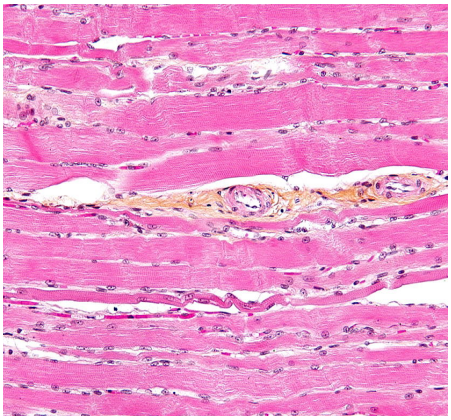
FRED HUTCH
CURES START HERE®



THERAPEUTIC FOR FSHD

Targeting DUX4 for the Treatment of Facioscapulohumeral Muscular Dystrophy (FSHD)

Brief Technology Description

Anti-sense targeting of DUX4 to inhibit disease pathogenesis.

BUSINESS OPPORTUNITY
Non-exclusive license
Sponsored research

TECHNOLOGY TYPE
Therapeutic
Orphan
Epigenetic
Nucleic acid
Anti-sense

STAGE OF DEVELOPMENT
Preclinical *in vitro*

PATENT INFORMATION
US 20130288976
EP 2606152

LEARN MORE
Tech ID: 11-003
partnering@fredhutch.org
206-667-4304

Technology Overview

Facioscapulohumeral dystrophy (FSHD) results in progressive loss, wasting and atrophy of all skeletal muscles and is the most prevalent hereditary muscular dystrophy affecting men, women, and children. FSHD is caused by the contraction of the D4Z4 macrosatellite repeat in the sub-telomeric region of chromosome 4q and results in bursts of misexpression of the DUX4 transcription factor in muscle cells. A team of researchers led by Dr. Tapscott at Fred Hutch have discovered therapeutic strategies to inhibit DUX4 activity through anti-sense nucleotide approaches, such as siRNA.

Applications

- Therapeutic treatment for FSHD

Advantages

- Targets the underlying pathogenesis of FSHD
- Potential to be used in combination with other therapeutic approaches in development for FSHD

Market Overview

The prevalence of FSHD across the world stands at nearly 870,000. There are currently no approved treatment options for patients with FSHD. Symptoms develop slowly and usually appear before the age of 20. Therefore, there is a critical unmet medical need to bring new treatment options for patients with FSHD.

Investigator Overview

Stephen Tapscott, MD, PhD, Human Biology and Clinical Research Divisions