Agents useful in treating facioscapulohumeral dystrophy

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

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Patent


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