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# **Benitec Receives Orphan Drug Designation in the European Union for BB-301, a ddRNAi Therapeutic in Development for the Treatment of Oculopharyngeal Muscular Dystrophy**

SYDNEY, Jan. 17, 2017 /PRNewswire/ -- Benitec Biopharma Limited (ASX: BLT; NASDAQ: BNTC; NASDAQ: BNTCW) is pleased to announce that the European Commission, based on a favourable recommendation from the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP), has granted Orphan Drug Designation to BB-301 as an orphan medicinal product for the treatment of patients with oculopharyngeal muscular dystrophy (OPMD).

Orphan Drug Designation by the European Commission provides regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (EU), and where no satisfactory treatment is available. In addition to a 10-year period of marketing exclusivity in the EU after product approval, orphan drug designation provides incentives for companies seeking protocol assistance from the EMA during the product development phase, and direct access to the centralised authorisation procedure.

OPMD is a rare inherited myopathy characterised by dysphagia (difficulty in swallowing), the loss of muscle strength, and weakness in multiple parts of the body. Patients typically suffer from severe dysphagia, ptosis (eye lid drooping), tongue atrophy, proximal lower limb weakness, dysphonia (altered and weak voice), limitation in looking upward, as well as facial muscle and proximal upper limb weakness. Progressing throughout that patient's life, OPMD is not typically diagnosed until the individuals reach their 50's or 60's. As the dysphagia becomes more severe, patients become malnourished, lose significant weight, become dehydrated and suffer from repeated incidents of aspiration pneumonia. The latter two ailments often result in death.

Currently, therapeutic strategies employ repetitive surgical interventions that have limited efficacy.

"We are very excited that BB-301 has received Orphan Drug Designation in Europe from the EMA COMP. This is a key program in our pipeline and we are happy to see the EMA recognising the urgent and unmet medical need for a safe and effective treatment for OPMD patients. We believe that our innovative approach may offer new treatment options for patients who might not otherwise be able to receive benefit in treating their disease. Having

European Orphan Drug Designation will allow us to optimise steps to further advance BB-301 towards regulatory approval," said David Suhy, Chief Scientific Officer.

BB-301 is a ddRNAi therapeutic for the treatment of OPMD comprised of a single expression construct for the 'knockdown and replace strategy' of mutant PABPN1, the principle cellular component that leads to the diseased condition in humans. BB-301 is currently in preclinical development and Benitec plans to initiate IND-enabling studies later this year.

Entry into the clinic with a Phase I/II study in OPMD patients is anticipated in 2018, subject to toxicity results and future regulatory review.

For further information regarding Benitec and its activities, please contact the persons below, or visit the Benitec website at [www.benitec.com](http://www.benitec.com)

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***About Benitec Biopharma Limited:***

Benitec Biopharma Limited (ASX: BLT; NASDAQ: BNTC; NASDAQ: BNTCW) is a biotechnology company developing innovative therapeutics based on its patented gene-silencing technology called ddRNAi or 'expressed RNAi'. Based in Sydney, 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 hepatitis B, wet age-related macular degeneration and OPMD. Benitec has also licensed ddRNAi to other biopharmaceutical companies for applications including HIV/AIDS, Huntington's Disease, chronic neuropathic pain, cancer immunotherapy and retinitis pigmentosa.

***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 the press release are subject to risks and uncertainties relating to the difficulties in Benitec's plans to develop and commercialise 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.

To view the original version on PR Newswire, visit <http://www.prnewswire.com/news-releases/benitec-receives-orphan-drug-designation-in-the-european-union-for-bb-301-a-ddrna-therapeutic-in-development-for-the-treatment-of-oculopharyngeal-muscular-dystrophy-300391632.html>

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