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Key pre-clinical data on oculopharyngeal muscular dystrophy (OPMD) published in Nature Communications

SYDNEY, April 4, 2017 /PRNewswire/ -- Benitec Biopharma Limited (ASX: BLT; NASDAQ: BNTC; NASDAQ: BNTCW) is pleased to announce that the initial pre-clinical efficacy results of the OPMD program have been published in Nature Communications, an open access scientific journal published by the Nature Publishing Group. OPMD, a rare progressive muscle-wasting disease caused by mutation in the poly(A)-binding protein nuclear 1 (PABPN1) gene, is characterised by eyelid drooping, swallowing difficulties, and proximal limb weakness.

A direct link to the article can be found on Nature's website
http://www.nature.com/articles/ncomms14848.epdf

The key results from these studies demonstrate that a DNA directed RNA interference (ddRNAi) approach to 'silence and replace' the mutant PABPN1 protein, results in the correction of the muscular dystrophy and of key clinical features of OPMD including a progressive atrophy and muscle weakness associated with nuclear aggregates of insoluble PABPN1. These data were generated in the A17 mouse model that expresses the mutant PABPN1 gene and mimics most of the features of human OPMD patients. These findings were central in being able to receive the Orphan Drug Designation in the European Union for the OPMD program in January of this year.

"These published results have been critical for establishing the proof of concept that a ddRNAi approach may be able to treat this orphan disease," said Dr David Suhy, Chief Scientific Officer at Benitec. "Furthermore, this program highlights one of the unique aspects of the Benitec technology that is not readily attainable by other gene therapy approaches. Specifically, through our unique approach to gene silencing and gene therapy, we are able to knock out the mutated form of the gene and have the ability to express a normal copy to restore function. We are extremely excited about the progress we have made with our OPMD program and, with our European Orphan Drug Designation, we look forward to streamlining the process towards regulatory approval."

Benitec has been working with research groups headed by Prof George Dickson at the Royal Holloway University of London (RHUL) as well as by Dr Capucine Trollet at the Myology Research Center (INSERM/UPMC/AIM/CNRS) based in Paris. This collaboration, initiated from RHUL, has specifically shown that the combination of two recombinant adeno associated virus (AAV) vectors, one allowing the inhibition of mutated PABPN1 by ddRNAi, and the other expressing a functional PABPN1, significantly reduces the amount of PABPN1 nuclear aggregates, decreases muscle fibrosis, reverts muscle strength to the level of healthy muscles and normalises the expression of RNA. The efficacy of the combined

treatment was also confirmed in cells derived from OPMD patients.

Benitec and its collaborators are currently pursuing the advancement of BB-301, a next generation, follow-on ddRNAi therapeutic for the treatment of OPMD, that combines both the 'silence and replace strategy' of mutant PABPN1 into a single vector. 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

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About OPMD:

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. These last two 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 aforementioned complications.

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/key-pre-clinical-data-on-oculopharyngeal-muscular-dystrophy-opmd-published-in-nature-communications-300433996.html

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