# *Nano GBA Enzyme Replacement Therapy as a novel treatment for Parkinson Disease*

## THE INVENTION

**A new therapeutic strategy for Parkinson Disease (PD) based on the delivery of Glucocerebrosidade (GBA) to the brain, using a new nose-to-brain nanotechnology.**

## Innovative aspects and competitive advantages

- Our proposed treatment is **intranasally administered** to reach the CNS and **deliver pharmacologically active GBA to the brain.**
- GBA Enzyme Replacement Therapy is a **treatment approved for other indications for decades**, apt for pediatric and pregnant patients.
- There is a **lack of alternative therapies** to delay the progressive neurodegeneration in PD
- Possibly **relevant** non only for GBA-PD patients, but **also in idiopathic patients**.

## IP Rights

- EP priority patent application in November 2022.

## Summary

Parkinson's Disease is the most common neurodegenerative movement disorder. To date, only symptomatic treatments are available that cannot halt or delay the progressive death of dopaminergic neurons. Mutations in GBA gene are the main genetic risk factor for PD.

While ERT is successfully used to treat non-neurological symptoms for several diseases, recombinant proteins cannot bypass the blood-brain barrier, making RT ineffective to treat PD's symptoms.

Our researchers have developed a novel therapeutic strategy restoring neuronal GBA to avoid PD's neurodegeneration. By delivering GBA through a polypeptide-based carrier that can be intranasally administered, they have been able to reach the CNS and deliver pharmacologically active GBA to the brain in mouse models.

## Market size

- Around 6.2 million people suffer from PD globally. PD's patients are expected to reach close to 13 million people in 2040 (Parkinson Europe).
- The global PD treatment was valued at USD 4.28 billion in 2021 and is expected to grow at CAGR of 12.1% until 2030 (Grandview Research).

## 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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