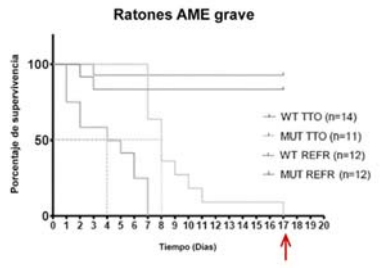
ENERGY AND ENVIRONMENTAL TECHNOLOGIES

Development of a therapeutic solution for Spinal Muscular Atrophy (SMA) focused to restore protein SMN levels in neurodegenerative disorder which primarily affecting motor neurons (MNs)



BUSSINESS OPORTUNITY

Technology available for licensing

IP STATUS

PCT application filed

TAGS

Spinal Muscular Atrophy (SMA), Survival Motor Neuron, Nerve Cells, muscle, Spinal Cord, Autophagy, Calpain

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NEW THERAPEUTIC CANDIDATE FOR SPINAL MUSCULAR ATROPHY

SUMMARY

Spinal Muscular Atrophy (SMA) is a genetic neuromuscular disease characterized by muscle atrophy and weakness. The disease generally manifests early in life and is **the leading genetic cause of death in infants and toddlers**. SMA is caused by defects in the Survival Motor Neuron 1 (SMN1) gene that encodes the SMN protein. The SMN protein is critical to the health and survival of the nerve cells in the spinal cord responsible for muscle contraction (motor neurons).

THE TECHNOLOGY

Researchers in the University of Lleida have **preclinical evidence for the positive effects of a calpain inhibitor** in in vitro and in vivo models for SMA (severe model and mild model for SMA).

When treated with this inhibitor, **mice survival is substantially prolonged and the clinical course of the disease is improved** by means of attenuating body weight loss and motor behaviour (righting reflex and tube test). The treatment increases SMN protein level in motor neurons and also prevents neurite degeneration in SMA mutants MNs.

THE MARKET NEED

Disease modifying therapeutic approaches are currently aiming to increase SMN protein levels, using gene therapy to add an exogenous copy of SMN, or protecting motor neurons from degenerating. However, there is no effective treatment for SMA, particularly for the most severe forms.

SMA has generally been believed to affect as many as 10,000 to 25,000 children and adults in the US, and therefore it is one of the most common rare diseases. One in 6,000 to one in 10,000 children are born with the disease. In Spain, there are around 60 new SMA diagnoses every year.

Spinraza© (Nusinersen, Biogen) is the only drug approved by the FDA and EMA, but there are other candidates in the pipeline. The SMA therapeutics pipeline is segmented into early stage candidates and late stage candidates under development, as Spinraza© long-term effects are still unknown.

ADVANTAGES

Treatment with a calpain inhibitor in vitro cultures of MNs and in animal models of AME is able to **improve survival**, the most apparent symptoms and the clinical course of the disease by **attenuating weight loss** and **improving motor behaviour**.

APPLICATIONS

The invention can be used for the treatment of patients who have already shown or who have yet to show symptoms of the disease.

LEVEL OF DEVELOPMENT

This project is currently in a preclinical stage of development. In vitro and in vivo assays have been performed with this potential therapeutic.