Protein injection points to muscular dystrophy treatment

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Scientists have discovered that injecting a novel human protein into muscle affected by Duchenne muscular dystrophy significantly increases its size and strength, findings that could lead to a therapy akin to the use of insulin by diabetics. These results were published today in the *Proceedings of the National Academy of Sciences* by Dr. Julia von Maltzahn and Dr. Michael Rudnicki, the Ottawa scientist who discovered muscle stem cells in adults.

"This is an unprecedented and dramatic restoration in muscle strength," says Dr. Rudnicki, a senior scientist and director for the Regenerative Medicine Program and Sprott Centre for Stem Cell Research at the Ottawa Hospital Research Institute. He is also a Canada Research Chair in Molecular Genetics and professor in the Faculty of Medicine at the University of Ottawa.

"We know from our previous work that this protein, called Wnt7a, promotes the growth and repair of healthy muscle tissue. In this study we show the same types of improvement in a mouse model of Duchenne muscular dystrophy. We found that Wnt7a injections increased muscle strength almost two-fold, to nearly normal levels. We also found that the size of the muscle fibre increased and there was less muscle damage, compared to mice not given Wnt7a."

Duchenne muscular dystrophy is a genetic disorder that affects one of every 3,500 newborn males. In Canada, all types of muscular dystrophy affect more than 50,000 people. The disease often progresses to a state

where the muscles are so depleted that the person dies due to an inability to breath. For people with Duchenne muscular dystrophy, this usually happens in their 20s or 30s.

"This is also exciting because we think it's a therapeutic approach that could apply to other muscle-wasting diseases," says Dr. Rudnicki.

Dr. Rudnicki's lab is a world leader in research on muscle stem cells. They have contributed significantly to our understanding of how these cells work at the molecular level. This basic research, which takes place in OHRI's multidisciplinary environment of collaboration with clinicians, led to the identification of Wnt7a as a promising candidate to help people with this muscle wasting disease.

Biotechnology partner, Fate Therapeutics is currently developing Wnt7a-based therapeutic candidates for treatment of muscular dystrophy and atrophy. Preclinical assessments are ongoing and the company plans to initiate clinical trials in the near future.

More information: The full article, "Wnt7a treatment ameliorates muscular dystrophy," is available online ahead of print through the *Proceedings of the National Academy of Sciences* site: www.pnas.org/content/early/201 … /1215765109.abstract

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