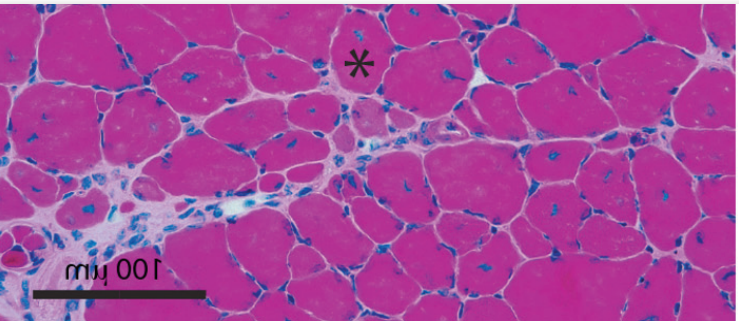



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# Agents useful in treating facioscapulohumeral dystrophy

UMONS health
UMONS RESEARCH INSTITUTE FOR HEALTH SCIENCES AND TECHNOLOGY

## Description

Our team has identified a strategy for potential **therapeutics against facioscapulohumeral dystrophy (FSHD)**. FSHD is an inherited disease characterized by progressive muscle atrophy from top to bottom of body. It affects 1 in 15000 individuals, and with the first symptoms at the age of 20 years, it requires the use of the wheelchair at about 45 years for 20% of patients. No therapy is available currently. Our laboratory identified the DUX4 gene whose inappropriate activation in muscles of patients triggers the disease process.

As a promising therapeutic strategy, we have identified **siRNAs** that specifically target the mRNA DUX4 and block the expression of the DUX4 protein in an experimental model of myoblast (muscle cell) culture. On the other hand, we identified **antisense oligonucleotides** targeting the DUX4 messenger RNA and also block the expression of DUX4 protein in mice or patient myoblasts.

## Inventors

From Department of Molecular Biology (University of Mons): Alexandra Belayew, Frédérique Coppée, Eugénie Ansseau, Céline Vanderplanck
From Centre for Neuromuscular and Neurological Disorders (University of Western Australia, Queen Elizabeth II Medical Centre): Donald Stephen Wilton.

## Patent

Regarding antisense oligonucleotides : European patent application EP11179924 filed on September 2nd 2011 and US divisional application US14/078.138 (filed on November 12th 2013 based on mother application US13/225.384 on September 2nd 2011).

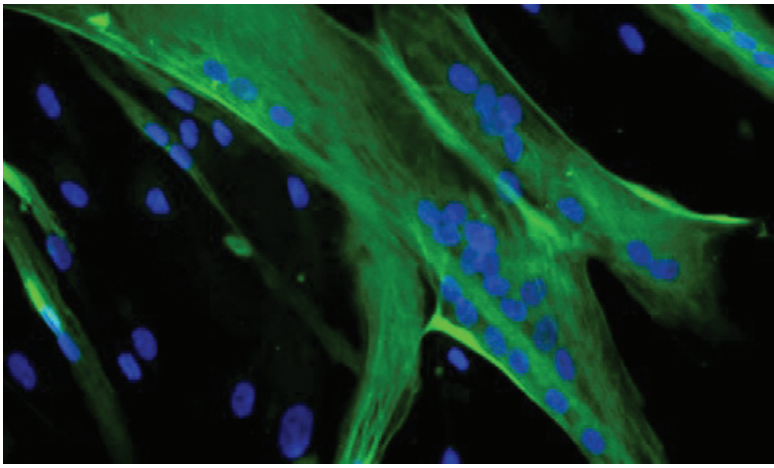
Regarding siRNA: new divisional application (in progress) based on EP11179924

## Stage of development

**TRL 2** – lab scale

## Type of collaboration needed

Research collaborations – License agreements



**KEYWORDS**

**Therapeutics - FSHD - Facioscapulohumeral Dystrophy - DUX4 - Myopathy - Antisense Oligonucleotides - SiRNA**