Editorial

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CRISPR Inspires New Hope to Disease Sufferers

Pushpanathan Muthuirulan[1*] and Vaishnavi Gururajan[2]

[1]Laboratory of Gene Regulation and Development, National Institute of Child Health and Human Development, National Institutes of Health, Bethesda, Maryland, USA

[2]Christ University, Bengaluru, Karnataka, India

[*]**Corresponding author:** Pushpanathan Muthuirulan, Laboratory of Gene Regulation and Development, National Institute of Child Health and Human Development, National Institutes of Health, Bethesda, Maryland, United States, Tel: +1301-674-3108; E-mail: pushpanathan31@gmail.com

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The discovery of CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats)-based gene editing system led to revolution in molecular biology, opening doors for myriad subsequent discoveries in the field of therapeutics for prevention of human diseases. CRISPR technology is a simple yet very powerful, programmable molecular scissors enable the scientist's to alter an organism's nucleic acid sequences in a much faster, cheaper and more efficient ways. CRISPR-based technologies have gained increasing attention in recent years due to its unique ability to precisely edit nucleic acid sequences within any living cells or animal models [1]. These technologies have strengthened our knowledge of understanding about pathologies associated with wide variety of human diseases including singe-gene disorders such as cystic fibrosis, hemophilia and sickle cell diseases. This technology also holds promise to transform the development of therapies to permanently cure more complex human diseases involving genetic components such as cancer, heart diseases, mental disorders and infectious diseases [2,3].

Till date, several molecular tools have been developed to edit nucleic acid sequences [4]. However, CRIPSR-cas9 and CRIPSR-cas13 provide a more efficient ways of precisely editing DNA and RNA sequences, respectively. CRISPR-Cas9 system is based on bacterial antiviral immune mechanism first discovered in *Streptococcus thermophilus*, wherein the Cas9 endonuclease is directed to cut any DNA sequence of choice in the genome. The simplicity, high efficiency and versatility of CRISPR-Cas9 system makes this technology more amenable for application in genome editing [5]. Recent developments with Cas13 endonuclease led to CRISPR-Cas13 system that can knockdown messenger RNA of choice with similar efficiency to RNA interference technology [6]. Both these technologies would allow researchers to successfully achieve targeted nucleotide alterations in the genome or transcriptome. For successful treatment of human genetic diseases, editing the genome to permanently fix mutation may be more desirable using CRISPR-Cas9 system. Alternatively, CRISPR-Cas13 system can be used under certain circumstances that require short-term changes in gene expression without causing permanent alterations to the genome including temporary reduction of inflammation and treatment to acute diseases.

CRIPSR-Cas9 has proven to be versatile tool for genomic research. Genome-wide screening using CRIPSR-Cas9 mediated mutagenesis approach has proven to be useful in identifying novel gain-of-function and drug resistant alleles in disease related signaling pathways [7]. Besides this, CRISPR-cas9 technology has wide range applications including gene silencing, homology-directed repair to fix mutations, transient activation of endogenous genes, pooled genome-scale knockout screening and functional testing of disease variants [8-10]. Recently, researchers have successfully tested CRIPSR-Cas9 gene editing technique towards repairing fatal mutation (MYBPC3) causing heart disease using viable human embryos [11]. This approach also has immense promise in cancer research, for instance, it is being used to program immune cells for enhanced killing of cancer cells [12]. The appeal of using CRISPR-cas9 as an antimicrobial drug in the treatment of infectious disease would replace conventional antibiotics in near future and would benefit treatment by specific killing of pathogens while leaving beneficial microbes intact in human body [3].

The immediate power of CRISPR-Cas13 system as a molecular tool in introducing specific changes into RNA molecules would benefit the researchers in understanding the common forms of RNA processing mechanisms such as alternative splicing, translation and RNA editing in many disease conditions. This technology has also been used in eliminating faulty RNA's up to an efficiency of 95%. In the new study, Zhang and his colleagues have used RNA-guided RNA-targeting CRISPR–Cas effector Cas13a to reduce RNA expression of genes associated with cancer. This technology also leveraged programmable tracking of RNA transcripts in live cells using exogenous tags [6]. The biggest challenge now in CRISPR approach is delivering the CRIPSR machinery into tissues of the human body. But, researchers have been currently working on delivering CRISPR components to different tissues of the body to fight or prevent human diseases.

The use of CRISPR approach revives ethical concerns with humans, which takes into account of human risk assessment, genome editing in germline, safety issue with efficient delivery of CRISPR components into cells and its implications in genetic enhancement [13]. The technical challenges and ethical concerns associated with CRISPR approach have raised caution flags to this technology, which requires scientific community and stakeholders to engage in thoughtful discussion in moving this technology forward for potential therapeutic application. We are nearly ready to use CRISPR to target far more complex diseases in human. Editing genetic contents within specific cells of the body would allow us to treat far more genetic disorders in human. Thus, CRIPSR approach could revolutionize medicine, allowing us to treat or even permanently cure a wide range of genetic disorders. Altogether, CRISPR approaches are showing great promise, suggesting the technique's potential in treating complex human disorders and bringing in new hopes to disease sufferers.

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