P47 BIOSENSORS AND CELLULAR MODELS DERIVED FROM PATIENTS: TOWARDS MOLECULAR CHARACTERIZATION AND PERSONALIZED MEDICINE

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Amyotrophic lateral sclerosis (ALS) is a fatal neurodegenerative disease characterized by motor neuron (MN) death that yields in progressive paralysis. Currently, drug development is hampered due to the heterogeneity of the disease and the lack of knowledge of the mechanism triggering selective MN death.[1]

Molecular profiling is an innovative powerful technology for unravelling complex molecular pathways that underlie physiological and pathological processes. Quantum dots (QDs) are luminescent nanoparticles with a high potential to become promising tools to detect molecular mechanisms at the subcellular level enabling multiplexing applications.[2] Currently, a wide-number of QDs linked to different biomolecules of interest, including antibodies, are commercially available, enhancing their use as fluorescent probes.[3] Using this technology, different ALS targets will be analyzed at the single-cell level in human cell models such as immortalized lymphocytes derived from ALS patients.

The scientific aim of this project is to explore molecular changes in key protein targets upon pharmacological treatment to help select therapeutic candidates with a molecular pathology modulation. Our group holds already promising therapeutic candidates from the treatment of ALS, such as IGS2.7, a CK-1 inhibitor that has reached preclinical phases. The molecular modulation of IGS2.7, has been studied using these tools, alone and in combination with Riluzol, the only drug approved by the EMA for ALS treatment, showing promising results.

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