

February 14, 2025



# **Benitec Biopharma Announces Acceptance of Late- Breaking Oral Abstract for the BB-301 Phase 1b/2a Clinical Study at the Muscular Dystrophy Association Clinical and Scientific Conference**

**-Interim clinical study updates for the first three Subjects treated with BB-301 in the Phase 1b/2a Clinical Treatment Study to be presented as a late-breaking oral presentation at the 2025 Muscular Dystrophy Association Clinical & Scientific Conference on March 19, 2025-**

HAYWARD, Calif., Feb. 14, 2025 (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 "Silence and Replace" DNA-directed RNA interference ("ddRNAi") platform, today announced the acceptance of a late-breaking oral abstract for the BB-301 Phase 1b/2a Clinical Treatment Study ongoing in Subjects diagnosed with Oculopharyngeal Muscular Dystrophy (OPMD) with moderate dysphagia. Interim clinical study updates for the first three Subjects will be discussed in an oral presentation at the Muscular Dystrophy Association Clinical and Scientific Conference on March 19, 2025 at 1:15 pm Central Time.

"In October of 2024 we shared interim clinical study data which demonstrated durable, clinically meaningful improvements in swallowing function for the first two Subjects safely treated with BB-301, and we are excited to share updated interim clinical study data with caregivers and families in the OPMD community during the Muscular Dystrophy Association Clinical and Scientific Conference in March," said Jerel A. Banks, M.D., Ph.D., Executive Chairman and Chief Executive Officer of Benitec. "Dysphagia is a severe, life-threatening complication of OPMD, and our goal is to develop a safe and efficacious treatment which meaningfully reduces the burden of dysphagia. Additional clinical updates for Subjects enrolled in the BB-301 Phase 1b/2a Clinical Treatment Study are planned for the fourth calendar quarter of this year."

## **Late-Breaking Oral Abstract Presentation:**

An interim study update for the Phase 1b/2a Clinical Treatment Study of BB-301 in OPMD subjects with moderate dysphagia will be presented in a late-breaking oral presentation entitled "Interim Study Update for the BB-301 Gene Therapy Phase 1b/2a First in Human

Trial in Subjects with Oculopharyngeal Muscular Dystrophy with Dysphagia” at 1:15 pm Central Time on March 19<sup>th</sup> at the 2025 Muscular Dystrophy Association Clinical & Scientific Conference in room Coronado ABCD.

### **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 causative gene for OPMD). 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 the silence and replace mechanism of BB-301 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 “Silence and Replace” DNA-directed RNA interference platform combines RNA interference, or RNAi, with gene therapy to create medicines that simultaneously facilitate sustained silencing of disease-causing genes and concomitant delivery of wildtype replacement genes following a single administration of the therapeutic construct. The Company is developing Silence and Replace-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](http://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 completion of pre-clinical and clinical trials, the timing of the availability of data from our clinical trials, the timing and sufficiency of patient enrollment and dosing in clinical trials, the timing of expected regulatory filings, and the clinical utility and potential attributes and benefits of ddRNAi and Benitec’s product candidates, 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: the success of our plans to develop and potentially commercialize our product candidates; the timing of the completion of preclinical studies and clinical trials; the timing and sufficiency of patient enrollment and dosing in any future clinical trials; the timing of the availability of data from our clinical trials; the timing and outcome of regulatory filings and approvals; the development of novel AAV vectors; our potential future out-licenses and collaborations; the plans of licensees of our technology; the clinical utility and potential attributes and benefits of ddRNAi and our product candidates, including the potential duration of treatment effects and the potential for a “one shot” cure; our intellectual property position and the duration of our patent portfolio; expenses, ongoing losses, future revenue, capital needs and needs for additional financing, and our ability to

access additional financing given market conditions and other factors, including our capital structure; the length of time over which we expect our cash and cash equivalents to be sufficient to execute on our business plan; 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 and other regulatory developments; 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 impact of, and our ability to remediate, the identified material weakness in our internal controls over financial reporting; 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.

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