



Treatment of Spinal Muscular Atrophy using moxifloxacin

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 death of motor neurons of the spinal cord which results in the insufficient nerve impulse transmission leading to the muscle atrophy. The disease is caused by an alteration in the gene that encodes the SMN (surviving motor neuron) protein. It is considered the second leading cause of neuromuscular diseases, with a prevalence of 4 affected per 100,000 people. Although SMA is considered a motoneuron disease, there are recent evidences that it affects not only skeletal 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.

Universitat de València's researchers in collaboration with the INCLIVA Foundation have identified a new candidate drug for developing a new therapy for SMA. This new approach consists in using moxifloxacin, a widely used antibiotic, as a treatment for SMA. Moxifloxacin has been found to increase significantly SMN protein levels in various cellular disease models.

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 less invasive treatment than the one currently used. In addition, besides its lower cost, moxifloxacin would allow to develop a treatment where the drug is distributed all over the body, that would contribute to palliate both the neurological effects and the other alterations that also occur in this disease.

MARKET APPLICATION SECTORS

The main application of the technology is in the pharmaceutical sector, as an active principle for the treatment of Spinal Muscular Atrophy.

TECHNICAL ADVANTAGES AND BUSINESS BENEFITS

The main advantages of this invention are:

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

CURRENT STATE OF DEVELOPMENT

Moxifloxacin has been validated at laboratory, and tests are being carried out both *in vitro* and *in vivo* in animal models.

INTELLECTUAL PROPERTY RIGHTS

The technology is protected through the patent application P201930436 and PCT/ES2020/070311, entitled "Moxifloxacin for use in the treatment of Spinal Muscular Atrophy" with priority date 05/17/2019.

COLABORATION SOUGHT

- Pharmaceutical company to license.
- Subcontracting agreement with another company.
- R&D project to advance development.

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- Pharmaceutical company or investors to run clinical studies.

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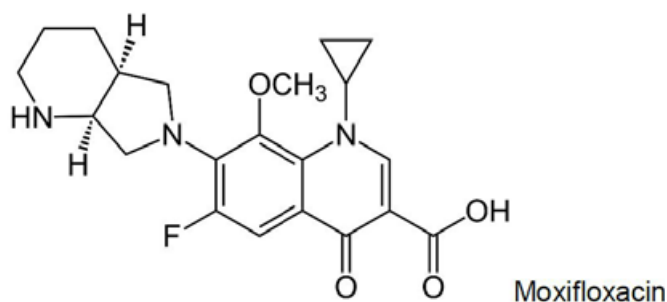


Figure 1. Moxifloxacin molecule.

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