HEAL ITALIA

SPOKE N. 6: Genome editing through delivery systems for personalized medicine

GENERE: Genome Editing for the treatment of retinal deseases through nano-delivery systems

Project Leader
Dr. Antonia Romitelli

Proponent: Tecnobios
Duration: 11 months
Objective: Develop an innovative system for CRISPR/Cas9 delivery to treat retinal deseases

HEAL ITALIA

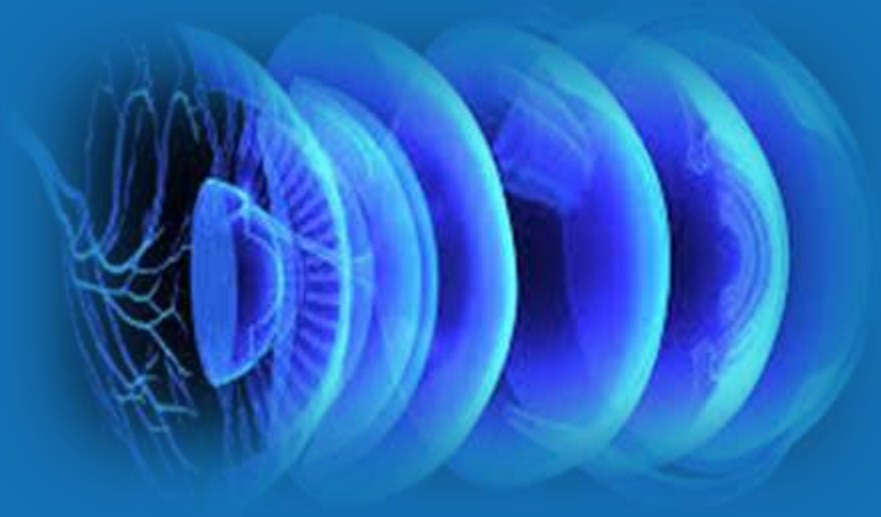
Tecnobios is a Campania-based company founded in 1989 in Apollosa (BN). It specializes in developing innovative therapies by combining clinical and translational research in the biomedical and pharmacological fields. The company has over 30 years of experience in research and development of innovative medical devices, formulations for ophthalmology, regenerative medicine, and in vitro

GENERE: Genome editing for the treatment of retinal deseases through nano-delivery systems

Retinal diseases, such as age-related macular degeneration (AMD), are one of the leading causes of irreversible blindness, primarily affecting the elderly population

The GENERE project aims to:

- Develop an innovative system based on the delivery of **CRISPR/Cas9** for the treatment of retinal diseases.
- Adopt a multi-omic approach to identify new biomarkers for monitoring the effectiveness of gene therapies.



Project Leader
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Innovation

- Development of an advanced delivery system to overcome ocular biological barriers.
- Targeted and controlled release of the **CRISPR/Cas9** system.
- Validation of efficacy and safety using a multi-omic approach.

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TECNOBIOS
LAB TO BUSINESS

Project structure and Key Phases

WP1
Months 1-4

Development and optimization of liposomal formulations

WP2
Months 4-8

Evaluation of genetic editing efficacy

WP3
Months 8-11

Validation through multi-omic analysis

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WP1
Months 1-4

Development of Liposomal Formilations

Objective: design and develop liposome formulations for the encapsulation and targeted delivery of CRISPR/Cas9 to retinal cells

Characteristics:
- Creation of innovative formulations such as LPH e LCP
- Optimization of particles and evaluation of physicochemical properties

Expected Outcome:
- Production of biocompatible and biodegradable nanoparticles
- Efficient and controlled release of CRISPR/Cas9

WP2
Months 4-8

Evaluation of Genetic editing Efficacy

Objective: Test and validate the efficacy of nano-delivery system in advanced cellular models, focusing on the genetic editing of the VEGF target gene in retinal cells

Characteristics:
- In vitro testing od formulations
- Genetics editing analysis using NGS sequencing and qPCR techniques

Expected Outcome:
Validation of nano-delivery system and confirmation of genetic editing

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WP3
Months 8-11

Velidation through Multi-omic Analysis

Objective: Validate the efficacy and safety of the CRISPR/Cas9 nano-delivery system using a multi-omic approach (genomic, transcrptomic, and proteomic)

Characteristics:

- Genomic and transcriptomic analysis (RNA-seq)
- Proteomic analysis and identification of efficacy and safety biomarkers

Expected outcome:

- Validation of the system under clinically relevant conditions
- Identification of new biomarkers to monitor therapy efficacy

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Expected Results

- **Validate a biocompatible and biodegradable liposomal nanoparticle system**
- Create a nano-delivery system for targeted CRISPR/Cas9 editing
- Identify efficacy and safety biomarkers

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Project Impact and Conclusion

Value for research and healthcare:

- Introduction of advanced nano-delivery systems for new perspectives in genome editing
- Development of targeted therapies for retinal diseases, particularly age-related macular degeneration (AMD)
- Promotion of precision medicine
- New perspectives for treatment

Project Leader
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Thank you

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