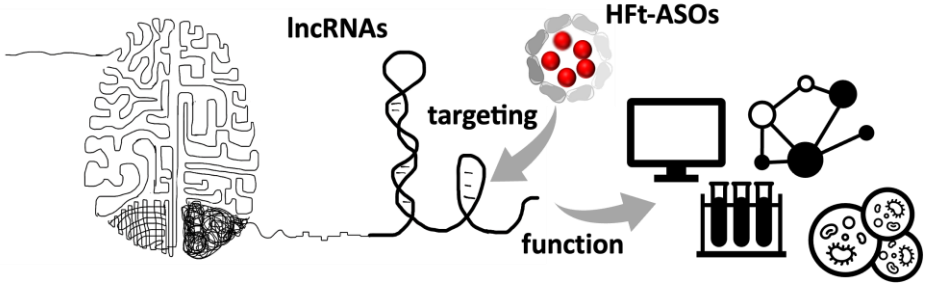
Project title: National Center for Gene Therapy and Drugs based on RNA Technology



Subproject: Targeting long noncoding RNAs in Group 3 Medulloblastoma through ferritin-based nanovectors to characterize functions and applications

Acronym: MEDFER

Partners:
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Description:
Regulatory noncoding transcripts, including long noncoding RNAs (lncRNAs), attract significant interest in pathophysiology due to their peculiar molecular, cellular, and functional properties. This aligns with a growing focus on their potential as oncological biomarkers, driver genes, and therapeutic targets. Our current work is dedicated to expanding knowledge and targeting tools for oncogenic lncRNAs we previously identified in the Group 3 (G3), the most aggressive and largely uncharacterized subtype of the cerebellar pediatric tumor Medulloblastoma (MB), a leading cause of cancer-related deaths in children worldwide.

Aims:
By leveraging expertise in molecular oncology and biochemical technologies, we will develop an innovative approach for targeting G3 MB lncRNAs. This will be achieved through human ferritin (HFt)-based nanocarriers engineered for the efficient delivery of RNA drugs, such as antisense oligonucleotides (ASOs) against pathological lncRNAs. With its unique properties, HFt holds great potential as a versatile platform for advancing anticancer strategies, with the ultimate goal of establishing an RNA-vs-RNA approach against G3 MB.

Expected results:
Customised ASOs, validated for suppressing the activity of the G3 MB antiapoptotic lncRNA MB3 will trigger the:
- Functional/mechanistic characterization of MB3 by -omics/molecular assays
- Analysis of synergistic interplays between MB3 targeting and chemotherapy treatments
- Design, production and biophysical characterization of novel HFt-ASO complexes
- Set-up of a HFt-mediated MB3 targeting strategy.

This plan will promote the understanding and tackling of deregulated noncoding pathways in G3 MB.

Funded by the European Union – Next Generation EU, M4C2 – CUP B83C22002860006