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NZ leads world in trial of futuristic therapy

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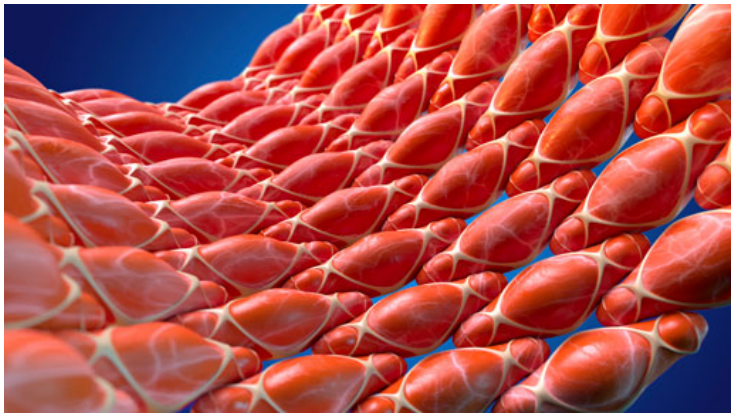
An innovative treatment that repairs a faulty genetic mechanism is being trialled by Kiwis with a rare muscledisease for the first time in the world



A treatment that works at the gene level to solve the root cause of a genetic disease will soon be tested at the University of Auckland, New Zealand (NZ) for the first time in the world.

The molecular therapy will be used to treat ten people with myotonic dystrophy, a progressive muscle disease that is passed on from generation to generation. It affects around half of family members and gets worse over time and over generations.

“Myotonic dystrophy is a muscle disease, but it also affects the brain and heart; it causes diabetes. We come across it in all sorts of specialties,” neurologist Associate Professor Richard Roxburgh says.

Myotonic dystrophy affects more than 300 people in Aotearoa New Zealand. It can cause sudden unexpected death in some people, while others live with the disease for many years.

Scientists found the genes causing many genetic diseases in the 1990s, but only in recent years have therapies been reaching the stage of clinical trials and/or availability for prescription.

US company Dyne Therapeutics is running the ‘first in human’ trial at the University of Auckland, with other sites around the world joining once they also get regulatory approval. Roxburgh is hopeful about the treatment, which has shown a good safety profile in overseas animal studies.

Clinicians hope that the trial medication will halt or even reverse the disease’s impacts, because it acts at such a fundamental level of the disease process.