Universidad de Navarra
Facultad de Ciencias

MASTER'S DEGREE IN BIOMEDICAL RESEARCH
Research Project Proposal
Academic year 2024-2025

Project Nº 16

Title: Generation of a multiplexed AAV reporter library for rare disease and cancer gene therapy

Department/ Laboratory *DNA and RNA Medicine/ Laboratory of gene therapy for congenital hearing loss and AAV engineering*

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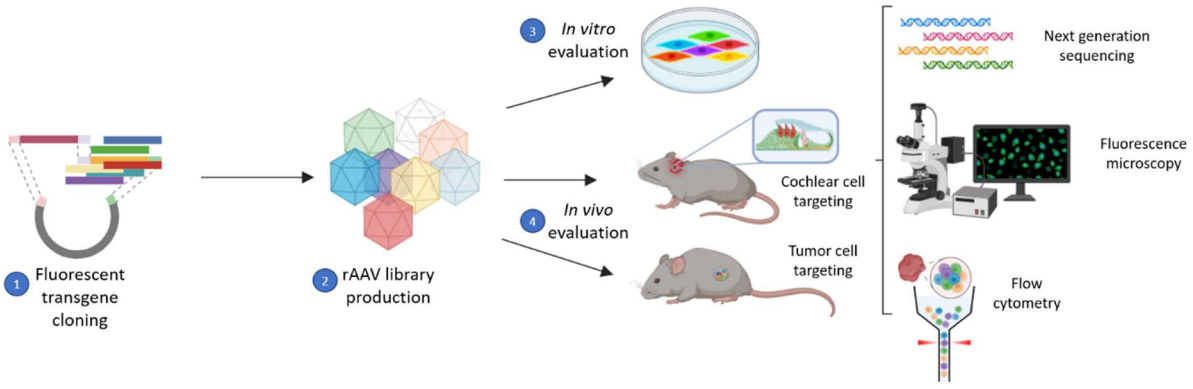
Summary *Short summary of the project with a **maximum extension of 250 words**, including the goals and the methodology that will be used*

Gene therapy is a medical technology that allows to treat the origin of genetic diseases by supplementing a missing gene, silencing a gene that is overexpressed, or editing the genome. Viral vectors are commonly used for delivering DNA payloads to the cells, among which recombinant adeno-associated viral (rAAV) vectors are the flagship vehicle for therapeutic applications. The payload can be a therapeutic gene to treat a genetic disorder or an immunostimulatory gene for cancer immuno-gene therapy. Importantly, there are many rAAV capsid variants that can be used for this type of applications and that would define the interaction of the vector with the target cell.

The **goal of this project** is to generate a rainbow reporter library for rAAV capsid screening in rare disease and cancer models. First, different fluorescent reporters with a unique barcode will be cloned in a rAAV plasmid and the multiplexed rAAV library will be produced. When the library is complete it will be tested in cell culture, both in a hearing loss and in a cancer cell line. Next-generation sequencing, flow cytometry and fluorescence microscopy will be used to evaluate the functionality of the library. The final step will be to validate the rainbow rAAV library in the mouse cochlea as well as in a mouse model of cancer.



yes	X
no	

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