OPTOGENETIC PROTEIN THERAPY FOR MULTIPLE SCLEROSIS

www.optogenerapy.eu

CONSORTIUM:

THE PROJECT

Optogenerapy aims to develop and demonstrate a new optogenetics implant for controlled beta interferon (IFN-β) protein delivery for treating patients suffering from multiple sclerosis.

- Optogenerapy represents an innovative and effective therapeutic delivery with an impact on slowing the disease progression and increasing the Multiple Sclerosis patients' quality of life.
- The Optogenerapy IFN-β drug therapy is generated by cells confined within a chamber sealed by a porous membrane that allows the device to be easily implanted or removed.
- Implantation will require an ambulatory surgical procedure, with local anaesthetics. The practitioner will be able to regulate the drug delivery using an external controller module.

OPTOGENETICS

Optogenetics is the combination of genetics and optics techniques to control and monitor activities of cells in a living tissue with light. The Optogenerapy team will use optogenetics technology to develop an implant with light-sensitive cells with the mission to create and release IFN-β protein once activated by light.

MULTIPLE SCLEROSIS

Multiple Sclerosis (MS) is the most common demyelinating disease, in which the body's immune system attacks myelin, the substance that surrounds and protects the nerve fibres of the central nervous system, forming scar tissue and distorting or interrupting the nerve impulses travelling to and from the brain and spinal cord.

OBJECTIVES

TECHNOLOGICAL

1. To develop a miniaturized implant including: a cell chamber, a frame of biocompatible plastic optics and a membrane with a defined pore size designed for optimal protein therapeutic delivery.
2. To develop stable therapeutic cell lines having the interferon transgene under the control of a synthetic optogenetic pathway.
3. To develop a miniaturized wireless powered NIR light source.
4. To develop suitable in-silico modelling tools to assist in the therapeutic cell line development considering the optogenetic pathway activation and IFN-β delivery.
5. To develop an industrial micro-injection moulding process for manufacturing the minimally invasive implant.
6. To validate suitable sterilization protocols for the implant.
7. To prove biocompatibility and therapeutical efficiency of the new implant by *in vitro* and *in vivo* testing.

CLINICAL & OPERATIONAL

1. To prove a health gain due to continuous low dose drug delivery that will prevent relapses and will delay disease progression into neurological decline stages.
2. To define a clear regulatory path as a combined advanced-therapy medicine.

EXPECTED IMPACT

PATIENTS

• Improved drug effectiveness

The novel solution for IFN-β eliminates the serum levels peaks, the flu-like effects, and liver toxicity risks of the current treatment based on injections.

• Improved compliance with drug taking

The Optogenerapy solution will improve patients' treatment adherence as there is no need for daily to weekly self-injections. The novel treatment will help preventing patients to stop their medications by therapy fatigue and delaying disease progression.

SYSTEM

• Reduce direct and indirect costs linked to Multiple Sclerosis

Optogenerapy therapy deployment will save the costs of non-adherence to the healthcare system and to the society, mainly related to labour market productivity losses.

MORE INFORMATION

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SOCIETY

• Social awareness of efficient therapies improving patient compliance

The project's highly innovative concept and the real interest and capability to persist during the exploitation stages once the project ends will contribute to increase the social awareness about more efficient therapies with improved patient compliance. The project will also help to generate awareness about Multiple Sclerosis and its prevention measures, treatment options and adherence benefits.

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