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CRISPR-Cas gene-editing to Disease Treatment in Clinics

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INTRODUCTION

Clustered regularly interspaced short palindromic repeats (CRISPR)-associated systems (Cas) are effective methods for focusing on particular genes for use in biotechnology, agricultural engineering, laboratory research, and the treatment of human disease. The most widely used gene-editing nuclease, Cas9, has showed considerable promise for the treatment of malignancies, viral infections, genetic illnesses, and other conditions. According to recent research, certain other CRISPR-Cas system varieties may also have the unexpected promise to join the fight as gene-editing tools for a variety of purposes. Despite the quick advancements in basic studies and clinical trials, numerous fundamental issues such as editing effectiveness, relative delivery difficulty, off-target consequences, immunogenicity, etc. present ongoing, major obstacles. Many people who suffer from diseases that are currently incurable may have great hope for therapeutic therapies thanks to CRISPR-Cas systems, a potent gene-editing technique.

For manipulating genomes, this technology excels thanks to its simple design, low cost, quick turn-around time, and, most importantly, its high precision and efficiency. As a result, CRISPR-Cas systems outperform earlier gene-editing technologies like transcription activator-like effector nucleases (TALENs) and zinc-finger nucleases (ZFNs) due to their numerous advantages. CRISPR-Cas technologies have been used by scientists in recent years to modify the genomes of practically all creatures, including human cells, primates, mice, zebrafish, *Bombyx mori*, and small microbes. These initiatives show that the CRISPR-Cas system has created a fresh preclinical and clinical research pathway for the treatment of a variety of difficult diseases.

There are various preclinical/clinical studies employing the CRISPR-Cas system to reverse the underlying genetic causes of a number of monogenic human genetic illnesses that affect people. Additionally, the CRISPR-Cas repair of genetic codes may help some disorders with numerous or complex genetic alterations. The CRISPR-Cas system has also been developed to realise its potential as a workable therapy option for various malignancies, autoimmune diseases, and infectious diseases. The first ex vivo clinical trial using Cas9-edited cells to treat cancer was authorised in 2018. The Food and Drug Administration approved the first in vivo clinical trial in 2019. Despite the potential for therapeutic applications, a number of obstacles still need to be overcome before CRISPR-Cas technologies may be used to treat patients with a variety of disorders.

The most lethal pandemic in our lifetime, the coronavirus disease 2019 (COVID-19), which is caused by the severe acute respiratory syndrome coronavirus-2 (SARS-CoV-2) and was initially identified in late 2019, has affected more than 215 nations or regions worldwide.

The CRISPR-Cas systems were created as quick and accurate SARS-CoV-2 diagnosis tools. CRISPR-based SARS-CoV-2 diagnostic detection may be less expensive, more precise, more sensitive than the standard molecular diagnostic technique reverse transcription-quantitative polymerase chain reaction (RT-qPCR), and it does not require sophisticated instruments. The possibility for quicker detection and practical usage at the point-of-care is thus present with this unique technique. By removing or lowering RT-potential qPCR's for producing false negative, false positive, or unclear results, CRISPR-facilitated detection may result in tests that are more accurate.

Additionally, COVID-19 has undergone testing of CRISPR-Cas systems for therapeutic use. Such a CRISPR-Cas13-based method is PAC-MAN (prophylactic antiviral CRISPR in human cells), which eliminates the SARS-CoV-2 virus using the RNA-guided RNA endonuclease activity of Cas13d in human cells. This study specifically shown that the CRISPR-Cas13d system may be employed in lung epithelial cells to successfully target and cleave the RNA sequences of SARS-CoV-2 fragments. Additionally, bioinformatics analyses showed that as less as 6 crRNAs can target 91% of 3051 sequenced coronaviruses, which may help us prepare for upcoming coronavirus pandemics. PAC-MAN is a solid evidence for antiviral strategy, and the CRISPR-Cas13 system may be an alternative therapeutic approach for COVID-19, especially with improved versions of effective, secure and dependable distribution methods. A mechanism for in vivo delivery is necessary for the therapeutic use of CRISPR-Cas13 systems in treating COVID-19, and PAC-MAN must be verified in pertinent preclinical models, including *rhesus macaques*, to assess its antiviral activity, specificity, and Cas13d-induced immunogenicity. A possible method to combat COVID-19 and upcoming viral threats is PAC-MAN.

The ideal gene treatment should be affordable, straightforward, targeted, quick, portable, simple to use, safe, and extremely effective. The CRISPR-Cas systems have quickly become the most popular method for precise gene editing and are regarded as some of the best for therapeutic gene editing for a variety of disorders. Due to the aforementioned difficulties and barriers, there is still a long way to go before many anxiously anticipating patients with diseases that are currently incurable can receive therapy. Our toolbox is being expanded as more CRISPR-Cas systems are discovered. CRISPR-based technologies have indeed demonstrated some improved outcomes to treat or alleviate disease as an unique genome-editing strategy. Future studies are undoubtedly required to create the necessary standards for CRISPR-based toolkits to be utilised in clinical settings. The best method for successful gene repair, precise

expression in the right tissues, and minimum off-target consequences and immunogenicity. The standard of clinical care for diseases using those authorised medications and therapies must also be reached before CRISPR gene editing techniques can be used. We can expect that the CRISPR-Cas system will one day realise the great potential to ameliorate or cure a wide range of human diseases in the future, given the numerous advantages of CRISPR-Cas technology for modifying genomes over other previous methods and the enthusiastic efforts of scientists from all corners of the universe.