Genetic silencing is here

02 Jun 2014

By Yasmin Noone

Australian scientists have used a novel gene silencing technology to inject DNA and treat a patient with Hepatitis C, for the first time.

Scientists have pioneered a new technology called ddRNAi which uses a single injection to silence Hepatitis C virus genes.

Australian biotech company, Benitec Biopharma, is developing the technology called ddRNAi, which was originally discovered by the CSIRO.

Clinicians at Duke University Medical School in the United States administered the treatment, TT-034, as a single infusion to an American patient last week.

It will be tested on 14 patients with Hep-C at varying doses to ensure its safety, before being trialed in a larger number of patients with the condition.

“This is the first time this technology has been tested in humans for Hep- C and tested by a single-shot injection”, said Dr French, CEO and managing director at Benitec.

“The way ddRNAi works is that it turns off genes associated with the disease. Because it is DNA-based, the technology has the potential to treat and even cure diseases such as hepatitis C with a single injection.”

Hepatitis C is a blood-borne virus that causes inflammation of the liver, and is often spread by sharing needles.

To-date, Dr French explains, the only way of genetically treating hepatitis C would be to take cells from the patient, treat them with the technology and then put the cells back into the patient.

Above: Dr Peter French, CEO and managing director of Benitec

more news» (/news)

- New brand, name & partnership for Domain (/news/new-brand-name-and-partnership-for-domain)
- Medicare: Thanks but farewell (/news/medicare-thanks-but-farewell)
- New 'No GP Tax in our name!' campaign (/news/new-no-gp-tax-in-our-name-campaign)
- In it to win it (/news/in-it-to-win-it)

“Once injected into the patient, the DNA of the drug will go into a patient's liver cells. It will sit there and stay therefor the lifetime of the liver celss - months to years- and continue to produce the molecules that turn off the hep-C virus. “

Conventional non-genetic treatments currently available require patients with hep-C to take three drugs orally a day for at least 12 weeks.

Dr French said poor compliance rates and side effects mean patients do not usually complete the course and treatment can fail.

Genetic silencing via injection means a one-off treatment that could last months or even years.

“This will be eaiser for patients as it is a simple, straight forward procedure.

“The patient goes into hospital, has a one-hour long infusion of the drug and then goes home.

“And that will be the only treatment they receive.”

The trial will aim to investigate and prove the safety of genetic silencing using ddRNAi and the treatment procedure.

It also marks the start of a long journey in clinical testing.

“The era of gene therapy medicine is a number of years away but we are optimistic this trial will show that there is no serious toxicity [that results from the injection].

But, Dr French said, the trial offers hope that genetic silencing technology can provide long -lasting treatments for a range of diseases, not just hepatitis C.

“The importance of this first trial is that it demonstrates the capability of the technology and validates it as a whole.”

He said there are plans to use genetic silencing to treat other conditions like lung cancer and macular degeneration with research programs currently underway.

“Every new medicine has potential risks. This is a new technology that, once injected, cannot be withdrawn [from the human body because it inserts a piece of DNA into patients’ cells.].

"But I've been a medical research scientist for 35 years and I've never seen technology that comes anywhere near this in its potential to address serious diseases

“If we can demonstrate its ability to treat and even cure a range of difficult life threatening diseases, then ddRNAi will be another example of game-changing Australian innovation.”

brunibrewin • 9 days ago

It is an exciting potential future help. Whilst I am all for Natural Therapy, when a disease is so debilitating and difficult to treat, then this furture potential gives hope to many.