FIBROSIS THERAPEUTIC TREATMENT IN A MUSCULAR DYSTROPHY CONTEXT

A palliative treatment for fibrosis in a Muscular Dystrophy context consisting in an antago-miR that effectively enhances tissue regeneration, ameliorates fibrosis, allows for a slower development of the illness and improves patient's quality of life.

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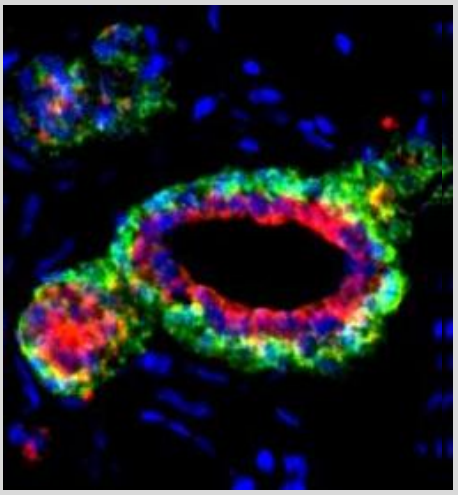

BACKGROUND

Muscular dystrophy (MD) refers to a group of inherited myopathies that weakens the muscles that move the human body. MDs are characterized by progressive skeletal muscle weakness, defects in muscle proteins, and the death of muscle cells and tissue. There are more than 100 diseases in total with similarities to MD. Most types of MD are multi-system disorders with manifestations in body systems including the heart, gastrointestinal and nervous systems, endocrine glands, skin, eyes and even brain. The condition may also lead to mood swings and learning difficulties. Fibrosis is a hallmark not only for MD but for many other muscle conditions like accidental lacerations, sportive lesions, ageing. Although gene therapy and cell therapy may provide a cure for MD in the future, currently there is no effective therapy. Anti-fibrotic therapies cannot, however, prevent skeletal muscle fiber degeneration, let alone induce muscle regeneration. Thus, muscle dysfunction, once occurred, cannot be in any way reversed.

THE TECHNOLOGY

The inventors have found that skeletal muscle tissue dysfunction in MD is related to the deregulation of a miR-21. miR-21 regulates collagen deposition and fibrosis progression It has also been surprisingly found that down regulation of miR-21 effectively enhances tissue regeneration and ameliorates fibrosis. As dystrophin replacement therapies are presently unsuccessful, delivery of agents decreasing fibrin accumulation may constitute alternatives for MD treatment.

ADVANTAGES

- The patient can benefit from the treatment both at an early stage of the disease, to avoid muscular damage, as well as at a later stage, to recover muscle functionality.
- Allows for a slower development of the illness and improves patient's quality of life.

STATE OF DEVELOPMENT

Treatment has been tested in mice and a reduced number of human patients samples with good results.

INTELLECTUAL PROPERTY

A European patent covering target as well as a few agonists of miRNA-21 has been filed.

MARKET OPPORTUNITY

Muscular Dystrophies are a group of Rare Diseases. Duchenne muscular dystrophy is the most common genetic muscle disease affecting 1/3,500 live male births. Currently there is no effective therapy for MD.

COMMERCIAL OPPORTUNITY

We are looking for a partner to continue the preclinical experiments and/or license the patent for clinical development.

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KEYWORDS

Fibrosis, Muscular Dystrophy, miRNA.

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