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Benitec Reinvents ddRNAi As Platform For Pain, Cancer, More

Benitec has taken its “Chief Investigators Group” to investor and analysts meetings in Melbourne and Sydney to unveil a new commercialization strategy.

Along with chairman Peter Francis, chief executive officer Dr Peter French and director Iain Ross, the company presented with inventor Dr Michael Graham, the originator of the core Graham '099 patent, the chief executive officer of the Shanghai-based Biomics Biotechnologies Dr York Zhu, the head of pharmacoproteomics at the University of New South Wales Prof Maria Kavallaris and the California-based City of Hope's Prof John Rossi.

At an investor briefing at Patersons Securities in Melbourne, Dr French said that a program targeting the PKC-gamma to control neuropathic pain with a single injection had replaced the previous hepatitis C and HIV programs as the company's lead program.

Dr French and Prof Kavallaris provided details of new timelines for the range of programs.

Dr French said that the DNA-directed RNA interference program (ddRNAi) had applications in cancer-associated pain, drug-resistant lung cancer, hepatitis B and muscular dystrophy.

Dr French said the company hoped to take an investigational new drug application package to the US Food and Drug Administration for the neuropathic pain program by May 2012 and complete a preliminary clinical trial design.

Dr French said that PKC-gamma was “at the top of the pain pathway, specific to neuropathic cancer pain” and the end stage cancer patients made the process easier in terms of regulatory and scientific process and “easier to monetize than hepatitis C and HIV”.

Dr French said that Biomics and Benitec were developing a “curative therapy for hepatitis B” and Dr Zhu’s team had selected several short hairpin RNA constructs that would be packaged into a triple cassette and delivered to the liver via an adeno-associated virus AAV8.

He said pre-clinical work had shown that the vector could be delivered to 100 percent of liver cells.

Dr French said the company had begun work on a therapy for the orphan indication ocular-pharyngeal muscular dystrophy which caused death through choking.

He said he expected the compound to be in the clinic in 2014.

Prof Kavallaris said that higher levels of beta-III tubulin were found in drug-resistant non-small cell lung cancers and would be the target of a Benitec short-hairpin RNA program.

Prof Kavallaris said that survival rates for non-small cell lung cancers were less than 10 percent at five years and it was one of the most common cancers.

She said that shutting down beta-III tubulin appeared to sensitize tumor cells to chemotherapy, and they were not expressed in normal lung cells.

Prof Kavallaris said the first mouse treated had its beta-III tubulin count knocked down by 60 percent.

A chart of overall survival indicated that 50 percent survival was about 300 days longer in people with lower beta-III tubulin levels.

The Benitec presentation provided a timeline with its investigational new drug application preparation by 2013 and a phase I trial in 2014.

Prof Kavallaris said beta-III tubulin was also expressed in ovarian, breast, prostate cancers and glioblastomas, with potential for the treatment to work in any cancer.

Dr French said that the first patient City of Hope HIV patient treated with a Lentivirus short hairpin RNA stem cell transplant was still expressing short hairpin RNA, three years after the single treatment.

Benitec fell 0.1 cents or 6.25 percent to 1.5 cents with 7.1 million shares traded.