NIHR Great Ormond Street Biomedical Research Centre

Doctoral Training Support Fund 2022

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Development of a 3D iPSC-derived cortical assembloid model of Dravet Syndrome

Dravet syndrome is a life-limiting, drug resistant epilepsy that presents in children during the first year of life. Currently there are no effective treatments to alter the long-term disease course. Advances in technologies now allow to make three-dimensional (3D) brain systems from patient's skin. For this project, we generated such patient 3D brain systems to better understand Dravet syndrome and to search for a cure. In order, to improve these systems, we want to incorporate a specific type of brain cells called "microglia", which are normally present in the developing human brain but not in the 3D brain systems traditionally used. We believe that making this improvement will give us insights on diseases associated with epilepsy, like Dravet Syndrome, and will help the development of new cures for these diseases. Eventually, our project aims to accelerate development of new gene-based treatments for Dravet patients, with the aim of translating our research findings to Dravet patients in the shortest possible timescale.