ANTISENSE RNA-THERAPEUTICS IN MYOTONIC DYSTROPHY

DESCRIPTION OF THE TECHNOLGY

Myotonic Dystrophy (DM) is a rare degenerative disease (DM, prevalence of 1⁄8000 that constitutes a great burden to the national health systems). Although rare, DM is the most common muscle dystrophy in adults and it is a highly disabling disease with symptoms involving the nervous system, the heart, and skeletal musculature. Nowadays, management of symptoms is the only way of preserving the quality of life for individuals living with DM.

DM type 1 (DM1), the most common form of DM, is originated by sequestration of alternative splicing factors known as Muscleblind-like proteins (MBNL) by aberrant gene transcripts. Lack of MBNLs causes RNA metabolism alterations originating the symptoms of the disease. In consequence, increasing MBNLs levels may be an alternative for the treatment of DM1

Our technology is based on the discovery of miRNAs inhibitors of MBNL as potential targets for the development of a DM1 therapy. In particular, the use of patented antagomiRs (chemically engineered oligonucleotides that silence these microRNAs by preventing their binding to MBNL mRNA molecules) increases MBNL expression and rescues characteristic DM defects.

This strategy has been confirmed in human cell lines from DM1 patients, fly-fruit models of DM1 and the well-stablished DM mouse model, in which subcutaneous injections of our antagomiRs at concentrations below the standard range published in literature, improved muscle histopathology, and myotonia and rescued MBNL-dependent splicing events.

MARKET APPLICATION SECTORS

Pharmaceutical and biotechnological companies interested in development of therapies for myotonic dystrophy as a clinical indication.

TECHNICAL ADVANTAGES AND BUSINESS BEENFITS

Our technology is highly translational and is supported by prestigious scientific team in collaboration with clinical key opinion leaders in DM1.

We count on a novel strategy for an unmet clinical need candidate for orphan drug designation.

CURRENT STATE OF DEVELOPMENT

Preclinical phase.

INTELLECTUAL PROPERTY RIGHTS

A European patent application has been filed related to this technology.

COLABORATION SOUGHT

- Patient advocacy groups willing to fund the clinical study
- Pharmaceutical companies interested in myotonic dystrophy.
- Biotech companies and academic intitutions with experience in optimization of RNA chemistries for PK/PD properties or propietary of other RNA chemistries.

CONTACT

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