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Benitec Up 124% On Up-To \$892m Axovant Gene Therapy Deal

Benitec climbed as much as 124.1 percent on an up-to \$US665 million (\$A891.65m) deal with the London and Bermuda based Axovant Sciences for its gene therapy programs.

Benitec said it would receive an upfront cash payment of \$US10 million with additional cash payments totalling \$US17.5 million for near-term milestones for BB-301 for oculo-pharyngeal muscular dystrophy (OPMD), renamed AXO-AAV-OPMD.

The company said it would be eligible for \$US187.5 million in milestone payments, including the \$US17.5 million, and would retain 30 percent of the net profits on the worldwide sales of AXO-AAV-OPMD.

Benitec said it would partner with Axovant on five additional gene therapy programs for neurological disorders, receive full research funding for each program and be eligible for \$US93.5 million in development, regulatory and commercial milestones, for each program.

Benitec executive chairman Dr Jerel Banks said that “today marks a milestone for Benitec as we believe this transaction to be transformative for our company”.

“In addition to bolstering our opportunity to drive broad-based, clinically meaningful patient benefit across several areas of clinical medicine with true unmet need, this partnership significantly enhances the financial, intellectual, and clinical development resources available to facilitate our efforts to build Benitec into a diversified biopharmaceutical company,” Dr Banks said.

“The non-dilutive capital expected over the near term will allow Benitec to continue to invest in ... programs across a range of indications,” Dr Banks said.

“Our management team is focused exclusively on expanding the research, development, and commercial opportunities for the core silence-and-replace platform with the dual goals of enhancing patient benefit and generating shareholder value,” Dr Banks said.

Benitec said that oculo-pharyngeal muscular dystrophy was “a rare progressive, and often fatal, muscle-wasting disease caused by mutation in the poly(A)-binding protein nuclear 1 (PABPN1) gene, that is characterized by eyelid drooping, swallowing difficulties and proximal limb weakness”.

The company said AXO-AAV-OPMD was a single vector, gene-therapy construct system that used a silence-and-replace method employing DNA-directed RNA-interference (ddRNAi) to silence expression of the mutant gene associated with OPMD, while simultaneously expressing a copy of the normal, healthy version of the same gene to restore the function of that gene.

Benitec said that Axovant planned to begin a placebo-controlled clinical study in 2019 in which a single intra-muscular administration of AXO-AAV-OPMD would be given to patients to treat the dysphagia associated with OPMD.

Axovant chief executive officer Dr Pavan Cheruvu said that “the silence-and-replace platform is a targeted approach which directly addresses the underlying genetic cause of diseases arising from expression of dysfunctional proteins, including those caused by nucleotide repeat expansion”.

“I am excited about the potential of this platform for patients suffering from OPMD, many of whom have limited treatment options today,” Dr Cheruvu said.

Benitec said that the first of the five additional programs would focus on developing a single vector silence-and-replace gene therapy product targeting the c9orf72 gene, which was associated with amyotrophic lateral sclerosis and fronto-temporal dementia.

Benitec said that in addition to funding for development of the new research programs, each program target was eligible for milestones totalling \$US93.5 million and tiered royalties on global sales.

Benitec closed up 16 cents or 110.3 percent to 30.5 cents with 28.2 million shares traded.