



## **ASX RELEASE – FOR IMMEDIATE RELEASE**

### **Benitec undertakes new Europe-based collaboration to develop a novel therapeutic for a life-threatening muscular dystrophy**

- **New project in an orphan indication**
- **Benitec's first genetic diseases program**
- **No current cure or treatment for this extremely debilitating and potentially life-threatening disease**
- **Bolsters Benitec's pipeline and further demonstrates acceptance of RNAi approach**

Sydney, October 31, 2011. The Directors of Benitec Ltd (ASX:BLT) are pleased to announce that the Company has signed an agreement with the Royal Holloway University London (RHUL) to commence a new program aimed at developing a novel therapeutic and potentially curative approach to a currently untreatable and fatal genetic muscle disease, oculopharyngeal muscular dystrophy (OPMD).

The research will be carried out under the direction of Professor George Dickson, at RHUL in London and in laboratories in Paris, by Dr Capucine Trollet at the Institut de Myologie, in the Universite de Pierre et Marie Curie (UPMC).

OPMD is an inherited, slow progressing, late onset degenerative muscle disorder. It is caused by a genetic mutation that results in a progressive destruction of muscles involved in the eye, face, swallowing and general movement. The most severe effect is on the swallowing muscles. This causes severe swallowing problems for patients which can lead to life-threatening consequences.

There is no cure, nor any medical treatment available for OPMD. Symptomatic surgical interventions are used to temporarily improve swallowing in moderate to severely affected individuals, but these are inadequate treatments for most afflicted individuals.

OPMD is a rare disease (1/100 000 in Europe), with a worldwide distribution, however it is the most common muscular dystrophy in Quebec, Canada (1/1000). OPMD therefore qualifies as an orphan disease. There are some regulatory advantages and significant financial incentives for developers of orphan disease therapies in several countries including the US (where there is an Office of Orphan Products Development under the USFDA), Australia, Europe and Japan. There is a growing trend with big pharmaceutical companies seeking orphan indication opportunities or orphan drug opportunities.

Benitec has undertaken this collaboration with the aim of developing a ddRNA interference-based therapy to silence the expression of the mutant gene in muscle cells of OPMD patients. OPMD is particularly adapted to gene therapy since the affected cells are limited, the genetic mutation is small, known, and located on a relatively small gene.

Professor Dickson said, "The opportunity to work with Benitec and to access their impressive patent position in gene silencing is very exciting for us. We believe that Benitec's technology can lead to a breakthrough treatment, and even a cure, for this rare but often fatal disease. We are looking forward to making this program a clinical reality utilising the wonderful team and resources at the Royal Holloway University and at the Institut de Myologie."

Dr French, Benitec CEO said, "Benitec's strategy is to demonstrate the power and the potential of

its RNA interference technology across a range of severe life threatening diseases, and the OPMD program extends our reach into treating and curing genetic disorders in addition to our cancer and infectious disease programs, Professor Dickson and Dr Trollet will join Benitec's Chief Investigators Group and, as international experts in genetic disease and RNAi, will contribute significantly to Benitec's intellectual capital and reputation for scientific and clinical excellence."

### **For Further Information**

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### **About Benitec** [www.benitec.com](http://www.benitec.com)

Benitec Limited is developing novel treatments for chronic and life-threatening conditions based on gene silencing using a transformational technology, DNA-directed RNA interference (ddRNAi) - sometimes called expressed RNAi. The technology's potential to address unmet medical needs and, potentially, to cure disease results from its demonstrated ability to permanently silence genes which cause the condition.

Benitec now either owns or exclusively licences from CSIRO more than 40 granted or allowed patents in the field of RNA interference for human therapeutic applications. Patents have been granted in key territories such as the USA, the UK, Japan, Europe, Canada and Australia. In addition, Benitec has almost 50 patent applications pending for which it is the owner or exclusive licensee from CSIRO, and has further intellectual property under development as a result of its pipeline program.

Benitec trades on the Australian Securities Exchange (ASX) under the symbol "BLT". The Company was founded in 1997 and has been publicly held since 2001. The Company aims to deliver a range of novel ddRNAi-based therapeutics to the clinic in partnership with the pharmaceutical industry. In-house it is pursuing a focused R&D strategy in infectious diseases, cancer and chronic cancer-associated pain, as well as programs with licensees that have advanced to pre-clinical and/or clinical trials.