Application of CRISPR-Cas9 genome editing system in biomedical studies: recent progress and future perspectives

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Abstract

Introduction: Recent advances in genome engineering are starting a revolution in biological research. The clustered regularly interspaced short palindromic repeats (CRISPR) and its associated protein (Cas9) enables diverse manipulations of genome function. It has become a predictable and precise method of choice for genome engineering by specifying a 20-nt targeting sequence within its guide RNA. Regarding its simplicity, broad applicability, and high efficiency, CRISPR-Cas9 tool can be used in understanding of cellular mechanisms, generating animal models, correcting defective gene(s) causing diseases, cancer treatment, removal of bacterial/ viral infections, and in drug discovery. Moreover, the capability of CRISPRi to gene regulation without altering its function can be utilized for exceeding our knowledge of gene expression in prokaryotic and eukaryotic systems.

Results: In this lecture, the history and biology of CRISPR system were described firstly. Also, the applications of CRISPR-Cas9 in various ways, such as efficient generation of a wide variety of biomedical important cellular models as well as animal ones, modifying epigenomes, conducting genome-wide screens, labeling specific genomic loci in living cells, genome editing applications in xenotransplantation and endogenous gene expression regulation by an altered version of this system were reviewed. Moreover, we describe how this technology can be used as an antimicrobial or antiviral tool. However, this technology still needs optimization and will require a better understanding of how this system works on molecular level.

Conclusions: The broad applications of CRISPR-Cas9 technology in biological research will greatly advance our knowledge of basic biology as well as opening a door to treat a wide spectrum of human diseases.

Key words: CRISPR-Cas9, Genome editing, Applications, CRISPRi