Universidad de Navarra
Facultad de Ciencias

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

Project Nº 33

Title: Opening the door to renal gene therapy: treatment of ADPKD and FHHNC

Department/ Laboratory.
Renal Gene Therapy and Chimeric AAV Development Laboratory, Gene Therapy for rare diseases program, CIMA

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Summary

The kidney is a highly complex organ, and its dysfunction can give rise to a broad spectrum of diseases. In many cases, kidney transplantation remains the only curative treatment available. To date, the genetic basis of more than 160 kidney disorders has been identified, highlighting gene therapy as a promising strategy for the treatment of chronic renal diseases. However, a key challenge persists: the lack of efficient vectors capable of delivering therapeutic genes specifically to kidney cells.
This project is part of the TEGER strategic initiative, funded by the Government of Navarra, which aims to develop gene therapy approaches for autosomal dominant polycystic kidney disease (ADPKD) and familial hypomagnesemia with hypercalciuria and nephrocalcinosis (FHHNC).
The limited availability of adeno-associated virus (AAV) vectors with renal specificity has driven the development of AAV libraries through capsid engineering, generating a collection of variants with enhanced kidney tropism. These novel renal-targeted vectors represent a highly promising avenue for the development of the first effective gene therapy tools for kidney diseases. The project is structured around three main objectives:
1. To identify kidney-tropic AAV variants from the generated libraries for the treatment of ADPKD and FHHNC.
2. To develop therapeutic vectors for gene augmentation or gene editing strategies.
3. To assess the therapeutic efficacy of selected vectors in preclinical models of ADPKD and FHHNC.
The specific work undertaken during the Master's Thesis will align with one or two of these objectives, depending on the stage of the project at the time of initiation.
The methodologies employed will include molecular biology and cell culture techniques, generation of recombinant AAVs, and characterization of viral vectors using approaches such as PCR, Western blotting, and infectivity assays. Functional evaluation of therapeutic vectors will be performed in cultured cells, followed by renal delivery in mouse models. Treated animals will undergo comprehensive biochemical, histological, and molecular analyses. Statistical methods will be applied for rigorous interpretation of the experimental data.

yes	X
no	

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