Politics

Balmain biotech has 10 shots at billions

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The journey from Commonwealth Scientific and Industrial Research Organisation test tube to US Food and Drug Administration approval is one that few medical technologies survive, but Balmain-based Benitec is trying 10 different routes for its "gene-silencing" treatment, including one potential shortcut through the FDA process.

A shortcut to monetisation would be welcome news for Benitec's investors, especially the mums and dads who have stuck with the company since its ASX listing in 1997 and are lauded by Peter French, the chief executive since 2010, as "visionaries", albeit visionaries whose investment is, at October 5's share price of 46¢, "deep under water".

Benitec's technology, which it is hoping might provide a one-shot cure for everything from hepatitis C to AIDS, is called DNA-directed ribonucleic acid interference, or ddRNAi.

Benitec chief executive Peter French hopes the company's hepatitis C trial can gather enough supportive data to entice a big pharmaceutical company to buy the application.

It was patented in the 1990s by former CSIRO scientist Dr Michael Graham, who founded Benitec in 1997 and rejoined in 2012, the same year Benitec's patents were re-issued at the conclusion of lengthy litigation.

The next 18 months are critical in Benitec's 18-year rollercoaster ride, French tells BRW. Over his five-year tenure he has already transformed Benitec from a company with $500,000 at bank, no labs and no clinical programs to one with $40 million cash, a Nasdaq dual listing, a lab in San Francisco and clinical programs testing ddRNAi's effectiveness against 10 diseases globally.

Most advanced

The most advanced of these on paper is treating hepatitis C, with Benitec just adding a fourth site [http://www.asx.com.au/asxpdf/20150916/pdf/431c2hrndpmlg9.pdf] to its US trial. French hopes this trial can gather enough supportive data to entice a big pharmaceutical company to buy the application and undertake the $500 million business of getting it through "phase 3" FDA approval and on to the market.

Wistfully, he points to the $US11 billion ($15.5 billion) paid by Gilead in 2011 for Pharmasset, maker of a 12-week pill-based treatment for hepatitis C that is the biggest-selling drug in the world now.

However, the hepatitis C application for ddRNAi might be beaten to commercialisation by an application Benitec is about to test for a rare form of muscular dystrophy called oculopharyngeal muscular dystrophy (OPMD).

Known as an "orphan" disease because so few people suffer it – 3000 across the entire US – a successful treatment for OPMD could qualify for "breakthrough therapy" status with the FDA, French says.

"We can get phase I/II data from 10 patients, and if the FDA is satisfied that it's safe and that there's no other treatment for the disease, we'd be able to take it to market ourselves."

Already played out

This scenario has already played out for Dutch gene therapy maker UniQure, which received the European Union's equivalent of breakthrough status for Glybera, a one-shot treatment for an "orphan" disease called familial lipoprotein lipase deficiency, which effects about 200 people in Europe.

Each shot of Glybera is priced at $US1 million, although subsequent doubts about its efficacy from health authorities in some countries have made it less likely insurers will reimburse its cost [http://www.bloomberg.com/news/articles/2015-05-21/world-s-most-expensive-medicine-faces-first-test-in-germany], at least for now.

Benitec wouldn't charge anything like $1 million for a breakthrough-approved OPMD treatment, French says.

"We'd charge less than that to get it on to the market quickly. It could even be a break-even or a loss leader for us, just to show that this technology works – in the case of OPMD we can both turn off the mutant gene and put in a healthy gene – and announce to people that we've also got applications for hep C, hep B et cetera in the pipeline."

Benitec is working with London's Royal Holloway University and the French national reference centre for muscle disorders on the OPMD application, which will proceed to clinical trial once the construct to be injected is finalised.