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NZ Pharma to supply drug candidate for NIH trial

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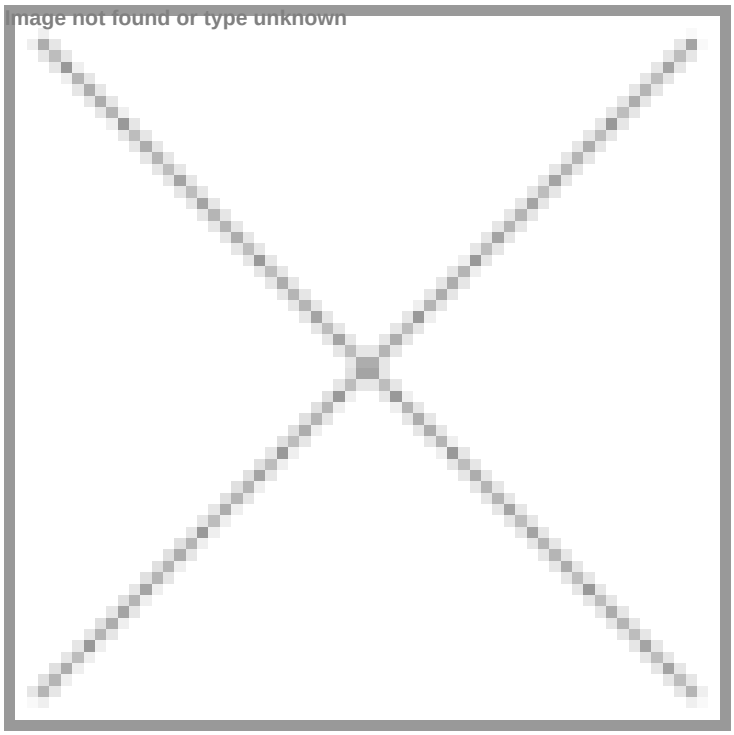
New Zealand Pharmaceuticals to manufacture drug candidate for NIH trial



Singapore: New Zealand Pharmaceuticals (NZP), a manufacturer of pharmaceutical intermediates and APIs, will manufacture DEX-M74 for clinical trial to evaluate it as a treatment for patients suffering from hereditary inclusion body myopathy (HIBM), a rare degenerative muscle disease.

The phase I clinical trial has been launched by the US National Institutes of Health (NIH). NIH scientists from the National Center for Advancing Translational Sciences (NCATS) and the National Human Genome Research Institute (NHGRI) will conduct the clinical trial at the NIH Clinical Center, using DEX-M74 manufactured and supplied by NZP. NZP is the licensee of the NIH IP for DEX-M74.

HIBM, also known as GNE myopathy, is a rare genetic disease with no available therapy. Disease symptoms emerge in adulthood and slowly lead to progressive muscle weakness. Most patients develop symptoms while in their early 20s and eventually require a wheelchair as their arm, hand and leg muscles weaken. Mutations in the GNE gene lead to low sialic acid levels in muscle proteins, which researchers think causes HIBM. Normally, GNE produces an enzyme that produces sialic acid in the body, a sugar important to muscle development and kidney function.

In 2007, Dr Marjan Huizing, an associate investigator in NHGRI's Medical Genetics Branch, led a team of scientists in search of an HIBM treatment. They hypothesized that a compound called ManNAc, now called DEX-M74, might have an impact on the muscle weakness caused by HIBM. DEX-M74 is a sugar converted by the body to sialic acid. Huizing and colleagues

conducted studies that showed the compound was effective in controlling muscle weakness in a mouse model with a specific GNE mutation. The researchers published their findings in the June 2007 issue of the *Journal of Clinical Investigation*.

In 2009, NIH established its Therapeutics for Rare and Neglected Diseases (TRND) program, now a part of NCATS, to facilitate the pre-clinical development of new drugs for these ailments. TRND scientists selected the development of DEX-M74 as a treatment for HIBM as one of its initial pilot projects. The collaboration includes the laboratory of Dr William Gahl, principal investigator of NHGRI's Medical Genetic Branch, New Zealand Pharmaceuticals, which is manufacturing DEX-M74, and TRND researchers.

The HIBM phase I clinical trial will test a single dose of DEX-M74 in a small group of patients with a focus on drug safety and how patients tolerate the drug. Dr Nuria Carrillo, TRND staff physician and principal investigator of the HIBM phase I trial, plans to follow-up with a phase IIa trial in which patients will receive multiple doses of DEX-M74. Researchers will monitor patients for drug tolerance and indications of drug effectiveness as well.

Mr Andy Lewis, CEO of NZP, expressed his gratitude that the TRND supported program has started and acknowledges with thanks the huge effort the NHGRI, the Clinical Center and the NCATS teams put into the program. "The NIH has achieved a significant milestone in the development of a potential treatment for HIBM and we are excited about this research reaching the clinical trial stage. The pre-clinical data is very strong and we are keen to see DEX-M74 progress through the Clinical Phases".

NZP holds the NIH license to take DEX-M74 forward when safety and proof of efficacy have been achieved for HIBM and glomerular kidney diseases.