

PHARMACEUTICAL COMPOSITIONS FOR THE TREATMENT OF CHARCOT-MARIE-TOOTH DISEASE

A research group from CIBER has developed a mitochondrial antioxidant and an antibiotic for use in the treatment of Charcot-Marie-Tooth disease

The Need

Charcot Marie Tooth (CMT) disease is a neuropathy for which there are currently no effective treatments.

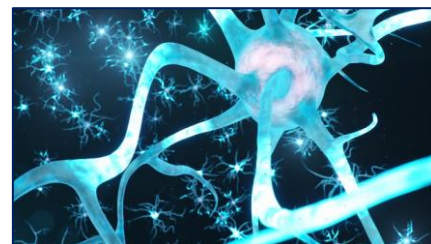
CMT is a rare disease, being the most common inherited condition of the peripheral nervous system, with an estimated prevalence of 10-28/100,000 inhabitants.

The Solution

The present technology provides pharmaceutical compositions for the preventive treatment of the symptoms of Charcot-Marie-Tooth disease. Specifically, two compounds, a mitochondrial antioxidant (MitoQ) and an antibiotic (Flofernicol).

Innovative Aspects

The compositions have no toxic effects. The main advantage of treatment with MitoQ is the reduction of symptoms related to motor deficiencies of the lower extremities. In addition, MitoQ has been shown to prevent the development of the associated phenotype and motor deficits associated with CMT disease in Gdap1^{-/-} mice. It is currently marketed as a dietary supplement for humans.



Flofernicol's beneficial effect is due to its lipophilic nature and its uncoupling activity which, on the one hand, increases mitochondrial respiration and decreases the production of mitochondrial reactive oxygen species (ROS). Its use has already been approved by the FDA for use in domestic animals.

Stage of Development:

Preclinical stage. In both cases, a significant increase in the motor skills of mice has been demonstrated.

Intellectual Property:

- Priority Spanish patent application filed for both compositions
- Suitable for international extension (PCT application)

Aims

Looking for a partner interested in a license and/or a collaboration agreement to develop and exploit this asset.

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