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Benitec Biopharma Provides Update on BB-301 Tissue Transduction Study

HAYWARD, Calif., July 8, 2020 /PRNewswire/ --Benitec Biopharma, Inc. (NASDAQ: BNTC), a development-stage, gene therapy-focused, biotechnology company developing novel genetic medicines based on the proprietary DNA-directed RNA interference ("ddRNAi") platform, today announced the initiation of the BB-301 Tissue Transduction Study in large animal subjects.

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 Oculopharyngeal Muscular Dystrophy (OPMD). BB-301 has been granted Orphan Drug Designation in the United States and the European Union.

The BB-301 Tissue Transduction Study is the first of three planned IND-enabling studies that will be conducted in large animals. These IND-enabling studies will be carried out under the guidance of the scientific team at Benitec, with key elements of the study design and execution conducted in close collaboration with a team of leading experts in both medicine and surgery that have been deeply engaged in the treatment of OPMD patients for several decades. The BB-301 Tissue Transduction study, along with the subsequent non-clinical studies, will be conducted in canine subjects and will support the validation and optimization of the newly designed method of BB-301 administration, confirm the efficiency of vector transduction in the key tissue compartments underlying the natural history of OPMD, confirm the optimal drug doses in advance of initiation of human clinical studies, and facilitate observation of key toxicological data-points.

The BB-301 Tissue Transduction Study is designed as an 8-week study in Beagle dogs to confirm the transduction efficiency of BB-301 upon administration via direct intramuscular injection into specific anatomical regions of the pharynx through the use of an open surgical procedure. This new route of BB-301 administration was developed in collaboration with key surgical experts in the field of Otolaryngology, and this novel method of BB-301 dosing will significantly enhance the ability of a treating physician to accurately administer the AAV-based investigational agent to the muscles that underlie the characteristic deficits associated with the progression of OPMD. It is important to note that prior non-clinical studies of BB-301 have reproducibly validated the robust biological efficacy achieved following direct intramuscular injection. As an example, direct injection of BB-301 into the tibialis anterior muscles of A17 mice facilitated robust transduction of the targeted skeletal muscle cells and supported complete remission of the OPMD disease phenotype in this animal model.

Interim data for the BB-301 Tissue Transduction Study is expected in late 2020 or early 2021.

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 validation and optimization of the non-clinical and potential clinical attributes of BB-301 for the treatment of OPMD, our team has an unprecedented opportunity to develop a novel genetic medicine that could facilitate clinically meaningful patient benefit in a fatal disorder for which profound unmet medical need exists."

About Benitec Biopharma, Inc.

Benitec Biopharma, Inc. ("Benitec" or the "Company") is a development-stage biotechnology company focused on the advancement of novel genetic medicines with headquarters in Hayward, California. 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. The Company is developing ddRNAi-based therapeutics for chronic and life-threatening human conditions including Oculopharyngeal Muscular Dystrophy (OPMD), and Chronic Hepatitis B. A comprehensive overview of the Company can be found on Benitec's website at www.benitec.com.

Forward Looking Statement

Except for the historical information set forth herein, the matters set forth in this press release represent forward-looking statements, including statements regarding 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, and other forward-looking statements.

These forward-looking statements are based on the Company's current expectations and subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to: unanticipated delays; further research and development and the results of clinical trials possibly being unsuccessful or insufficient to meet applicable regulatory standards or warrant continued development; the ability to enroll sufficient numbers of subjects in clinical trials; determinations made by the FDA; the Company's dependence on its relationships with its collaboration partners; the efficacy or safety of the Company's products and the products of the Company's collaboration partners; the acceptance of the Company's products and the products of the Company's collaboration partners in the marketplace; market competition; sales, marketing, manufacturing and distribution requirements; greater than expected expenses; expenses relating to litigation or strategic activities; and other risks detailed from time to time in the Company's reports filed with the Securities and Exchange Commission. The Company disclaims any intent or obligation to update these forward-looking statements.

Media & Investor Relations Contact:
Jay A. Morakis

CEO of M Group Strategic Communications (for Benitec Biopharma, Inc.)
Phone: 646-859-5951
Email: jmorakis@mgroupsc.com

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