Nano-ERT: Nano Enzyme Replacement Therapy to improve treatment for Gaucher's Disease

THE INVENTION

An innovative nanotechnological development that allows for an extended-release, more effective Enzyme Replacement Therapy (ERT) for Gaucher's Disease.

Innovative aspects and advantages

- Improved stability: 30-fold improvement in the enzyme's half life.
- Higher capacity to deliver to the most affected organs: it delivers up to 4 times more enzyme to the spleen and liver when compared to current ERT treatments.
- Reduced treatment frequency: representing significant savings over the patients' lifetimes, improving quality of life in patients as well as patient adherence to treatment.

IP Rights

- EP priority patent application in November 2022.
- Possibility to apply for Orphan Drug Designation.

Summary

Changes in the glucocerebrosidase (GBA) gene cause Gaucher Disease (GD), a lysosomal storage disease. Current standard treatment with ERT presents important shortcomings: due to the high instability of replacement GBA in the bloodstream, patients require intravenous administration every two weeks. This is also one of the factors that make GD ERT extremely expensive.

By conjugating the GBA enzyme to a polypeptide polymer, our researchers have been able to restore/enhance GBA activity while reducing GBA instability and expanding its half-life in animal models.

Additionally, current standard ERT only treats non-neurological symptoms. This conjugation may favor BBB crossing (as already demonstrated with other therapeutic compounds) which would make our therapy disruptive in terms of GD treatment.

Market size

- The GD treatment market was valued at USD 1.54 billion in 2021 and is expected to reach USD 1.88 billion by 2029, with a CARG of 2.50% (Data Bridge Market Research)
- The high cost of the treatment is an important access barrier.

We are looking for

A partner interested in a partnership, development collaboration and/or license agreement leading to the exploitation of the asset.

Scientific Team

Marta Martínez – Vall d'Hebron Institute of Research; Julia Lorenzo – Autonomous University of Barcelona ; María Jesús Vicent – Centro de Investigación Príncipe Felipe; Fernando Novio - ICN2.

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