Chapter 1
Genome Editing Technologies in Precision Medicine: Recent Innovations and Challenges

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ABSTRACT

Recent developments in genome editing technologies have revolutionized the field of genome precision medicine, offering unprecedented opportunities for genetic flaw correction, disease manipulation, and personalized treatment plans. These technologies include CRISPR-Cas systems, base editing, and prime editing, which can revolutionize treatments for monogenic disorders, cancers, and multifactorial complex diseases. However, challenges such as delivery bottlenecks, reactions, unintentional genetic changes, and ethical issues remain. The chapter emphasizes

DOI: 10.4018/979-8-3373-4857-5.ch001

the need for improved viral and non-viral vectors, specificity tests, and efficient regulatory methods. It also addresses socioeconomic and accessibility concerns for equitable access to genome editing treatments. The chapter aims to balance innovation and responsible translation, considering both preclinical and clinical advances and current issues in genome editing in precision medicine.

1. INTRODUCTION TO GENOME EDITING IN PRECISION MEDICINE

Modern medicine is no longer focused on a one-size-fits-all approach but on precision medicine that is focused on diagnostics, prognostics, and treatment of each individual patient. This paradigm recognizes the fact that genetic predisposition is at the center of disease susceptibility, progression and response to treatment. Precision medicine can be achieved by leveraging genomics, transcriptomics and proteomics to uncover the molecular pathogenesis of disease, which can be used to predict, prevent and treat disease uniquely. The new landscape has turned out to be genome-editing technologies. They enable accurate alteration of DNA in living organisms and cells. These tools can not only repair pathogenic mutations to their origin, but they can also verify disease-relevant genes and pathways by functional genomics to connect genetic diagnosis and targeted therapy. (Giamarellos-Bourboulis et al., 2024),(Chakraborty et al., 2024). The direction of such technologies has been characterized by increasing accuracy, effectiveness and accessibility. Zinc -Finger Nucleases (ZFNs) and Transcription Activator -Like Effector Nucleases (TALENs) showed that the targeted genomic modification became possible, introducing double strand breaks (DSBs) at a defined locus, which were then repaired using intrinsic cellular processes, including non-homologous end joining (NHEJ) or homology directed repair (HDR) shown in Figure 1(Afrose, Chakraborty, Bhowmick, et al., 2025; Chakraborty, Afrose, et al., 2025).Despite their groundbreaking step over previous, less specific methodologies, and the success of ZFNs and TALENs in the initial clinical uses, extensive use was limited by major drawbacks. The most significant of these were the complexity and high cost of protein engineering each new target and low predictable efficacy which hindered scalability to high throughput projects. With the identification and engineering of the CRISPR-Cas system, a prokaryote adaptive immune system re-purposed to specific genetic control in eukaryotic cells, the genome editing landscape was forever changed. The CRISPRCas9 system, specifically, has become groundbreaking due to its simplicity and range; the specificity of the targeting is defined by a short programmable guide RNA, which can be customized to match any sequence of interest in DNA, so no sophisticated protein engineering technologies are required.The high rate of CRISPR-based systems has