

MOXIFLOXACIN FOR SPINAL MUSCULAR ATROPHY TREATMENT

DESCRIPTION OF THE TECHNOLOGY

Spinal Muscular Atrophy (SMA) is a genetic neuromuscular disease characterized by a progressive loss of muscle strength. This is mainly due to the involvement of the motor neurons of the spinal cord, which means that the nerve impulse cannot be transmitted correctly to the muscles and the muscles eventually atrophy. The disease is caused by an alteration in the gene that encodes for the SMN (surviving motor neuron) protein. It is considered the second leading cause of neuromuscular diseases, with an prevalence of 4 affected per 100,000 people. Although SMA is considered a neuromotor disease, there are recent evidences that it affects not only muscles but also the brain, heart, pancreas, and even blood vessels.

The only therapeutic approach available for SMA patients is based on the use of oligonucleotides that increase the amount of functional SMN protein. However, the high cost of therapy, the type of administration (lumbar puncture) and the relatively modest improvement of patients are still important limitations. Therefore, the search for new effective drugs with the ability to be distributed systemically is an urgent requirement in the research of treatments against SMA.

INCLIVA-Universitat de València researchers led by Prof. Rubén Artero have identified a new candidate drug for developing a new therapy for SMA. This new approach consists on using moxifloxacin, a widely used antibiotic, as a treatment for SMA. Moxifloxacin has been found to significantly increase SMN protein levels, being a route of treatment for spinal muscular atrophy.

Moxifloxacin has demonstrated its efficacy as an antibiotic drug when it is administered as oral, injectable and ophthalmologic treatment; in such a way offering to SMA patients the possibility of a treatment less invasive than the one currently used. In addition, besides its lower cost, moxifloxacin would allow to develop a treatment where the drug distributed all over the body, which would contribute to palliate both the neurological effects and the other alterations that also occur in this disease.

APPLICATIONS

Pharmaceutical sector, as an active principle for the treatment of Spinal Muscular Atrophy.

ADVANTAGES AND BENEFITS

- Non-invasive administration: Oral, injectable, ophthalmic.
- Systemic distribution: It allows to treat, in addition to neurological signs, other problems associated with the disease.
- Lower cost compared to existing treatments.

STATE OF DEVELOPMENT

- The current technology has been developed using *in vitro* cultured cells (fibroblasts) from SMA patients.
- It is worthy to note that moxifloxacin is a drug authorized for commercialization, paving the way for developing this technology towards clinical phase.

INTELLECTUAL PROPERTY

Spanish Patent application filed in 2019 with a positive written opinion in the preliminary search report.

COLABORATION SOUGHT

- Biotech/pharma companies willing to license the technology.
- Biotech/pharma willing to collaborate in the development of the technological applications mentioned previously.

CONTACT

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