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Benitec Biopharma Receives FDA Clearance of the IND for BB-301 for the Treatment of Oculopharyngeal Muscular Dystrophy

Dosing of the first subject with BB-301 is expected in the second half of 2023, following the rollover of subjects currently enrolled in the ongoing Natural History (NH) study

HAYWARD, Calif., June 26, 2023 (GLOBE NEWSWIRE) -- Benitec Biopharma Inc. (NASDAQ: BNTC) ("Benitec" or "Company"), a clinical-stage, gene therapy-focused, biotechnology company developing novel genetic medicines based on its proprietary DNA-directed RNA interference ("ddRNAi") platform, today announced the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for BB-301, its silence and replace gene therapy for the treatment of Oculopharyngeal Muscular Dystrophy-related Dysphagia.

Oculopharyngeal Muscular Dystrophy (OPMD) is a chronic, life-threatening genetic disorder affecting approximately 15,000 patients in the United States, Canada, Western Europe, and Israel. OPMD patients lose the ability to swallow liquids and solids, resulting in chronic malnutrition, aspiration, and fatal episodes of aspiration pneumonia. Currently, there are no approved therapeutic agents for the treatment of OPMD.

"The FDA's clearance of our IND for BB-301 is a significant milestone for OPMD patients and for Benitec as a Company," said Jerel A. Banks, M.D., Ph.D., Executive Chairman and Chief Executive Officer of Benitec. "The clearance of BB-301 for clinical use represents the first potential treatment for these frequently debilitating and possibly fatal symptoms of OPMD."

Subjects from Benitec's ongoing NH study will be eligible for rollover onto the Phase 1b/2a clinical study of BB-301 for the treatment of OPMD-related Dysphagia after 6 months of baseline data collection. Following a 1-day dosing procedure for BB-301, each study subject will be evaluated for the same radiographic and clinical outcome measures as were evaluated during the NH study, including quantitative radiographic swallowing studies to facilitate objective assessments of swallowing safety, swallowing efficiency, and functional performance of the pharyngeal muscles underlying the OPMD-related Dysphagia. Currently, there are 13 subjects enrolled into the NH study, with each subject having the potential to rollover onto the Phase 1b/2a clinical dosing study for BB-301. Interim safety and efficacy data is expected to become available from the BB-301 Phase 1b/2a study approximately every 90 days following the dosing of each subject.

About BB-301

BB-301 is a novel, modified AAV9 capsid expressing a unique, single bifunctional construct promoting co-expression of both codon-optimized Poly-A Binding Protein Nuclear-1 (PABPN1) and two small inhibitory RNAs (siRNAs) against mutant PABPN1. The two siRNAs are modeled into microRNA backbones to silence expression of faulty mutant PABPN1, while allowing expression of the codon-optimized PABPN1 to replace the mutant with a functional version of the protein. We believe BB-301's silence and replace strategy is uniquely positioned for the treatment of OPMD by halting mutant expression while providing a functional replacement protein.

About Benitec Biopharma Inc.

Benitec Biopharma Inc. ("Benitec" or the "Company") is a clinical-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). A comprehensive overview of the Company can be found on Benitec's website at www.benitec.com.

Forward Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release include forward-looking statements, including statements regarding Benitec's plans to develop and commercialize its product candidates, the timing of the initiation and completion of pre-clinical and clinical trials, the timing of patient enrollment 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 and other governmental authorities; the Company's ability to protect and enforce its patents and other intellectual property rights; the Company's dependence on its relationships with its collaboration partners and other third parties; 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; the Company's ability to satisfy its capital needs through increasing its revenue and obtaining additional financing; given market conditions and other factors, including our capital structure; our ability to continue as a going concern; the length of time over which the Company expects its cash and cash equivalents to be sufficient to execute on its business

plan; the impact of COVID-19, the disease caused by the SARS-CoV-2 virus, which may adversely impact the Company's business and pre-clinical and future clinical trials; the impact of local, regional, and national and international economic conditions and events; 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.

Investor Relations Contact:

William Windham
VP, Solebury Strategic Communications
Phone: 646-378-2946
Email: wwindham@soleburystrat.com

Source: Benitec Biopharma Inc.