This project has received funding from the *European Union's Horizon 2020 research and innovation programme* under Marie-Sklodowska Curie grant agreement No 101034285

UNIVERSITY: University of Navarre (UNAV)

WIT PROGRAMME´S RESEARCH LINE NAME:

Advances Therapies and diagnostic innovation: Gene therapy for rare diseases.

DOCTORAL PROGRAMME: Doctoral program of applied medicine and biomedicine https://en.unav.edu/web/doctoral-program-of-applied-medicine-and-biomedicine

COMPLETE DESCRIPTION OF THE LINE

The main objective of our line of research is the study of liver diseases of genetic origin for which the only curative treatment is liver transplantation. These diseases that normally manifest in childhood are within the group of rare diseases and their consequences are devastating. In addition to working on trying to understand the molecular mechanisms involved in the development of some of these diseases, we are working on the development of new treatments. The therapeutic strategies we use are based on gene transfer and gene editing. Additionally, a very important line of work of our group is the development of more effective and safer gene transfer vectors, as well as the evaluation of methods that allow us to address some of the limitations associated with the use of viral vectors such as: the immune response and the loss of the therapeutic effect due to the growth or regeneration of the liver. In parallel, we are initiating a new line of research for the treatment of genetic diseases that affect other organs such as the ear or kidney.

RESEARCH GROUP NAME:

Wit
Welcoming
International
Talent

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Laboratory of gene therapy for liver diseases

COORDINATOR:

- Last and first name; link to the "Portal of scientific production":
  Dra Gloria Gonzalez Aseguinolaza

  https://orcid.org/0000-0002-1600-4562
  https://www.webofscience.com/wos/alldb/summary/5c5401ac-86e4-4a4c-b1dc-bf9b9139ddea-04479ee7/relevance/1

- Department: Gene Therapy and Regulation of Gene Expression
- Email: ggasegui@unav.es
- Telephone number: +34 948194700 x4024

MEMBERS OF THE LINE RESEARCH:

Dr Rafael Aldabe

Dra Oihana Murillo

Dra Mirja Hommel

Dr Daniel Moreno

Dra Cristina Gazquez

Dr Gracian Camps

Miren Barberia

Laura Torella

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Pedram Moeini

ANOTHER RESEARCH LINES OF THE GROUP:

Development of new vectors for gene and genome editing system transfer to the inner ear. The goal of this line is the development of treatments for hereditary deafness and hearing loss.

1. Development of new vectors for gene transfer to the kidney. Among the rare diseases the one with a higher incidence is polycystic kidney disease, due to the absence of adequate tools for the transfer of genes to the liver there are no gene therapy treatments for these diseases today, which is the final objective of this line of research.

- Entities involved in research lines and contact person:

✓ Academic entities:

Clínica Universidad de Navarra y Universidad de Navarra.

Hearing loss gene therapy

- Manuel Manrique y Gloria Gonzalez Aseguinolaza

Renal gene therapy

- Rafael Aldabe

✓ Industrial entities:

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Hearing loss gene therapy

- Viralgen. Cesar Trigueros.

Renal gene therapy

- Askbio. Jude Samulski.

- Joint supervision of doctoral thesis with international universities or non academic institutions:

  Dr David Salas Gómez; Joint supervision: University of Navarra-UniQure (Dutch gene therapy company)
  Irene Ros Gañan; Joint supervision: University of Navarra-Vivet Therapeutics (French gene therapy company)

- Group review

  The group of gene therapy for liver diseases began its journey in 2001 with the incorporation of Dr. Gonzalez Aseguinolaza as a Ramón y Cajal researcher. Although the group initially worked on the development of new therapies against viral hepatitis, in the last 12 years, it has focused on the development of gene therapy for monogenic liver diseases. This work has led us to conduct the first European clinical trial of gene therapy for a metabolic liver disease (acute intermittent porphyria, European project Aipgene) and to the creation of an international gene therapy company, Vivet therapeutics SAS. Our laboratory has stable national and international competitive funding and a large number of publications in high-impact journals. In our group, 18 new doctors have been trained who continue their research career in academic institutions or industry. We also have numerous national and international

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collaborations, and our group has been considered recently a reference group in gene therapy internationally (Human Gene Therapy, Apr. 2021).

- Link of the group to the "Portal of scientific production"

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  https://www.webofscience.com/wos/alldb/summary/5c5401ac-86e4-4a4c-b1dc-bf9b9139ddea-04479ee7/relevance/1

- Pictures, links… to academic or industrial partners (if any)
  https://www.vivet-therapeutics.com/en
  https://www.askbio.com/
  https://viralgenvc.com/