



Research Project Proposal
Academic year 2019-2020

Project Nº 01_ASIGNADO

Title: *GENETIC DRONES: Development of renal gene therapy vectors*

Department/ Laboratory *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 with a defined tropism. This platform is based on the combination of virology and nanotechnology that will allow the nanomaterials to coat the viral vectors, modifying their specificity and changing 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, without vectors with the capacity to transfer the therapeutic gene to the specific diseased 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:

- Construct vectors based on AAVs to be able to transport therapeutic genes to the kidney.
- Select molecules with a high affinity for renal cells.
- Enhance the renal tropism of AAVs by combining the capacity of directionality demonstrated to target organs and / or cells by the selected molecules together with the capacity of gene transduction of vectors based on AAVs.
- Validate the therapeutic potential of vectors developed by treating preclinical models of renal diseases such as ADPKD.

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

yes	<input checked="" type="checkbox"/>	Does the project include the possibility of supervised animal manipulation to complete the training for animal manipulator?
no	<input type="checkbox"/>	