November 27, 2017

Benitec Biopharma provides update on OPMD orphan disease program

- Orphan application submitted with the U.S. Food & Drug Administration

- Pre-IND and scientific advice meetings completed with regulatory agencies

- On track to advance into human clinical trials by the end of calendar year 2018

SYDNEY, Nov. 27, 2017 /PRNewswire/ -- Benitec Biopharma Limited (ASX:BLT; NASDAQ:BNTC; NASDAQ:BNTCW) is pleased to announce it has submitted an application with the U.S. Food & Drug Administration (FDA) seeking orphan drug designation for BB-301 as a treatment of oculopharyngeal muscular dystrophy (OPMD).

BB-301 is a single vector (gene therapy construct) system which uses DNA directed RNA interference (ddRNAi) to silence expression of the mutant gene associated with OPMD, while simultaneously adding back a copy of the normal version of the same gene to restore gene function.

To receive orphan drug designation from the FDA, a company must demonstrate that the condition addressed by the drug or biologic affects less than 200,000 persons in the U.S. The company must also provide the FDA with sufficient information to establish a medically plausible basis for expecting the product will be an effective treatment.

The incentives for receiving orphan drug designation include a seven-year market exclusivity commencing on product approval; tax credits; assistance in regulatory proceedings; and full exemption from the FDA's drug registration fees.

Benitec CEO Greg West commented, "This submission follows on from receiving earlier this year orphan designation from the European Medicines Agency and is an exciting first step in our effort to secure orphan status for BB-301 in the U.S. where we believe BB-301 has the potential to be a valuable asset in the treatment of OPMD."

In addition, over the past several weeks, the Benitec team has now completed pre-investigational new drug application (pre-IND) and scientific advice meetings with the U.S. FDA, Health Canada and several European agencies. The purpose of these meetings was to discuss the regulatory development pathway for BB-301 as a treatment for OPMD and to ensure Benitec's proposed development program addressed the regulatory expectations of these agencies. The agencies addressed Benitec's questions related to nonclinical,

manufacturing and the clinical design of the proposed initial BB-301 clinical trial. In addition to the meetings, the transfer of production protocols and optimisation of the processes related to manufacturing of BB-301 are well underway at Benitec's contract manufacturing organisation (CMO). Benitec intends to file an IND in the 4th quarter of calendar year 2018.

Greg West CEO of Benitec said "Our meetings with the FDA and other regulatory agencies were very productive and their guidance will be most valuable in assessing the appropriate clinical and regulatory strategies for BB-301. The Benitec team is executing on the key initiatives required to advance BB-301 into human clinical trials and we remain on track with our plan to file an IND with the FDA in the 4th quarter of 2018 and, assuming approval on a normal time-frame, we should be in an initial human clinical study by the end of 2018."

Background information

OPMD is a rare progressive, muscle-wasting disease caused by mutation in the poly(A)-binding protein nuclear 1 (PABPN1) gene, that is characterised by eyelid drooping, swallowing difficulties, and proximal limb weakness.

In August 2017, Benitec reported on a single AAV vector system that uses a ddRNAi approach combined with protein replacement to 'silence and replace' the mutant PABPN1 protein. In an A17 mouse model of OPMD, in a dose-dependent and time-dependent manner, BB-301 treatment produced robust knockdown of PABPN1 levels, including the mutant form of the protein, by up to 88%, while simultaneously restoring wild type PABPN1 to levels of up to 90% of normal. A17 mice display many of the clinical signs of OPMD including intranuclear inclusions (INIs), fibrosis, and loss of muscle strength. BB-301 treatment results in substantial correction of INIs and fibrosis as well as muscle strength. Importantly, treatment with the new clinical candidate BB-301 restored the ratio of muscle weight to body weight to normal levels. This single vector system represents the clinical candidate that Benitec intends to advance into human clinical trials.

For further information regarding Benitec and its activities, please contact the persons below, or visit the Benitec website at 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 head & neck squamous cell carcinoma, OPMD retinal based diseases such as wet age-related macular degeneration, and hepatitis B. 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.

About OPMD:
OPMD is a rare inherited myopathy characterized 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 last two symptoms are often the cause of death. No cure is currently available for OPMD. The cricopharyngeal myotomy is the only treatment available to improve swallowing in these patients, but because the root cause of the genetic disease has not been addressed, the pharyngeal musculature still undergoes progressive degradation leading to the previously mentioned complications.

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 this ASX/Nasdaq announcement 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.

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