Long QT Syndrome, CPVT & ARVC: Personalized Medicine and Drug Development - Induced Pluripotent Stem Cells Derived Cardiomyocytes (BioRap)

Prof. Lior Gepstein's hiPSCs (human induced pluripotent stem cells) approach for modeling inherited cardiac disorders, such as Pompe glycogen storage disease, congenital long QT syndrome type II, CPVT and ARVC, has many implications in cardiovascular regenerative medicine, drug discovery, disease modeling, and optimizing patient-specific therapies

The ability to reprogram adult somatic cells into human induced pluripotent stem cells (hiPSCs) that could later be coaxed to differentiate into a variety of cell-lineages (including cardiomyocytes) opened new avenues for basic and translational cardiac research.
At Prof. Lior Gepstein's laboratory at The Rappaport Family Research Institute, recent efforts have been made to establish and coax the cardiomyocyte differentiation of patient-specific hiPSCs lines derived from patients with a variety of genetic (familial arrhythmogenic and cardiomyopathy syndromes) and acquired (ischemic cardiomyopathy) cardiac disorders. These include Pompe glycogen storage disease (due to a homozygous mutation in the lysyomoal enzyme acid alpha-glucosidase); the congenital long QT syndrome type II (due to a mutation in the KCNH2 gene); The latter three disease states may lead to the development of malignant ventricular arrhythmias and sudden cardiac death in otherwise healthy individuals.
The ability of the hiPSCs approach to recapitulate the in vivo disease phenotype in the dish, to provide novel mechanistic insights into disease pathogenesis, and to evaluate potential disease aggravators and novel customized treatment options has been established.
Finally, the potential applications of the iPSCs derived cardiomyocyte technology for drug toxicity screening (QT screening), for optimizing patient-specific therapy, and as a novel methodology for drug discovery is being further studied.

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