



Research Project Proposal
Academic year 2021-2022
Máster en Investigación Biomédica

Project Nº 05

Title: GENETIC DRONES: Development of chimeric gene therapy vectors to treat renal diseases

Lab 4.04. Gene Therapy and Regulation of Gene Expression. CIMA.

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Summary: This project is part of the platform "Genetic Drones" that we have created recently in order to develop gene therapy vectors to coat the viral vectors with nanomaterials, modifying vector specificity and selecting their target cell.

We have defined kidney as our first target organ as it is a complex organ with a large number of genetic diseases, including one of the monogenic diseases with highest prevalence (ADPKD), and there are no vectors with the capacity to transfer the therapeutic gene to the renal cells.

The main objective is the development of the first gene therapy vectors that can be used for the treatment of kidney diseases following several intermediate objectives:

1. Construct vectors based on AAVs to be able to transport therapeutic genes to the kidney.
2. Select molecules with a high affinity for renal cells.
3. Enhance AAVs renal tropism combining targeting capacity demonstrated by the selected molecules together with the ability of AAVs to transduce genetic material.
4. Validate the therapeutic potential of the developed vectors treating a preclinical mouse model of ADPKD.

The methodologies to be used will be: molecular biology and cell culture technique, production, purification and modification recombinant AAV virus with nanomaterials, characterization of the generated viruses (PCR, western blot, structure, infectivity, ...), renal inoculation of viruses and biochemical, histological and molecular studies of treated animals.

yes	<input checked="" type="checkbox"/>
no	<input type="checkbox"/>

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