Universidad de Navarra
Facultad de Ciencias

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

Project Nº 21

Title: *Development of novel gene therapy vectors for inherited renal diseases*

Department/ Laboratory *Next generation gene delivery technologies group, Gene therapy for rare diseases program, DNA&RNA medicine division, CIMA*

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Summary

Autosomal dominant polycystic kidney disease (ADPKD) is a disease of high frequency (1:1,000) mainly caused by mutations in the PKD1 gene (around 80% of the cases). There are no curative therapeutic approaches for this patients and gene therapy offers a promising alternative. However, delivering genes to the kidney has proven to be difficult due to its complex anatomy and function. This project aims to generate a novel gene delivery platform that efficiently transduces the kidney to enable the development of efficacious and safe gene therapies for ADPKD. For this, we will generate a large adeno-associated virus (AAV) vector library that contains millions of different AAV capsids that are barcoded and can be identified by next generation sequencing (NGS). This library will be administered in two mouse models: wild type mice and a mouse model of ADPKD. AAV barcode representation will be quantified in DNA and RNA extracted from treated kidneys by NGS and analyzed using bioinformatic approaches. The top variants found to highly transduce the kidney will be individually validated in mouse models. This project is innovative and very relevant for the generation of novel gene therapies for inherited kidney diseases. The student will learn different techniques, including advanced molecular biology (cloning, ddPCR, NGS, etc), vector production, vector administration in mice, mice handling, and bioinformatic analysis.

yes	X
no	

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