

# Intravenous Enzyme Replacement Therapy for CNS Disorders

<https://www.neurodegenerationresearch.eu/survey/intravenous-enzyme-replacement-therapy-for-cns-disorders/>

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### Country

USA

## Title of project or programme

Intravenous Enzyme Replacement Therapy for CNS Disorders

## Source of funding information

NIH (NINDS)

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31/08/2015

## Total duration of award in years

1

## Keywords

beta-Glucosidase Stimulating Protein, Neuronopathic Gaucher Disease, enzyme replacement therapy, glucosidase, nanovesicle

## Research Abstract

? DESCRIPTION (provided by applicant): Central nervous system (CNS) disorders caused by enzyme deficiency are among the most devastating illnesses. For these, enzyme replacement therapy (ERT) is ineffective because enzymes cannot cross the blood brain barrier. There is a considerable medical need for innovative and non-invasive strategies to deliver functional enzymes into patients' brains, thus providing significant improvements in quality of life. In the

proposed research, we formulate proteolipidic nanovesicles that effectively transport functional enzymes into the brains of animal models with neuronopathic disease caused by defective lysosomal enzymes. CNS uptake of the nanovesicles occurs via a phosphatidylserine-mediated mechanism targeting inflamed cells in the brain. Our preliminary studies demonstrate that this intravenous enzyme delivery system has excellent selectivity towards inflammatory cells, a strong safety profile, and potential therapeutic properties to treat neuronopathic symptoms in animal models of enzyme deficiency (i.e. Gaucher disease and Gaucher-related synucleinopathies). Upon completion, the proposed studies are expected to provide a new low risk, cost-effective intravenous ERT for clinical CNS disorders resulting from enzyme or protein deficiency.

**Further information available at:**

**Types:**

Investments < €500k

**Member States:**

United States of America

**Diseases:**

N/A

**Years:**

2016

**Database Categories:**

N/A

**Database Tags:**

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