

July 8, 2018



Benitec Announces Global Licensing Agreement for BB-301 for Treatment of Oculopharyngeal Muscular Dystrophy and Broad Platform Collaboration with Axovant

- Benitec to receive upfront cash payment of US\$10 million with additional cash payments totalling US\$17.5 million (a total of US\$27.5M) upon completion of near-term milestones for BB-301, now named AXO-AAV-OPMD**
- Benitec is potentially eligible for US\$187.5 million in total payments upon the achievement of development, regulatory and commercial milestones on AXO-AAV-OPMD**
- Benitec will retain 30% of the net profits on the worldwide sales of AXO-AAV-OPMD**
- Benitec and Axovant to partner on the development of five additional gene therapy programs; Benitec to receive full research funding and be eligible for US\$93.5 million in development, regulatory and commercial milestones for each program**

SYDNEY, July 8, 2018 /PRNewswire/ -- Benitec Biopharma Limited ("Benitec" or the "Company") (ASX:BLT; NASDAQ: BNTC; NASDAQ: BNTCW) today announced that it has licensed to Axovant Sciences ("Axovant") the exclusive global rights for BB-301 (now named AXO-AAV-OPMD) intended for the treatment of oculopharyngeal muscular dystrophy (OPMD), and has also entered into a fully funded research collaboration for the development of five additional gene therapy products in neurological disorders.

Under the terms of the agreement, Benitec will receive an upfront cash payment of US\$10 million and additional cash payments totaling US\$17.5 million upon completion of four specific near-term manufacturing, regulatory and clinical milestones. Axovant has been granted worldwide rights to AXO-AAV-OPMD and will assume all future development costs. The total potential value of all of the development, regulatory and commercial milestones achievable by Benitec, of which there are eight milestones including the four near-term milestones, is US\$187.5 million. Benitec, working in partnership with Axovant over the next few years, hopes to achieve all eight milestones and thus realize the maximum amount of US\$187.5 million. There can be no assurance as to the total amount of payments that the

Company will actually receive or when they will be received. Importantly, upon commercialization, Benitec will retain 30% of the net profits on worldwide sales of AXO-AAV-OPMD.

Jerel Banks, MD PhD, Executive Chairman, Benitec Biopharma commented on today's news, "Today marks a milestone for Benitec as we believe this transaction to be transformative for our company. In addition to bolstering our opportunity to drive broad-based, clinically meaningful patient benefit across several areas of clinical medicine with true unmet need, this partnership significantly enhances the financial, intellectual, and clinical development resources available to facilitate our efforts to build Benitec into a diversified biopharmaceutical company. The non-dilutive capital expected over the near term will allow Benitec to continue to invest in proprietary R&D programs across a range of indications."

Dr. Banks continued, "Our management team is focused exclusively on expanding the research, development, and commercial opportunities for the core 'silence-and-replace' platform with the dual goals of enhancing patient benefit and generating shareholder value. We believe Axovant is the ideal partner to advance our OPMD program, and we look forward to working closely with them to develop AXO-AAV-OPMD as we quickly progress towards clinical trials in 2019."

OPMD is a rare progressive, and often fatal, muscle-wasting disease caused by mutation in the poly(A)-binding protein nuclear 1 (*PABPN1*) gene, that is characterized by eyelid drooping, swallowing difficulties, and proximal limb weakness. AXO-AAV-OPMD is a single vector, gene therapy construct system that uses a unique "Silence-and-Replace" methodology that employs DNA directed RNA interference (ddRNAi) to silence expression of the mutant gene associated with OPMD, while simultaneously expressing a copy of the normal, healthy version of the same gene to restore the function of that gene. Axovant plans to initiate a placebo-controlled clinical study in 2019 in which a one-time intramuscular administration AXO-AAV-OPMD will be given to patients to treat the dysphagia associated with OPMD.

Commenting on the agreement, Pavan Cheruvu, MD, Chief Executive Officer of Axovant said, "The 'silence-and-replace' platform is a targeted approach which directly addresses the underlying genetic cause of diseases arising from expression of dysfunctional proteins, including those caused by nucleotide repeat expansion. I am excited about the potential of this platform for patients suffering from OPMD, many of whom have limited treatment options today."

In addition to AXO-AAV-OPMD, Axovant and Benitec will collaborate on a total of five additional investigational gene therapy products for neurological disorders, with Axovant fully funding each of the research programs. Axovant will have exclusive global rights to products developed under these programs. The first additional investigational gene therapy product will focus on developing a single vector "Silence-and-Replace" gene therapy product targeting the *c9orf72* gene, which is associated with amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). In addition to receiving funding for development of the new research programs, each new research program target is eligible for development, regulatory and commercial milestones totaling US\$93.5 million and tiered royalties on global sales. There can be no assurance as to the total amount of payments that the Company will actually receive or when they will be received.

Dr. Banks concluded, "We are extremely excited about Axovant's collaborative and financial commitments to these five additional research programs as it plants the seeds for a long and robust partnership between our organizations. This partnership provides Benitec with an extraordinarily rare opportunity to unambiguously demonstrate the exceptional breadth of the scientific, clinical, and commercial applications of the 'silence-and-replace' platform. Additionally, the non-dilutive capital expected by Benitec over the near term will be used to fund operations as we will continue to innovate and strengthen our platform. I look forward to making future announcements on our joint progress with Axovant as well as on other material developments."

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 life-threatening human conditions including OPMD, head & neck squamous cell carcinoma, retinal based diseases such as wet age-related macular degeneration, and hepatitis B.


About OPMD:

OPMD is a rare inherited myopathy characterized by dysphagia (difficulty in swallowing), the loss of muscle strength, and weakness in multiple parts of the body. Patients typically suffer from severe dysphagia, ptosis (eye lid drooping), tongue atrophy, proximal lower limb weakness, dysphonia (altered and weak voice), limitation in looking upward, as well as facial muscle and proximal upper limb weakness. Progressing throughout that patient's life, OPMD is not typically diagnosed until the individuals reach their late 40s. As the dysphagia becomes more severe, patients become malnourished, lose significant weight, become dehydrated and suffer from repeated incidents of aspiration pneumonia. The last two symptoms are often the cause of death. No cure is currently available for OPMD. The cricopharyngeal myotomy is the only treatment available to improve swallowing in these patients, but because the root cause of the genetic disease has not been addressed, the pharyngeal musculature still undergoes progressive degradation leading to the previously mentioned complications.

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.

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