Universidad de Navarra
Facultad de Ciencias

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

Project Nº 34

Title:

Targeting RNAi to the liver using aptamers for precision gene therapy in hepatocellular carcinoma.

Department/ Laboratory
CIMA, Enable Technologies, Aptamer Unit.

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Summary

RNAi therapy with non-viral vectors has shown promising results in preclinical and some clinical settings, but mainly restricted to few monogenic diseases. The use of a platform to improve the efficacy of delivery of genetic cargos without using immunogenic viral vectors might change the paradigm of treating many diseases. RNAi therapy is spearheaded in liver-associated diseases in part due to the successful delivery of genetic cargos to this organ using GalNac modifications to target hepatocytes. In a quest to expand the ligands to target genetic cargos to liver we select oligonucleotide RNA aptamers that binds to hepatocytes. The aptamers are internalized at high rate upon receptor engagement and show in vivo selectivity to liver tissue in mice. This aptamer represents an excellent tool to functionalize genetic cargos (e.g. RNAi, or ASOs) to improve targeted liver delivery for many diseases including hepatocellular carcinoma.

During the master's program, the student will become proficient in various technologies, including molecular biology techniques like cloning, RNAi, and aptamer technology, as well as cell and tissue culture, in vivo animal experiments, in vivo imaging in mice, flow cytometry, image confocal microscopy, and different bioinformatics pipelines for RNAseq and aptamer enrichment analysis.

yes	x
no	

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