Universidad de Navarra

Máster en Investigación Biomédica
Facultad de Ciencias

Research Project Proposal
Academic year 2016-2017

Project Nº 9

Title: Inducing tolerance against AAV to improve their therapeutic use in gene therapy

Department/ Laboratory: CIMA/ Dept**.** Terapia Génica y Regulación de la Expresión Génica/ Laboratory Terapia génica de enfermedades hepáticas, Center for Applied Medical Research (CIMA)

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Summary

Adeno-associated viruses (AAV) belong to the family of parvoviruses, which are linear, non-segmented single-stranded DNA viruses. Latent AAV-infection in humans is common but is generally not associated with disease. AAV are amongst the smallest viruses (about 25 nm in diameter), naturally replication defective and lack viral coding sequences. These properties make them ideal vehicles for gene delivery in gene therapy approaches. Although in general having a very low immunogenic potential, infection with AAV elicits the generation of neutralizing antibodies (nAbs), which poses a problem in cases where repeated administration is required to achieve an optimal therapeutic effect. Hence, strategies have to be developed to modify their surface in a way that renders the immune system unresponsive or 'tolerant'.

In this project we will address the immune response, in particular the production of antibodies, against novel AAVs with a variety of assays including ELISA, ELISPOT, and flow cytometry.

References

Mingozzi F and High KA: Immune responses to AAV vectors: overcoming barriers to successful gene therapy. Review. Blood, 2013, 122 (1) 23-36

POSSIBILITY OF PhD YES** (PhD grant required)