Long QT Syndrome, CPVT & Cradiomyopathies: Drug screening and toxicity testing by Induced Pluripotent Stem Cell-Derived Cardiomyocytes (BioRap)

An innovative platform for drug screening, toxicology testing and exploration of nevel means for treating severe cardiac pathologies, has been generated by Prof. Binah, focusing on inherited cardiac diseases such as Long Q-T Syndrome (LQTS), Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT) as well as a variety of cardiomyopathies (e.g., due to mutations in the myosin gene)

The cardiovascular system is a critical focus of drug screening when considering drug safety, since adverse effects on the heart might be not only chronic like other organs (e.g., liver), but can be life threatening. Much of the attrition rate of drugs in development is attributed to cardiotoxic side effects of the tested drugs, in humans, which were not apparent in preclinical animal models. This has become a significant problem facing the pharmaceutical industry. Recent advances in human stem cell biology have paved the way for the incorporation of human cell models into the two key aspects of developing new drugs: discovering new effective entities, and screening for their safety. Functional cardiomyocytes can now be derived from human pluripotent stem cells (hPSC), including both embryonic (hESC) and induced pluripotent (hiPSC) stem cells. Moreover, recent studies demonstrate the ability of cardiomyocytes derived from patients' iPSC to recapitulate the phenotype of several known cardiac diseases.

Professor Ofer Binah specializes in generating and investigating both embryonic and human induced Pluripotent Stem Cells (hiPSC)-derived healthy and diseases cardiomyocytes from patients with a large variety of inherited cardiac pathologies. These unique cardiomyocytes constitute excellent model systems for drug screening and testing new chemical entities. Furthermore, Professor Binah has generated iPSC-derived cardiomyocytes from patients with inherited cardiac diseases such as Long Q-T Syndrome (LQTS), Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT) as well as a variety of cardiomyopathies (e.g., due to mutations in the myosin gene). These latter cell lines can be effectively employed for the testing new treatment modalities of inherited cardiac diseases. To this end Professor Binah utilizes a diverse experimental system to fully characterize iPSC-cardiomyocytes cellular and functional properties.
These means include the patch clamp system, the Micro-Electrode-Array set-up, and a set-up for measuring intracellular calcium transients and contractions from cardiomyocytes.
In symmary, Professor Binah has the expertise and the means fpr comprehensive projects aimed at drug screening, toxicology testing, and exploring novel means to treat severe cardiac pathologies.

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