Disease modelling and therapeutic prototyping using human iPS cells and programmable nucleases

The lab of nuclease enabled cell therapies at VU EMBL partnership institute for gene editing technologies, **Jonathan Arias (PhD) Principal Investigator**

The convergence between human iPS cells and gene editing technologies allow us to access cell types and genome configurations that are rare or normally inaccessible for disease modelling and therapeutic prototyping *ex vivo*. During this talk I will present developments for the creation of isogenic cell models using bi-allelic editing with CRISPR-Cas9 in a deterministic manner. I will discuss how such bi-allelic editing can be used to model neurodegeneration in Parkinson's disease and the early onset rare-disease ceroid lipofuscinosis. Furthermore, I will show how genetically encoded sensors enable the stratification of patients and facilitates compound screening with high-throughput and high-content systems.

About our lab: Our lab is located in Vilnius, Lithuania. We develop human cell models in hematopoietic linages (HSC and NK cells), ventricular cardiomyocytes, neuronal epithelial stem cells (NESCs) and skeletal muscle. If you need the creation of isogenic or reporter lines in human iPS cells feel free to contact us, we are happy to collaborate jonathan.arias@gmc.vu.lt
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