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Benitec Biopharma Provides Update on BB-301 Oculopharyngeal Muscular Dystrophy (OPMD) Program

MELBOURNE, Australia, Sept. 16, 2019 /PRNewswire/ --Benitec Biopharma (ASX: BLT, NASDAQ: BNTC), a gene therapy-focused biotechnology company developing novel genetic medicines derived from the proprietary DNA-directed RNA interference ("ddRNAi") platform, today announced their plans to complete three non-clinical studies that will facilitate the filing of an Investigational New Drug (IND) application and the formal initiation of a Phase I clinical trial in patients suffering from Oculopharyngeal Muscular Dystrophy (OPMD).

BB-301 is an internally optimized, AAV-based gene therapy agent that can both silence the expression of mutated, disease-causing genes (to slow, or halt, the underlying mechanism of disease progression) and replace the mutant genes with normal, "wild type" genes (to drive restoration of function in diseased cells). This fundamental approach to disease management is called "silence and replace" and this biological mechanism offers the potential to restore the underlying physiology of the treated tissues and, in the process, improve treatment outcomes for patients suffering from the chronic and, potentially, fatal effects of OPMD.

The three non-clinical BB-301 studies will support the optimization of the methods of administration, confirm the efficiency of vector transduction in the key tissue compartments underlying the disease phenotype, confirm the optimal drug doses in advance of initiation of human clinical studies, and finalize experiments designed to characterize any toxicological data-points that would underlie future regulatory filings and clinical study designs. The non-clinical studies will be conducted in canine subjects.

The non-clinical studies will be carried out under the guidance of the scientific team at Benitec in close collaboration with a team of Thought Leaders in both medicine and surgery that have been deeply engaged in the treatment of OPMD patients for several decades. The execution of the three non-clinical studies will also be supported by the establishment of long-term engagements with both regulatory and toxicological consultants with demonstrable expertise in the field of gene therapy.

Jerel A. Banks, M.D. Ph.D., Executive Chairman and Chief Executive Officer of Benitec Biopharma commented on today's update, "Through our continued focus on the optimization of the non-clinical and clinical attributes of BB-301 for the treatment of Oculopharyngeal Muscular Dystrophy, our team has an unprecedented opportunity to develop a novel genetic medicine that could facilitate clinically meaningful patient benefit in a potentially fatal disorder for which profound unmet medical need exists. Additionally, the robust financial position of the Company, in addition to the streamlined operational profile, will facilitate efficient advancement towards our goal of completing the initial clinical evaluation of BB-301."

About Benitec Biopharma Limited

Benitec Biopharma Limited (ASX: BLT; NASDAQ: BNTC; NASDAQ: BNTCW) is a clinical-stage biotechnology company focused on the development of novel genetic medicines. The proprietary platform, called DNA-directed RNA interference, or ddRNAi, combines RNA interference, or RNAi, with gene therapy to create medicines that facilitate sustained silencing of disease-causing genes following a single administration. Based in Melbourne, 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 oculopharyngeal muscular dystrophy (OPMD), and chronic hepatitis B.

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 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.

Investor Relations
M Group Strategic Communications
Jay Morakis
Managing Director
Tel: +1 646 859 5951
Email: jmorakis@MGroupSC.com

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