Maximize Probability of Success

• Therapeutics
  – Deep understanding and expertise in ophthalmic drug development
    • Multiple retina specialists in management
    • Highly experienced clinical development team
  – Strong global network
    • Well known KOLs
    • Experienced clinical investigators

• Gene Therapy
  – Novel and cutting edge AAV gene therapy:
    • Dual function (knockdown + replace) single AAV vector strategy: RHO-adRP
    • Minigene strategy: LCA10 (CEP290) and Stargardt disease (ABCA4)*
    • AAV gene delivery methods*

*Sponsored research with UMASS Medical School; option to in-license resulting IP
## Pipeline Strategy: Build Sustainable Long-term Growth

<table>
<thead>
<tr>
<th>Indication</th>
<th>Research/Pre-clinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Status</th>
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<tbody>
<tr>
<td>Wet AMD (in combo with anti-VEGF)</td>
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<td></td>
<td></td>
<td>• Zimura: Phase 2a ongoing • Initial top-line data expected <strong>late 2018</strong></td>
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<tr>
<td>GA secondary to Dry AMD (monotherapy)</td>
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<td></td>
<td>• Zimura: Phase 2b ongoing • Initial top-line data expected <strong>Q4 2019</strong></td>
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<tr>
<td>STGD1 (monotherapy)</td>
<td></td>
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<td></td>
<td>• Zimura: Phase 2b ongoing* • Initial top-line data expected <strong>2020</strong></td>
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<tr>
<td>IPCV (in combo with anti-VEGF)</td>
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<td></td>
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<td>• Zimura: Phase 2a ongoing</td>
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<tr>
<td>RHO-adRP AAV vector</td>
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<td></td>
<td>• UPenn sponsored research ongoing • IND-enabling studies planned for <strong>2019</strong> • Phase 1/2 expected to initiate in <strong>2020</strong></td>
</tr>
<tr>
<td>Novel Gene Delivery Methods</td>
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* Option to in-license resulting IP

* First Zimura trial for this indication
Wet AMD: Zimura in Combination with Anti-VEGF

- Major market opportunity – Unmet medical need
- 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
  - **Patients may develop geographic atrophy**
- Role of anti-VEGF in complement activation and atrophy
  - VEGF increases Complement Factor H (CFH)
  - CFH decreases complement activation
  - **Anti-VEGF increases complement activation**
- Adding Zimura to anti-VEGF therapy may improve the efficacy and safety

Wet AMD: Zimura Phase 1/2a – 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
Wet AMD: Zimura Phase 2a – Ongoing

- Phase 2a open-label clinical trial

- N = 64 subjects enrolled

- 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 by the end of 2018
Geographic Atrophy Secondary to Dry AMD

• Major market opportunity – Unmet medical need
  – No FDA/EMA approved treatment options

• 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 and MAC accumulation lead to retinal pigment epithelial (RPE) cell death and loss of vision

Sources:
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 (FAF) at three time points

• Top-line data expected in 4Q 2019
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

**Stargardt Disease: Zimura Phase 2b – Ongoing**

- Phase 2b Clinical Trial
  - Randomized, double masked, sham controlled clinical trial
  - Two arms:
    - Zimura
    - Sham
  - 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**

- Foundation Fighting Blindness
  - Access to FFB’s publicly available ProgStar natural history study
  - Patient registry access to facilitate recruitment
Key Rationale for Ocular Gene Therapy Strategy

• Many ocular orphan indications are due to genetic defects

• Potential to cure diseases with significant unmet medical need

• Eye is an ideal target for gene therapy:
  – Localized delivery, minimizing systemic exposure
  – Immune privileged
  – Depth of monogenic disease characterization
  – Relatively easy access to pathology

• Positive implications for patients and health care providers
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

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

• Mutation independent strategy with single AAV vector technology
  – Silences / knocks out mutated toxic rhodopsin protein
  – Produces healthy wildtype rhodopsin protein

• Proof-of-concept in animal models (canine and mouse)
  – Preservation of retinal anatomy and function

• Clear path to IND submission
  – IND enabling and natural history studies planned

AAV Vectors are Appealing for Ocular Gene Therapy

• Extensive experience with intraocular application
• Well documented safety profile
• Tropism for retinal tissue
• Limited packaging capacity of < 5kb → Minigene Therapy
  - Engineer AAV-amenable genes that encode the functionally optimized proteins
AAV Vector Technology

• Minigene Strategy
  – Leber Congenital Amaurosis (CEP290)
    ▪ CEP290 mutations: one of the most common causes of LCA
    ▪ Early onset vision loss
  – Stargardt Disease
    ▪ Caused by mutations in the ABCA4 gene
    ▪ Progressive damage to the macula and retina

• Novel Gene Delivery Methods
Value Creation: Building a Leading Retina Company

• Upcoming Zimura Data Points
  – Wet AMD in 2018
  – Dry AMD (GA) in 2019
  – Stargardt in 2020

• RHO-adRP Clinical Trial Expected to Initiate in 2020

• Continue Business Development Activities to Expand Portfolio

• Strong Cash Position to Drive Growth
  – $146 million in cash and cash equivalents*

*As of June 30, 2018