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Stem Cell-based Models for Hereditary Renal Disease

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The convergence of human pluripotent stem cell (hPSC) technologies and advanced genetic tools has revolutionized research on genetic kidney diseases. hPSC-derived kidney organoids serve as innovative in vitro platforms to model disease mechanisms, identify therapeutic targets, and test drug candidates. These organoids accurately replicate key features of various genetic conditions, including polycystic kidney disease, Fabry disease, and nephrotic syndromes, leveraging CRISPR/Cas9 for precise gene editing to elucidate pathophysiological pathways. The inclusion of vascular and stromal components has enhanced their complexity, although challenges such as limited maturity, vascularization, and immune cell integration remain.

Complementary advancements in the use of induced pluripotent stem cells (iPSCs) have enabled the generation of patient-specific organoids, furthering personalized medicine. These organoids may address the limitations of traditional models by recapitulating human kidney development and genetic disease phenotypes, facilitating high-throughput drug screening and gene-editing approaches in the future.