October 5, 2026

Benitec Biopharma Announces Regulatory Update Following Type C Meeting with FDA for BB-301 Pivotal Study

- *The BB-301 pivotal trial remains on track to begin in mid-2027*
- *Discussions regarding an open-label, single-arm, pivotal study for BB-301 are ongoing, and based on these discussions, the FDA requested additional available data, including data from the Benitec-sponsored OPMD Natural History Study*
- *Final pivotal study design expected to be confirmed in 1Q 2027*
- *Benitec will present interim clinical results from the ongoing BB-301 Phase 1b/2a study as a late-breaking poster presentation, which will encompass 12-month follow-up data for all six Cohort 1 patients and interim follow-up data for all three Cohort 2 patients, at the European Society of Gene and Cell Therapy (ESGCT) Annual Congress in Hamburg, Germany, October 27-30, 2026*

HAYWARD, Calif., Oct. 05, 2026 (GLOBE NEWSWIRE) -- Benitec Biopharma Inc. (NASDAQ: BNTC) (“Benitec” or the “Company”), a clinical-stage biotechnology company developing disease-modifying genetic medicines based on its proprietary “Silence and Replace” DNA-directed RNA interference (ddRNAi) platform, today announced the receipt of formal feedback from the U.S. Food and Drug Administration (FDA) following the Type C meeting in 3Q 2026.

Discussions regarding an open-label, single-arm, pivotal study for BB-301 are ongoing, and based on these discussions, the FDA requested additional available data, including data from the Benitec-sponsored OPMD Natural History Study. The FDA has agreed to review the relevant information on a timeline that would allow Benitec to confirm the final pivotal study design in 1Q 2027.

Timelines for the BB-301 pivotal study remain on track. Patient enrollment is planned to begin in mid-2027.

“We greatly appreciate the collaboration from the FDA and are encouraged by the constructive nature of our discussions” said Jerel A. Banks, M.D., Ph.D., Executive Chairman and Chief Executive Officer of Benitec. “The OPMD natural history study was designed from the outset to provide a rigorous foundation for the clinical development of BB-301, and the additional available data requested by the Agency represent an important final component of this ongoing development work. We look forward to providing these data to the FDA in the coming weeks and confirming the pivotal study design for BB-301 in the first quarter of 2027. With the comprehensive body of clinical and natural history data generated to date, we remain on track to initiate the pivotal study in mid-2027 and are focused on

advancing BB-301 toward becoming the first approved disease-modifying therapy for patients living with OPMD-related dysphagia."

Benitec will present interim clinical results from the ongoing BB-301 Phase 1b/2a study as a late-breaking poster presentation, which will encompass 12-month follow-up data for all six Cohort 1 patients and interim follow-up data for all three Cohort 2 patients, at the European Society of Gene and Cell Therapy (ESGCT) Annual Congress in Hamburg, Germany, October 27-30, 2026.

About OPMD

There are currently no approved therapies for OPMD, a rare autosomal-dominant degenerative muscle disorder, that impacts nearly 15,000 patients in North America, Europe and Israel. OPMD is caused by a mutation in the poly(A)-binding protein nuclear 1 (PABPN1) gene; PABPN1 is a ubiquitous protein that controls the length of mRNA poly(A) tails, mRNA export from the nucleus and alternative poly(A) site usage. OPMD is a debilitating progressive disease that weakens the pharyngeal muscles, causing severe swallowing difficulties (dysphagia).[1] Progressive dysphagia impacts 97% of OPMD patients and is a severe, life-threatening complication of OPMD which can lead to chronic choking, malnutrition, aspiration pneumonia and death.

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. BB-301 is administered locally, in a one-time procedure, to the muscles in the throat that drive the swallowing process, an approach designed to maximize local benefit and minimize systemic exposure. We believe the silence and replace mechanism of BB-301 is uniquely positioned for the treatment of OPMD by halting mutant PABPN1 expression while simultaneously providing a functional replacement protein. BB-301 has received Orphan Drug Designation from the EMA and Orphan Drug and Fast Track Designations from the FDA and is currently being evaluated in a Phase 1b/2a, first-in-human, open-label dose escalation study to evaluate the safety and clinical activity of intramuscular doses of BB-301 administered to subjects with OPMD (NCT06185673).

About Benitec Biopharma Inc.

Benitec Biopharma Inc. (NASDAQ: BNTC) is a clinical-stage biotechnology company developing disease-modifying genetic medicines designed to improve the lives of people with life-threatening, genetically defined diseases. The company's proprietary "Silence and Replace" DNA-directed RNA interference (ddRNAi) platform combines RNA interference, or RNAi, with gene therapy to create medicines designed to facilitate sustained silencing of disease-causing genes and simultaneous delivery of replacement genes that restore normal cellular function following a single administration. The company's lead investigational candidate, BB-301, is the first and only disease-modifying genetic medicine in clinical development for the treatment of Oculopharyngeal Muscular Dystrophy (OPMD)-related

dysphagia.

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 preclinical 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 other regulatory steps, 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; 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; regulatory developments in the United States of America; 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 and market competition; reliance on third party manufacturers and suppliers, and the risks associated with manufacturing complexity, supply interruptions, and compliance with current good manufacturing practices, sales, marketing and distribution requirements for our product candidates; greater than expected expenses; including expenses relating to litigation or strategic activities; our incurrence of significant losses and the uncertainty of our ability to achieve or sustain profitability or generate any revenue; the Company's ability to satisfy its capital needs through increasing revenue and obtaining additional financing; the impact of local, regional, national and international economic conditions and events; including geopolitical instability, and risks associated with conducting business and seeking regulatory approvals in international markets; our ability to attract and retain key management, scientific, and technical personnel; cyber-security threats and vulnerabilities in our and our third parties' information technology systems; the impact of legislative and regulatory reforms affecting drug pricing and reimbursement, including

Medicare drug-price negotiation and government pricing frameworks; potential product liability claims arising from the use of our product candidates in clinical trials or following any future marketing approval; physicians, patients, third-party payers, or others in the medical community may not be receptive to our product candidates, and we may not generate any future revenue from the sale or licensing of our product candidates; and disruptions to U.S. government agency operations, including FDA staffing changes; 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.

References:

1. **https://www.mayoclinic.org/diseases-conditions/dysphagia/symptoms-causes/syc-20372028**

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Source: Benitec Biopharma Inc.