

Ophthotech

vision is our mission

NASDAQ: OPHT

3Q 2018 Earnings

Forward-looking Statements

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Ophthotech Adds HtrA1 Inhibitors Through Acquisition of Versant Ventures' Inception 4

- **Strong genetic link between HtrA1 (high temperature requirement A serine peptidase 1 protein) & AMD:**
 - Highest identified odds ratio to develop AMD, homozygotes have ~8.2 fold increased risk
- **AMD patients overexpress HtrA1:**
 - Increased HtrA1 protein level in aqueous humor of wet AMD patients
 - 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
- **HtrA1 protein overexpression may contribute to AMD:**
 - Damages the extracellular matrix and Bruch's membrane
 - Alters and disrupts RPE cells
 - Upregulates complement
 - Leads to drusen
 - Interferes with RPE cell function and secondarily impacting photoreceptors
- **OPHT new therapeutics for AMD: Small molecule inhibitors of HtrA1 with high affinity and specificity for HtrA1**

Sources: Human Molecular Genetics, 2005; 14, 3227–3236. Arch Ophthalmol. 2007;125:55–62. Aging Cell. 2018 May 5:e12710. doi: 10.1111/accel.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.

Ophthotech Enters into Exclusive Option Agreement for AAV Gene Therapy Product Candidates to Treat Best Disease

- **Best Vitelliform Macular Dystrophy (Best disease) is an orphan inherited retinal disease caused by mutations in the BEST1 gene:**
 - The BEST1 gene encodes for bestrophin, a multifunctional protein that regulates ion channels and homeostasis in retinal cells
 - Patients with Best disease develop an egg yolk-like vitelliform lesion in their macular region, which over time leads to macular atrophy and permanent loss of central vision
 - Best disease generally affects individuals in both eyes
 - Prevalence of ~10,000 individuals in the United States and the five major European markets (internal estimate)
- **OPHT new AAV gene therapy product candidates for Best Disease: IND Submission Expected in 2021**
 - Preclinical anatomical proof-of-concept studies conducted by the University of Pennsylvania and the University of Florida have demonstrated promising results in a canine Best disease model

Source: Proceedings of the National Academy of Sciences Mar 2018, 115 (12) E2839-E2848; DOI: 10.1073/pnas.1720662115