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Clinical Neuroscience / iPSC and genome editing research

I joined the Cell Therapy Center (CTC) at the University of Jordan in May 2017, after finishing my DPhil at the University of Oxford / Nuffield Department of Clinical Neurosciences in March 2017. During my DPhil, I worked on iPSC and CRISPR-Cas9 gene-editing technologies to study motor neuron disease. I used CRISPR technology to correct a repeat expansion mutation in *C9orf72* gene and to understand the disease process after the correction of the disease-related mutation. We resulted in a successful normalization of the disease phenotypes and rescuing of the disease pathways.
After finishing my Ph.D. studies, I became interested in studying rare hereditary neuromuscular diseases (NMDs) that have not been studied thoroughly, because they are most common in the Middle East region and rare in western countries. Since I joined the CTC, I have established functional iPSC and CRISPR gene editing laboratories and started to produce iPSC from patients and healthy controls and differentiating them into different types of neurons. In our first study on NMDs, we have generated iPSC lines from healthy controls and ataxia oculomotor apraxia (AOA1) patients, to study the underlying disease mechanisms and to try to develop relevant therapeutic strategies. After the successful reprogramming of fibroblast cells, iPSC colonies were isolated, expanded, and characterized for their pluripotency and ability to express the key stem cell markers. Ultimately, the best three lines from each patient were selected for downstream work, differentiated into neural progenitors and then into motor neurons to study the underlying disease mechanisms.