Gene editing of stem cells for kidney disease modelling and therapeutic intervention

Ricky Wk Lau
Monash University, Australia

Abstract:

Introduction: Recent developments in targeted gene editing have paved the way for the wide adoption of clustered regularly interspaced short palindromic repeats (CRISPR)-associated protein-9 nucleases (Cas9) as an RNA-guided molecular tool to modify the genome of eukaryotic cells of animals. Theoretically, the translation of CRISPR-Cas9 can be applied to the treatment of inherited or acquired kidney disease, kidney transplantation and genetic corrections of somatic cells from kidneys with inherited mutations, such as polycystic kidney disease. Human pluripotent stem cells have been used to generate an unlimited source of kidney progenitor cells or, when spontaneously differentiated into three-dimensional kidney organoids, to model kidney organogenesis or the pathogenesis of disease. Gene editing now allows for the tagging and selection of specific kidney cell types or disease-specific gene knock in/out, which enables more precise understanding of kidney organogenesis and genetic diseases. This review discusses the mechanisms of action, in addition to the advantages and disadvantages, of the three major gene editing technologies, namely, CRISPR-Cas9, zinc finger nucleases and transcription

activator-like effector nucleases. The implications of using gene editing to better understand kidney disease is reviewed in detail. In addition, the ethical issues of gene editing, which could be easily neglected in the modern, fast-paced research environment, are highlighted.

Biography:

Ricky Wk Lau is one of the renowned professor at Monash University, Australia

Recent Publications:

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