

## **ASX/NASDAQ ANNOUNCEMENT**

# Benitec Biopharma reports financial results for the 2018 fiscal first quarter and provides operational update

**Sydney, Australia, 29 November 2017:** Benitec Biopharma Limited (ASX:BLT; NASDAQ: BNTC; NASDAQ: BNTCW) ("Benitec" or "the Company"), a biotechnology company developing innovative therapeutics based on a combination of gene therapy with its patented gene-silencing technology called ddRNAi or 'expressed RNAi', today reported its consolidated financial results for the 2018 fiscal first quarter (1Q FY18), and highlighted recent progress in advancing its pipeline.

## 1Q FY18 Highlights

- Advancements of pipeline programs included:
  - Oculopharyngeal muscular dystrophy (OPMD); submission of application to U.S. Food & Drug Administration (FDA) seeking orphan drug designation for BB-301;
  - OPMD; positive pre-IND and scientific advice meetings with the U.S. FDA, Health Canada and several European agencies;
  - Head and neck squamous cell carcinoma (HNSCC); selection of partner contract research organisations to support the upcoming Phase 2 study.
- Cash on hand of A\$14.7 million at 30 September 2017
- Loss for the three months to 30 September 2017 was A\$2.9 million as compared to a profit of A\$0.8 million in the previous corresponding period

Commenting on recent company performance, Chief Executive Officer Greg West said: "I am very pleased with the excellent progress we have made in advancing our pipeline programs towards key value inflection milestones. The recent positive regulatory interactions for our OPMD program and the continued advancement of BB-401 towards the clinic as planned in the first calendar quarter of 2018, are just a few of the many milestones we expect to reach over the coming months as we execute on our goal of becoming a multi-product, clinical-stage company by the end of calendar year 2018."

#### 1Q FY18 Financial Results

Benitec reported a net loss of A\$2.9m for the September 2017 quarter (1Q FY18) compared to a profit of A\$0.8m in the September 2016 quarter (1Q FY17). The principal reason for the increase in net loss of A\$3.6m is that in the September 2016 quarter, federal government grants of A\$4.9m were recognised. These related to the 12-month period ended 30 June 2016. Since March 2017, the Company recognises R&D Grant revenue on a quarterly accrual basis, in accordance with revised accounting standards.



At the end of the September 2017 quarter, Benitec had cash on hand of A\$14.7m, a decrease of \$2.7m from the June 2017 quarter. This represents operating cash outflow of \$2.8m offset by income of \$0.2m.

### 1Q FY18 Operational Update

## BB-301: Orphan Disease (OPMD) Program

- IND filing planned for 4<sup>th</sup> quarter of calendar year 2018. Assuming approval on a normal time-frame, Benitec should be in an initial human clinical study by the end of calendar year 2018.
- Benitec continues advancement of an innovative single vector system with the capability to both 'silence and replace' disease causing genes. In addition to using RNA interference to 'silence' the mutant PABPN1 gene expression that causes the OPMD, BB-301 simultaneously introduces a normal copy of the same gene thus providing the potential to restore normal function to the treated tissues and in the process, improve treatment outcomes.
- A single gene therapy product, versus an equivalent system with two or more vectors, vastly simplifies the manufacturing and regulatory processes and reduces the complexity of the clinical strategy for BB-301.
- Benitec considers the 'silence and replace' modality a significant advancement not only for the OPMD program, but also in the potential treatment of other orphan diseases.
- Benitec submitted an application with the U.S. Food & Drug Administration (FDA) seeking orphan drug designation for BB-301 as a treatment of OPMD.
- Benitec completed successful pre-IND and scientific advice meetings with the U.S. FDA, Health Canada and several European agencies. Input from these meetings has been incorporated into the BB-301 regulatory strategies.
- Benitec and Royal Holloway University of London presented an oral presentation on 'OPMD Gene Therapy' at the 22<sup>nd</sup> International Congress of the World Muscle Society, which was held 3-7 October 2017 in Saint Malo, France.
- Benitec gave an oral presentation on the data from its OPMD program at the European Society of Gene & Cell Therapy (ESGCT) 25<sup>th</sup> Annual Meeting, that was held 17-20 October 2017 in Berlin, Germany.
- Benitec presented at the OPMD Patient and Family Conference that was held 11 November 2017 in Albuquerque, New Mexico.



#### BB-401/BB-501: Oncology (HNSCC) Program

- A Phase 2 human clinical trial is planned for first quarter calendar year 2018.
- Benitec is on track with its start-up activities to support the Phase 2 study for BB-401, a DNA construct that expresses an antisense RNA directed against the epidermal growth factor receptor (EGFR), for the treatment of patients with HNSCC.
- Pre-clinical testing in mouse xenograft models is ongoing for BB-501, the follow-on anti-EGFR based ddRNAi construct, to treat head and neck squamous cell carcinoma.
- As EGFR is a key factor in many epithelial malignancies and its activity enhances tumour growth, invasion, and metastasis, Benitec intends to explore other potential clinical indications for BB-401 and BB-501, including rare cancers.

### BB-201: Retinal disease (AMD) Program

- Benitec continues development of its program to treat retinal diseases with an initial program
  designed to treat subjects impacted by the wet form of age-related macular degeneration
  (AMD), a disease caused by the growth of new blood vessels into ocular tissues. Designated
  as BB-201 and intended as a single administration, this construct expresses three independent
  shRNA designed to silence VEGF-a, VEGF-b and PIGF, three well validated targets known to
  contribute to the disease.
- Benitec is using several novel AAV capsids with enhanced bio-distribution and transduction of retina cell layers following the commercially friendly route of intravitreal injection, a technique currently used to deliver the standard of care drugs most commonly used to treat wet AMD.
- Benitec has recently completed the in-life portion of an in vivo proof of concept study in a non-human primate model in which new blood vessel formation is induced by the laser treatment of the retina. Although the molecular analyses of all the retinal tissues have not been completed, it is clear from the initial in-life portion of the data, that additional work on BB-201 will be required if we are to continue the development of the AMD program.

## **Upcoming Presentations**

• Benitec will present at the 9th Annual Biotech Showcase™ during the J.P. Morgan Healthcare Conference, which is being held 8-10 January 2018 in San Francisco, CA.

#### **Conference Call Information**

Benitec management will provide an operational update to discuss the 1Q FY18 results and expectations for the future, via conference call on Thursday, 30 November 2017 at 9:00am AEDT / Wednesday, 29 November at 5:00pm EST. To access the call, please dial 1 800-908-299 (Australia) or 1 855-624-0077 (U.S.) five minutes prior to the start time and refer to conference ID 714460. An archive of the webcast will remain available on Benitec's website for 90 days beginning at approximately 11:30am AEDT on 30 November 2017.



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

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#### **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 lifethreatening human conditions including OPMD, head & neck squamous cell carcinoma, retinal based diseases such as wet age-related macular degeneration, and hepatitis B. 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 this ASX/Nasdaq announcement 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.