VEXOSOMES as stealth natural nanosystems to treat monogenic diseases

Currently, many monogenic diseases therapies are based on the use of adeno-associated viral vectors (AAV). However, these can be prematurely neutralized by the patient immune system after the first administration. In this context, an interesting improvement could be their coating with lipid components, such as extracellular vesicles (EVs). EVs could be advantageous in terms of being non-immunogenic and naturally targeted to the cell lineage they come from.

Therefore, VEXOSOMES, this is, engineered AAVs coated with EVs could become the next generation therapies of currently monogenic orphan diseases, such as muscular dystrophies. The main objective of this project is optimizing the efficient coating of AAVs with EVs to achieve they can be in vivo administered and arrive to their target organs without being previously cleared up by neutralizing antibodies.

This objective can be split up into:
1) Set up the methodology for the most efficient coating method.
2) In vitro study the tropism shift of the AAVs thanks to the EV coating.
3) In vivo studies of biodistribution of the engineered VEXOSOMES.

Responsible researchers: Dr. Cristina Fornaguera and Dr. Marta Guerra