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Development of siRNA Based Therapeutics for Fabry Disease Using Patient Derived Kidney Organoid System

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Genetic or hereditary kidney disease is one of the significant causes of chronic kidney disease (CKD). With the advancement and widespread application of DNA testing in clinical practice, diagnosing genetic kidney diseases that were once challenging has become easier. Many cases previously classified as CKD of unknown cause are now increasingly being identified as genetic kidney diseases. However, there are clear limitations in researching disease pathogenesis and developing new drugs for genetic kidney disease due to the absence of suitable animal models and challenges in clinical research arising from the limited number of patients. Meanwhile, the discovery and utilization of human induced pluripotent stem cells (hiPSCs), along with the development of hiPSC-derived kidney organoids or other kidney cells, offer new avenues for kidney disease research and also provides new platform for new drug development. In this presentation, I will present the research findings on modeling various genetic kidney diseases, including Fabry disease, using patient-derived hiPSCs. Additionally, I will discuss the process of developing a new drug for treating Fabry disease, based on siRNA-lipid nanoparticle technology.

Keywords: hiPSC, Kidney organoid, fabry disease, siRNA, substrate reduction therapy