Universidad de Navarra
Facultad de Ciencias

MÁSTER EN INVESTIGACIÓN BIOMÉDICA
Research Project Proposal
Academic year 2025-2026

Project Nº 01

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

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

Director 1: Rafael Aldabe Arregui
Contact: raldabe@unav.es
Codirector: Sergio Milagros Solchaga
Contact: smilagross@unav.es

Summary
The kidney is a highly complex organ, and its dysfunction can lead to a wide range of diseases. In most cases, the only curative treatment available is kidney transplantation. To date, the genetic basis of over 160 kidney diseases has been identified, positioning gene therapy as a promising strategy for addressing chronic kidney conditions. However, a major limitation remains: the lack of effective 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 to develop gene therapy treatments for autosomal dominant polycystic kidney disease (ADPKD). Project execution will be closely aligned with the activities carried out by other members of the consortium.
The platform being developed focuses on the creation of AAV (adeno-associated virus) libraries by genetic engineering of the capsid or chimeric AAVs.
These viral libraries represent a highly promising approach for developing the first gene therapy vectors capable of treating kidney diseases. The project is structured around several key objectives:
1. Engineering chimeric AAV vectors capable of delivering therapeutic genes to the kidney by targeting molecules with high renal affinity.
2. Screening and selecting kidney-tropic AAVs from the two types of libraries described.
3. Evaluating the therapeutic efficacy of the selected vectors in preclinical models of ADPKD.
The specific work carried out during the Master's Thesis will align with one of these objectives, depending on the project's progress at the time of initiation.
Methodologies involved in the project will include: molecular biology and cell culture techniques, generation and modification of recombinant AAVs using nanomaterials, characterization of the viral vectors (via PCR, Western blot, structural analysis, infectivity assays, etc.), renal inoculation in mouse models, and comprehensive biochemical, histological, and molecular evaluation of treated animals. Statistical analysis will be applied to interpret the experimental data.

yes	X
no	

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