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Instituto de Investigación Sanitaria

UCIE INCLIVA

MUSASHI 2 (MSI2) AS A THERAPEUTIC TARGET FOR THE TREATMENT OF MYOTONIC DYSTROPHY TYPE 1 AND 2

DESCRIPTION OF THE TECHNOLOGY

Myotonic dystrophy (MD) is a rare inherited muscle disease characterised by muscle weakness (myopathy), muscle stiffness and problems relaxing muscles (myotonia), and progressive muscle wasting (atrophy). Myotonic dystrophy is classified into two types, type 1 (DM1) and type 2 (DM2). Their signs and symptoms are similar, although type 2 is usually milder than type 1 as it is caused by mutations in different genes. DM1 is a multisystem disorder that mainly affects skeletal and smooth muscle, the nervous system and the heart, and can affect newborns to the elderly.

Researchers at INCLIVA and the University of Valencia have identified a new therapeutic target for the treatment of myotonic dystrophy type 1 and 2 (Fig.1). This target is based on the molecular mechanisms of the Musashi 2 (MSI2) protein, as its levels influence DM cells. Therefore, they have shown that the use of gapmer antisense oligonucleotides that promote the degradation of MSI2 transcripts results in increased intracellular levels of miR-7. This modulation triggers the inhibition of (overactivated) autophagy and represents an excellent alternative for the treatment of myotonic dystrophy, as no specific treatment currently exists.

ADVANTAGES

- ✓ Possibility of studies with a wide variety of drugs due to the detection of the therapeutic target.
- ✓ Possibility of developing a specific treatment for myotonic dystrophy type 1.

STATE OF DEVELOPMENT

In vitro studies in cell cultures and *in vivo* studies in a mouse model have been performed.

APPLICATION

Pharmaceutical companies with clinical applications to improve pathologies such as myotonic dystrophy type 1.

INTELLECTUAL PROPERTY RIGHTS

Patent applied in Europe 22707100.8 on 18 September 2023 and in the United States 18/277,744 on 17 August 2023.

COLABORATION SOUGHT

Biotech or pharmaceutical companies interested in signing a licensing agreement or co-developing this technology.

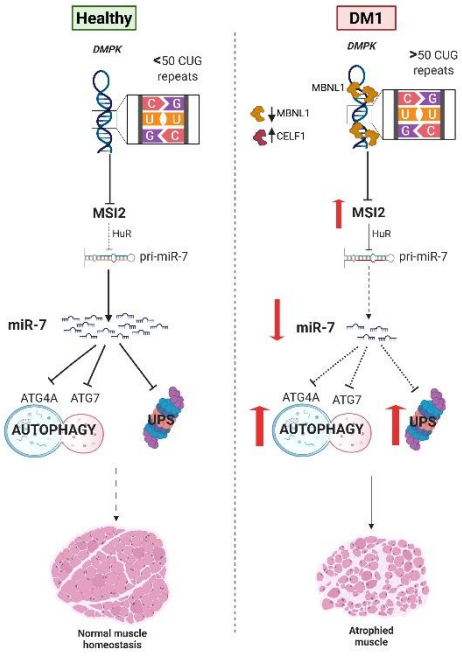

Fig. 1. Molecular mechanisms of MSI2 in healthy and diseased cells.

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