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Gene Technology

Commentary

Human Disease Using *CRISPR* Gene Editing and Gene Addition Techniques

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DESCRIPTION

The creation of genome editing techniques, which are based on bacterial nucleases, has made it possible to directly target and alter the genomic sequences in practically all eukaryotic cells. By encouraging the development of more precise cellular and animal models of pathological processes, genome editing has increased our ability to understand how genetics contributes to disease. It has also started to demonstrate extraordinary potential in a wide range of fields, from basic research to applied biotechnology and biomedical research. The transition of gene editing from theory to clinical application has been substantially accelerated by recent developments in programmable nucleases, such as Zinc-Finger Nucleases (ZFNs), Transcription Activator-Like Effector Nucleases (TALENs), and *CRISPR*-Cas-associated nucleases. Here, we examine the three main genome editing technologies' most recent developments the emphasis on eukaryotic cells and animal models to examine the uses of their derivative reagents as gene editing techniques in a variety of human disorders and potential future therapeutics. Finally, the summary of clinical trials using genome editing platforms to treat diseases and some of the difficulties encountered when employing this technique.

The rapid advancement of genome editing in recent years has changed the study of the human genome, allowing researchers to gain a deeper understanding of how a single gene product contributes to an organism's condition. Genome editing entered a new frontier in the 1970s with the introduction of genetic engineering. Over the past ten years, genome editing technologies have advanced at a rapid rate and started to demonstrate amazing applicability in a variety of domains, spanning from fundamental research to applied biotechnology and biomedical research. These technologies are based on manmade or bacterial nucleases. Delivering the editing machinery in situ, which effectively adds, deletes, and corrects genes as well as carries out other highly focused genomic alterations, allows for genome editing. Nuclease-induced Double Strand Breaks (DSBs), which stimulate very effective cellular DNA recombination mechanisms in mammalian cells, are the precursor of targeted DNA changes. One of the two main processes that practically all cell types and creatures use to repair nuclease-induced DNA DSBs is Homology-Directed Repair (HDR), which leads to targeted integration or Non-Homologous End-Joining (NHEJ), which disrupts genes, respectively.

The method for achieving targeted gene addition, replacement, or inactivation has historically been Homologous Recombination (HR), which uses undamaged homologous DNA fragments as templates. However, the utility of HR is severely constrained by its inefficiency in mammalian cells and model organisms. Targeted nucleases have been developed as an alternative method to boost the effectiveness of HDR-mediated genetic modification when it was shown that DSBs might increase the incidence of HDR by numerous orders of magnitude. An exogenous DNA template homologous to the break site sequence may be used by HDR to reconstruct the cleaved DNA once a specific DSB has been created. By directly sending an adequately created repair template into the targeted cells, this technique can be utilized to introduce precise mutations, leading to mutation correction or novel sequence insertion in a site-specific manner. In contrast, NHEJ-mediated repair frequently produces errors because it effectively creates gene insertions or deletions of various lengths at the DSB site, which ultimately results in gene inactivation.

The unique Zinc-finger nucleases (ZFNs) or mega nucleases has been the research emphasis in the early stages of genome editing in order to induce the desired DSBs at each specific DNA target site. To create artificial proteins with sequence-specific DNA-binding domains that could be customized and each attached to a nonspecific nuclease for target cleavage, these nuclease systems required specialized expertise, giving researchers unheard-of powers for genetic manipulation. Transcription Activator-like Effectors (TALEs), which are bacterial proteins with catalytic domains, have opened up new opportunities for precise genome editing. TALE-based programmable nucleases have a comparatively high frequency of cleaving any desired DNA sequence. The main issues with Transcription Activator-like Effector Nucleases (TALEN) techniques, however, are their ineffectiveness at efficiently screening the genomes of

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successfully targeted cells and the construction of sophisticated molecular clones for every new DNA target.

A powerful gene editing tool that was just recently found is called CRISPR-associated nuclease and it comes from a bacterial adaptive immune defense mechanism. This method has emerged as a viable substitute for ZFNs and TALENs to induce targeted genetic alterations since it can be effectively programmed to alter the genome of eukaryotic cells *via* an RNA-guided DNA cleavage module. The adaptable CRISPR technology has rapidly expanded its usage in modifying gene expression since 2013, when it was initially used in mammalian cells as a tool to edit the genome. This use includes everything from genomic sequence repair or alteration to epigenetic and transcriptional modifications.

The development of programmable nucleases has significantly sped up the process of gene editing from theory to clinical application and given scientists an unmatched power tool to manipulate practically any gene in a number of cell types and species. Genome editing is now being studied in preclinical studies largely for viral infections, Cardiovascular Diseases (CVDs), metabolic disorders, immune system abnormalities, hemophilia, muscular dystrophy, and the creation of T cell-based anticancer immunotherapies. Some of these techniques are currently undergoing phase clinical trials after moving beyond preclinical research. Here, we discuss applications of their derivative reagents as gene editing tools in various human diseases and in promising future therapies, with a focus on eukaryotic cells and animal models.