

# ASX/NASDAQ ANNOUNCEMENT

Benitec Biopharma makes significant progress in ocular program

- Data to be presented at industry leading conferences
- Development of novel viral vectors for delivery to the back of the eye
- Expanded potential of developing ddRNAi technology into ocular indications

**Sydney, Australia, 2 February 2017:** Benitec Biopharma Limited (ASX:BLT; NASDAQ: BNTC; NASDAQ: BNTCW) is pleased to announce today that it has made significant progress with the Company's ddRNAi technology for the development of therapeutics for the treatment of ocular diseases. Of particular importance is the output from Benitec's collaboration with 4D Molecular Therapeutics to identify novel viral vectors for delivery to the back of the eye using direct intravitreal injection, a commercially attractive route of administration.

## • Data to be presented at industry leading conferences

The results of this work will be presented by Dr David Suhy, Benitec's Chief Scientific Officer, at the Association for Research in Vision and Ophthalmology (ARVO)-Asia meeting being held in Brisbane on February 5<sup>th</sup> to February 8<sup>th</sup>, and the Translational Vision Summit (TVS), being held in conjunction. ARVO-Asia is a leading conference dedicated to eye and vision research in the Asia-Pacific region. The TVS meeting highlights "revolutionary approaches to advancing innovation in the diagnosis and treatment of eye disease".

# • Development of novel viral vectors for delivery to the back of the eye

Dr David Suhy commented, "Having a commercially attractive route of administration is a significant step forward in the program. One of the major limitations of most ocular gene therapy applications is the use of a highly complex surgical technique called subretinal injection for delivery into the eye. We are developing viral vectors which can efficiently transduce cells within the retina following an office-friendly, intravitreal injection. This is the same route of administration used for the standard of care treatments for Age-Related Macular Degeneration (AMD), including Lucentis® and Eylea®.

# • Expanded potential of developing ddRNAi technology into ocular indications

David continued, "Being able to deliver drugs in therapeutically relevant concentrations is a key challenge in drug development. It has taken significant time and effort, but we believe that these outcomes demonstrate the commercial applicability of having a vector that can transduce the retina following an intravitreal injection. The AMD program is our first program in this space and we anticipate being able to build a ddRNAi franchise for other ocular indications, in particular retinal diseases, using these novel viral vectors as a key component in that platform."

Benitec's ddRNAi technology is a unique combination of gene silencing using RNA interference coupled with the long term therapeutic activity of gene therapy vectors. The lead ocular candidate, BB-201, is designed to treat patients with the wet form of AMD and will be featured in the ARVO-Asia presentations.



As a gene therapy approach, BB-201 has been designed for long term expression of the therapeutic short hairpin RNA from a single injection. BB-201 is comprised of a novel adeno associated virus capsid (AAV) and a recombinant DNA cassette engineered to express steady state levels of three short hairpin RNA (shRNA) that inhibit VEGF-a, VEGF-b and PIGF, three clinically validated targets whose expression is shown to lead to the progression of the disease.

The full presentation will be posted on the Benitec website.

For further information regarding Benitec, please contact the persons below, or visit the Benitec website at www.benitec.com

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### About Age-Related Macular Degeneration (AMD):

AMD is a disease that has been estimated to account for 8% of blindness worldwide and has been projected to impact up to 196 million patients by 2020 and up to 288 million by 2040. The wet form of the disease accounts for about 10% of all AMD patients but accounts for up to 90% of all the blindness. The wet form of the disease is characterised by the growth of new blood vessels into the eye, a phenomenon that has been associated with the abnormally high expression of abnormally high levels of proteins from the vascular endothelial growth factor (VEGF) family. The most commonly used standard of care treatments for AMD require an intravitreal injection into the eye as frequently as monthly or bi-monthly. Such injections may be required indefinitely to be able to halt progression of the disease and stabilise vision.

### About Benitec Biopharma Limited:

Benitec Biopharma Limited (ASX: BLT; NASDAQ: BNTC; NASDAQ: BNTCW) is a biotechnology company developing innovative therapeutics based on its patented gene-silencing technology called ddRNAi or 'expressed RNAi'. Based in Sydney, Australia with laboratories in Hayward, California (USA), and collaborators and licensees around the world, the company is developing ddRNAi-based therapeutics for chronic and life-threatening human conditions including hepatitis B, wet age-related macular degeneration and OPMD. Benitec has also licensed ddRNAi to other biopharmaceutical companies for applications including HIV/AIDS, Huntington's Disease, chronic neuropathic pain, cancer immunotherapy and retinitis pigmentosa.

### Safe Harbor Statement:

This press release contains "forward-looking statements" within the meaning of section 27A of the US Securities Act of 1933 and section 21E of the US Securities Exchange Act of 1934. Any forward-looking statements that may be in the press release are subject to risks and uncertainties relating to the difficulties in Benitec's plans to develop and commercialise its product candidates, the timing of the initiation and completion of preclinical and clinical trials, the timing of patient enrolment and dosing in clinical trials, the timing of expected regulatory filings, the clinical utility and potential attributes and benefits of ddRNAi and Benitec's product candidates, potential future out-licenses and collaborations, the intellectual property position and the ability to procure additional sources of financing. Accordingly, you should not rely on those forward-looking statements as a prediction of actual future results.