



## **Opthea's Phase 1 Data from Wet AMD Clinical Trial Presented at EURETINA Congress 2016**

**Melbourne, Australia, September 12 2016** – Opthea Limited (ASX:OPT), a developer of novel biologic therapies for the treatment of eye diseases, today announced that data from its Phase 1 clinical trial of OPT-302 was presented at the European Society of Retina Specialists (EURETINA) Congress on Saturday, 10<sup>th</sup> September 2016, in Copenhagen, Denmark. OPT-302 is Opthea's lead program, a novel VEGF-C/D 'Trap' therapy for wet age-related macular degeneration (wet AMD).

The presentation titled "*A first-in-human Phase 1/2a study of the novel VEGF-C/D inhibitor OPT-302 alone and in combination with ranibizumab in patients with wet AMD*" was presented by Dr. Pravin Dugel, Managing Partner of Retinal Consultants of Arizona, Clinical Professor at the University of Southern California Eye Institute, Keck School of Medicine, and member of Opthea's Clinical Advisory Board and Clinical Investigator.

The presentation was part of the "*Novelties and Late-Breaking Developments in Retina and Technology*" session and provided an overview of the scientific rationale for targeting VEGF-C/D for the treatment of wet AMD and the recently announced positive data from Opthea's ongoing first-in-human clinical trial of OPT-302 (ClinTrials.gov ID#: NCT02543229). The encouraging results from the study demonstrate safety and tolerability of OPT-302 administered as a monotherapy and in combination with standard of care Lucentis® and suggest that combined administration of OPT-302 + Lucentis® may lead to improved clinical outcomes over Lucentis® alone.

The EURETINA Congress, held September 8-11<sup>th</sup> 2016, was attended by over 4500 professionals from the investor, pharmaceutical and clinical ophthalmology community and is the largest gathering of specialist retinal ophthalmologists and associated healthcare professionals in Europe.

A copy of Dr. Dugel's presentation is available in the "presentations" section of Opthea's website, [www.opthea.com](http://www.opthea.com).

### *Company & Media Enquiries:*

Megan Baldwin, PhD  
CEO & Managing Director  
Opthea Limited  
Tel: +61 (0) 447 788 674  
[megan.baldwin@opthea.com](mailto:megan.baldwin@opthea.com)

### *Join our email database to receive program updates:*

Tel: +61 (0) 3 9826 0399  
[info@opthea.com](mailto:info@opthea.com)  
[www.opthea.com](http://www.opthea.com)

### *Australia:*

Rudi Michelson  
Monsoon Communications  
Tel: +61 (0) 3 9620 3333

### *U.S.A. & International:*

Candice Knoll  
Blueprint Life Science Group  
Tel: +1 415 375 3340, Ext 4

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## **About Opthea Limited**

Opthea (ASX:OPT) is a biologics drug developer focusing on ophthalmic disease therapies. It controls exclusive worldwide rights to a significant intellectual property portfolio around Vascular Endothelial Growth Factor (VEGF)-C, VEGF-D and VEGFR-3. The applications for the VEGF technology, which functions in regulating blood and lymphatic vessel growth, are substantial and broad. Opthea's product development programs are focused on developing OPT-302 (formerly VGX-300, soluble VEGFR-3) for 'back of the eye' disease such as wet age-related macular degeneration (wet AMD).

## **About Wet AMD**

Wet (neovascular) age-related macular degeneration, or wet AMD, is a disease characterised by the loss of vision of the middle of the visual field caused by degeneration of the central portion of the retina (the macula). Abnormal growth of blood vessels below the retina, and the leakage of fluid and protein from the vessels, causes retinal degeneration and leads to severe and rapid loss of vision.

Wet AMD is the leading cause of blindness in the developed world in individuals aged 50 years or older. The prevalence of AMD is increasing annually as the population ages. Without treatment, wet AMD patients often experience a chronic, rapid decline in visual acuity and increase in retinal fluid. Sales of the drug Lucentis® (Roche/Novartis), which targets VEGF-A but not VEGF-C or VEGF-D, were over \$US4.5BN in 2015. Sales of EYLEA® (Regeneron/Bayer), which also targets VEGF-A but not VEGF-C/-D first marketed in November 2011 for the treatment of wet AMD, were over \$US2.6BN in 2015. Approximately half of the people receiving Lucentis®/EYLEA® are classified as non-responders or 'poor' responders and do not experience a significant gain in vision and/or have persistent retinal vascular leakage. There is great opportunity to improve patient responses by targeting more than one factor involved in disease progression. Existing therapies, such as Lucentis® and EYLEA®, target VEGF-A that promotes blood vessel growth and leakage through its receptor VEGFR-2. VEGF-C can also induce angiogenesis and vessel leakage through the same receptor as well as through an independent pathway. Combined inhibition of VEGF-A and VEGF-C/-D, has the potential to improve patient response by more effective inhibition of the pathways involved in disease progression.

## **About OPT-302**

OPT-302 is a soluble form of vascular endothelial growth factor receptor 3 (VEGFR-3) or 'Trap' molecule that blocks the activity of two proteins (VEGF-C and VEGF-D) that cause blood vessels to grow and leak. OPT-302 is currently being investigated in a Phase 1/2A clinical trial in wet AMD patients as a monotherapy and in combination with ranibizumab (Lucentis®). The trial is actively recruiting patients under an FDA approved IND at several US clinical sites. The purpose of the trial is to evaluate the safety, pharmacokinetics (PK) and pharmacodynamics of OPT-302 administered as monthly intravitreal injections for 3 months with and without Lucentis® in patients with wet age related macular degeneration (AMD). The study is being conducted in two parts: Part 1 (Phase 1) comprises an open label, sequential dose escalation that recruited 20 patients and Part 2 (Phase 2A) a randomized dose expansion that will recruit an additional ~30 patients and is aimed at further characterising the safety, pharmacokinetic profile and relationship between dose/PK and clinical activity of OPT-302 (+/- ranibizumab). Further details on the Phase 1/2A trial can be found at: [www.clinicaltrials.gov](http://www.clinicaltrials.gov), Clinical trial identifier: NCT02543229.

## **Inherent risks of Investment in Biotechnology Companies**

There are a number of inherent risks associated with the development of pharmaceutical products to a marketable stage. The lengthy clinical trial process is designed to assess the safety and efficacy of a drug prior to commercialisation and a significant proportion of drugs fail one or both of these criteria. Other risks include uncertainty of patent protection and proprietary rights, whether patent applications and issued patents will offer adequate protection to enable product development, the obtaining of necessary drug regulatory authority approvals and difficulties caused by the rapid advancements in technology. Companies such as Opthea are dependent on the success of their research and development projects and on the ability to attract funding to support these activities. Investment in research and development projects cannot be assessed on the same fundamentals as trading and manufacturing enterprises. Thus investment in companies specialising in drug development must be regarded as highly speculative. Opthea strongly recommends that professional investment advice be sought prior to such investments.

## **Forward-looking statements**

Certain statements in this ASX announcement may contain forward-looking statements regarding Company business and the therapeutic and commercial potential of its technologies and products in development. Any statement describing Company goals, expectations, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, particularly those risks or uncertainties inherent in the process of developing technology and in the process of discovering, developing and commercialising drugs that can be proven to be safe and effective for use as human therapeutics, and in the endeavour of building a business around such products and services. Opthea undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise. Actual results could differ materially from those discussed in this ASX announcement.