Universidad de Navarra
Facultad de Ciencias

MÁSTER EN INVESTIGACIÓN BIOMÉDICA
Research Project Proposal
Academic year 2026-2027

Project Nº 59

Title: *Strategies of mRNA repair for the treatment of Dravet syndrome*

Department/ Laboratory Gene Thereapy for Rare Diseases Department/Laboratory of gene therapy for genetic encephalopathies

Director 1 *Ruben Hernandez Alcoceba*
Contact: *rubenh@unav.es*
Codirector:
Contact:

Summary

Dravet syndrome is a genetic encephalopathy caused by mutations in the SCN1A gene, whose coding sequence comprises 6 Kb and is expressed preferentially in inhibitory GABAergic neurons. Gene supplementation is challenging for this disease and many other encephalopathies because the gold standard vectors for gene delivery into the brain (adeno-associated viruses, AAV) have a cloning capacity of 4.7 Kb.

We propose the development of mRNA trans-splicing technology (RTS) to overcome this hurdle. We will carry out a functional selection system for optimization of RNA structures in cell culture. The leading candidates will be incorporated together with segments of the SCN1A gene into AAV genomes. The vectors will be produced and validated in neuronal cultures. The final candidates will be administered to a mouse model of Dravet syndrome. Survival, epileptic, motor and behavioral phenotypes will be analyzed.

yes	X
no	

Does the project include the possibility of supervised animal manipulation to complete the training for animal manipulator?