

ASX ANNOUNCEMENT

Conference call transcript for the March 2017 Quarterly Report

Sydney Australia, 31 May 2017: Benitec Biopharma Limited (ASX: BLT; NASDAQ: BNTC; NASDAQ: BNTCW) today lodged its transcript for the March 2017 Quarterly Report conference call taking place at 8.00am AEDT on 31 May, 2017. The conference call briefing was provided by Greg West CEO and Dr David Suhy CSO.

Good morning everyone.

We have on this call our executive team, Bryan Dulhunty, Cliff Holloway, David Suhy, and Georgina Kilfoil. David Suhy and I will give the briefing which will take about 15 minutes and we will then take questions.

As a reminder, these quarterly calls are in line with our commitment to provide reporting following our US listing on the NASDAQ.

Today I will reflect on some recent key achievements across our programs and talk about how we are positioned to re-enter the clinic. By way of background, we describe our pipeline as being in four therapeutic areas - oncology, ocular diseases, orphan diseases and infectious disease.

We continue to see good progress in our pipeline programs as we complete on schedule our preclinical work and we are preparing to take two of our programs, in oncology and orphan diseases into the clinic in calendar 2018 with the other programs being made clinic ready in 2018.

I will now provide a brief update on each program and then hand over to David Suhy, our Chief Scientific Officer for his comments.

Oncology

In January this year we announced we had initiated work on two new oncology pipeline programs after executing a Research Collaboration Agreement with Nant. Nant is a well-respected, strategic investor who have a high regard for Benitec's scientific platform and expertise.

The transaction with Nant demonstrates we are delivering on our previously communicated strategy of building relationships with long term partners. Benitec can now access capital markets, with Nant as a major investor, to progress the development of these oncology programs and Benitec's other programs.

BB-401, our clinical stage antisense EGFR asset, has performed well in previous early stage trials and we look forward to continuing its clinical development. EGFR is a validated therapeutic target. Benitec has assembled a team of experts to plan the rapid progression of BB-401 into the next stages of development and targets starting a mid-stage clinical study early in 2018.

In parallel, the scientific team at Benitec has initiated work on a follow-on therapeutic, BB-501, using its proprietary ddRNAi platform. The data obtained from the BB-401 program will be used to inform the development pathway of BB-501.

Orphan diseases

In April this year we announced that, with our unique approach to gene silencing and gene therapy, we are able to knock out the mutated form of a gene and have the ability to express a normal copy to restore function. These initial preclinical efficacy results in the oculopharyngeal muscular dystrophy (OPMD) program using this 'silence and replace' technology were published in Nature Communications in April this year.

These data provided the basis for obtaining European Union Orphan Drug Designation earlier this year and may provide a streamlined process towards regulatory approval. EU Orphan Drug Designation provides regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons. In addition, the Orphan Drug Designation provides protection from competition once the medicine is placed on the market.

Currently, the only treatment that is available for OPMD is a surgical intervention that does not provide long term benefit for the patients. We believe that our innovative 'silence and replace' approach may offer new treatment options for patients. Entry into the clinic with a Phase I/II study in OPMD patients is anticipated in the second half of 2018, subject to toxicity results and future regulatory review.

Ocular diseases

We have announced promising results relating to the development of novel capsids for delivery into the retina following an intravitreal injection.

The development of these capsids is complex science and we are particularly pleased about the outcomes. Our focus now is to demonstrate that we can load these capsids with ddRNAi constructs and induce a therapeutic effect, initially targeting Aged Related Macular Degeneration (AMD). Once this has been achieved, we believe this is an opportunity for Benitec to build an ocular franchise.

We are in the process of preparing this program to be clinic ready late in 2018.

Infectious disease

In February, we presented pivotal *in vivo* data from BB-103, our lead therapeutic to treat subjects infected with the hepatitis B virus (HBV). These data demonstrate that a single administration of the Benitec ddRNAi agent, used in combination with current standard of care agents used to treat HBV, demonstrates a greater than 4 log reduction and sustained suppression of the disease in an *in vivo* model.

We believe our data using a one-time administration of a ddRNAi therapeutic offers a compelling case for the inclusion into a current standard of care treatment regimen.

We are now in the process of preparing this program to be clinic ready in the second half of 2018.

We are delivering on what we said we would, and our strategic focus remains unchanged:

- Firstly, to continue the scientific development on our existing pipeline programs through to commercialisation.
- Secondly, to prioritise the future development of our ddRNAi technology by identifying those opportunities with a high probability of commercial success and value to shareholders.
- Thirdly, to establish co-development agreements with other companies using our scientific capability and IP platform.

We are using the strong preclinical results we have achieved across our programs and the future expansion possibilities to engage in discussions with both the investment community and potential partners.

We will continue to explore ways to leverage off our scientific capabilities and current pipeline programs such as AMD and OPMD with expansion possibilities into other ocular indications and other orphan diseases.

I will now ask David Suhy to provide a more detailed update on selected pipeline programs and scientific matters.

Thank you, Greg,

I'd like to add a bit more colour and emphasis on the results that we have achieved in our pipeline programs since the last quarterly update.

With **HBV**, our comprehensive results from the chimeric mouse model have generated significant and important data that provide important insights into the design of the clinical study for BB-103, our lead candidate. As stated in the last quarterly call, Benitec's goal is to combine BB-103 with a NUC inhibitor in order to suppress the virus and allow the patient's own immune system to produce anti-s-antigen antibodies, a process that may lead to a functional cure in HBV patients.

More importantly, we have recently completed a pre-IND submission with the US Food and Drug Administration (FDA) in which the feedback provided from the agency has defined a clear and expeditious path towards the clinic. We have initiated the remaining IND-enabling studies and have been working closely with our Key Opinion Leaders and clinicians to finalise the design of the protocol for the BB-103 human study.

Next we turn to our program for the treatment of **oculopharyngeal muscular dystrophy, or OPMD**. As Greg mentioned, our initial *in vivo* efficacy data using a dual AAV vector system were published in Nature Communications early last month. These results, which come from an ongoing collaboration with George Dickson at the Royal Holloway University of London and Capucine Trollet at the Institut de Myologie, demonstrate that a 'silence and replace' approach that targets the mutant PABPN1 gene can correct many of the key clinical features of OPMD.

These data were generated in A17 mice, the only well validated animal model of the disease, and a system that mimics most of the features of human OPMD patients. These results highlight one of the unique aspects of the Benitec technology that is not readily attainable by other gene therapy approaches. Specifically, we are able to silence a mutant gene and have the ability to express a normal copy to restore function.

Over the past year, we have generated a single AAV vector approach which encompasses both the silence and replace modalities. Termed BB-301, this single vector system has been involved in extensive efficacy testing in the A17 model and we look forward to releasing that data in the near future.

For our **ocular program**, our collaborative efforts with 4D Molecular Therapeutics have focused on the identification and characterisation of novel AAV capsids with markedly enhanced transduction of ocular tissues following intravitreal administration. We have now completed biodistribution studies in non-human primates with five different novel AAV capsids. A portion of these data was presented at The Association for Research in Vision and Ophthalmology (ARVO) conferences in Brisbane and Baltimore and just last week at the Australasian Gene and Cell Therapy Society (AGCTS) meeting in Sydney. An extensive data analysis on one of the capsids was also presented at the Baltimore meeting by our collaborators.

We are taking the most promising capsids into efficacy studies in a Wet AMD model. Our clinical candidates contain recombinant ddRNAi expression vectors that express short hairpin RNAs (shRNAs) that target and silence the clinically well validated genes that are the causative agents in wet AMD including VEGF-a, VEGF-b and PlGF. The laser induced model of neovascularization in non-human primates that will be used in these studies provides one of the most well validated models to test the efficacy of our vectors.

Being able to deliver therapeutically relevant concentrations of drugs into the appropriate diseased tissues is a key challenge. Thus, positive results from these experiments not only validate the AMD program but will also assess the utility of these capsids for transducing retinal tissues and provides a platform to expand our ddRNAi therapeutics into a broad range of other retinal-based ocular indications.

Lastly, we continue to make significant advances with our two **oncology programs**. With BB-401, the antisense-EGFR program, we have been working in refining the proposed clinical protocol with our Key Opinion Leaders. We have also begun the process to manufacture clinical grade materials and remain on track to initiate the Phase 2 clinical study by the first quarter of 2018.

The second program, BB-501, is our next generation ddRNAi therapeutic which also targets EGFR. We have completed the selection and optimisation of the shRNAs and have already moved into mouse xenograft models to test for *in vivo* efficacy. In addition to expressing anti-EGFR shRNA, BB-501 may also incorporate other therapeutic entities in the same vector.

Back to you Greg

Thank you, David,

Over the past year, we have communicated what we are going to do and we have demonstrated that we can deliver on our promises.

We are now looking at most of our programs being in or near the clinic in 2018 backed by strong preclinical results.

- In early 2018 we will be in the clinic in our oncology program.
- In our orphan program we expect to be in the clinic in the second half of 2018 with our unique 'silence and replace' technology.



- In our ocular program, we have a vector which has demonstrated pan retinal distribution. This is a major achievement. We anticipate being clinic ready late in 2018.
- In our HBV program, based on our strong *in vivo* data and on our positive interaction with the FDA, we have a clear path towards the clinic in the second half of 2018.

Each of these achievements are significant and demonstrates strong scientific and business momentum across our pipeline.

At this time, I will ask the operator for questions.

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Thank you to all our participants today, and especially those shareholders and analysts who provided questions. I would like to thank all our investors for their continued support.

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For further information regarding Benitec and its activities, please contact the persons below, or visit the Benitec website at [www.benitec.com](http://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 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 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 commercialize 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.