



ASX and Media Release  
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## Opthea Presents at the Ophthalmology Innovation Summit

**Melbourne, Australia; 11 August 2017** – Opthea Limited (ASX:OPT), a developer of novel biologic therapies for the treatment of eye diseases, announced today that Dr Megan Baldwin, the Company's Chief Executive Officer, presented at the Ophthalmology Innovation Summit (OIS) in Boston on Thursday, August 10<sup>th</sup>, 2017.

Dr Baldwin's presentation was made in the "Combination Therapy in AMD and DME" session of OIS (<http://ois.net/ois-asrs-2017/agenda/>) along with other leading companies in the field. A panel discussion immediately followed company presentations. The presentation covered data from Opthea's Phase 1/2A clinical trial of OPT-302, a novel VEGF-C/D 'Trap' therapy for wet age-related macular degeneration (wet AMD). The encouraging results from that trial suggest that combined administration of OPT-302 + Lucentis<sup>®</sup> may lead to improved clinical outcomes over Lucentis<sup>®</sup> alone.

The OIS was attended by professionals from the investor, pharmaceutical and clinical ophthalmology community and is held annually in conjunction with the Annual Scientific Meeting of the American Society for Retina Specialists (ASRS).

Dr. Baldwin commented *"The OIS was an excellent opportunity to present the data from our Phase 1/2A of OPT-302 in wet AMD patients to an international expert audience. The presentation was followed by a robust panel discussion on the potential of novel combination therapies to address the unmet medical need for wet AMD. Opthea is strongly positioned with our novel mechanism to address this unmet need and we look forward to advancing our clinical program for OPT-302 in Phase 2B and Phase 2A clinical trials in wet AMD and diabetic macular edema respectively."*

A copy of the presentation is available on Opthea's website at [www.opthea.com](http://www.opthea.com)

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### About the Phase 1/2A study of OPT-302 for Wet AMD

The Phase 1/2A trial recruited a total of 51 patients with wet AMD, who were either treatment naïve (n=25) or previously treated with prior intravitreal anti-VEGF-A therapy (n=26). Mean best corrected visual acuity (VA) was 59.4 letters at baseline. The study recruited a high proportion of heavily pre-treated patients (51%) and occult wet AMD lesions (73%) which are considered to be more difficult to treat with existing standard of care therapies.

The study had two parts: a sequential dose escalation (Phase 1) and a randomised dose expansion study (Phase 2A). The Phase 1 enrolled 20 patients into three ascending OPT-302 dose level cohorts (0.3, 1 and 2 mg) in

combination with Lucentis® (0.5 mg), and an OPT-302 monotherapy group (2 mg). In the Phase 2A dose expansion, 31 subjects were randomised in a 3:1 ratio to two treatment cohorts with OPT-302 at 2 mg, either in combination with Lucentis® (n=23) or as monotherapy (n=8). Patients received three intravitreal injections of OPT-302 either alone or in combination with Lucentis® at 4 week intervals with a follow-up visit at week 12. For patients receiving OPT-302 monotherapy, Lucentis® rescue therapy was provided at investigator discretion or if there was a  $\geq 5$  letter decrease in VA and no reduction in central subfield thickness (CST) of at least 10% with presence of fluid.

### **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 \$US3.2BN in 2016. 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 \$US5.4BN in 2016. 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 being conducted 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 recruited an additional 31 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.

### **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. Opthea's intellectual property is held within its wholly-owned subsidiary Vegenics Pty Ltd. 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).

### **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.