Application of CRISPR/Cas Systems in Medicine

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**Abstract**

Genome editing is one of the most challenging area in order to provide more efficient ways to create a meaningful change in the genome. Drug development and design is one of the most important scientific procedures but nowadays most of the medicines cause side effects due to their non-specifity to the target cells. So the CRISPAR-Cas system will not only identify the target cells but it will also make sure the effective delivery of drugs to those cells. Therefore in this paper the application of CRISPR-Cas system in medicine will be discussed in detail.

**Introduction**

 During the past few years, new technologies have changed the field of medicinal sciences. Medicinal sciences are quite important for healthcare and it also helps in improving the lives of individuals. Gene targeting is a technique that was used by scientists from so many years in order to introduce new changes into the particular site in the genome by adding or completely removing whole genes or single bases.

Additionally, researchers are also using various technologies which are mainly derived from Prokaryotic Immune system 1. Systems like clustered interspaced short palindromic repeat (CRISPR) and all its linked proteins(Cas) are some of the most reliable tools for gene editing recently. CRISPR is clustered regularly interspaced short palindromic repeats and CRISPR-associated protein 9.

Development of CRISPR was a major improvement in genome editing. It is a genetic manipulation tool that is extracted from the defense system of specific bacteria. This method has been successfully used in cell lines, laboratory animals as well as in plants. The system works by directing the Cas9 nuclease to make a site‑directed double‑strand DNA. This is made under the guidance of a small RNA molecule. This technique has been taken from the bacterial immune system, it has been adopted by researchers all over the world and is applied to target important genes in various organisms and cell lines which include bacteria, rabbit and mice 1.

**Discussion**

 Although most of the researchers are interested in CRISPR-Cas system for its potential of treating various diseases including cancer with a genetic basis but recently it has also been proved that CRISPR-Cas system has a big part in drug discovery. This type of gene editing has a direct effect on drug discovery and also development. The CRISPR-Cas system helps in identifying target molecules which is an important step in the development of medicines 2. This system is used to activate and inhibit certain genes which helps the researchers to determine the proteins or genes which cause or prevent disease, as a result targets are identified for the potential drugs. This is also helpful in creating a whole animal model system which helps to mimic disease, it enables the researchers to determine the efficacy and also the accuracy of the medicines which helps to predict the impact of the medicines 3.

The system is also successful in discovering the novel targets for cancer therapy. It dissect chemical-genetic interactions and allow the study of different medicines. The study of this system show the response of tumours to drug treatment.

**Literature :**

 For plant genome modifications different techniques have been used. These allow the gene expression regulation at specific sites and allow the discovery of plant functional genomics. It is different from genetic engineering as it is the expression regulation at specific sites and does not use random mutagenesis or low-efficiency gene targeting. With this, these alterted palants cannot be distiinguised from the parents as there is no use of any foreign DNA. Previsouly, there was the use of random mutagenesis or low-efficiency gene targeting. Among the different techniques of genome editing, the versatile genome-editing tool CRISPR (Clustered regularly interspaced short palindromic repeats) is a relatively specific method for DNA modification.

 After its into mammalian cells , this technique has successfully been used in different fields of medical research. Scientists are using this for disease modelin, therapeutic explorations as well as genetic screening. They are using this for the best results that it delivers.

**Terms used:**

**CRISPR** :Clustered regularly interspaced short palindromic repeats and CRISPR-associated protein 9.

**DNA:** DeoxyRibonucleic acid

**RNA:** Ribonucleic acid

**Genetic engineering:** it is the use of biotechnology for the direct manipulation of an organism's genes.

**Databases searched:**

Different databases have been searched to understand the appalicaiton of CRISPR. Different terms were used for this purpose such as gentric engineering, CRISPR and CRISPR/Cas9.

**Applications:**

CRISPR/Cas9 is used in detecting the specific sequences of DNA and RNA. These are then used for research and diagnosis purpses. There is a use of heat and formamide to denature double‐stranded DNA (dsDNA) for this puspose. These allow the use of probe hybridization. But these can also affect the biological structure of the organisms. 1

Moreover, these can also be used to check the mutations and signle nucleotide variants (snvs). It has shown the great performance in site‐specific gene targeting. There is a use of this technique in HIV treatment, which is a main risk to global human health. There can be the use of highly active antiretroviral therapy (HAART) but these can only hold back virus replication and cannot eliminate latent viral reservoirs in HIV-1/AIDS patients. Tovercome this difficulty, there si a use of CRISPR)/CRISPR-associated nuclease 9 (Cas9). The use of (CRISPR)/CRISPR-associated nuclease 9 (Cas9) system has served as an effective gene-editing technology. The prociudre includes the targeting of cellular co-factors or HIV-1 genome. This result in the reduction of HIV-1 infection. This in return clears the provirus and cause transcriptional activation of latent virus.

Lentivirus are used for the activation of host restriction by CRISPR/Cas9. These have greater transduction competency and are able to easily prordcue virus. Moreover, specific sgRNAs can also be used for the activation of target gene.

**Results:**
 Genome engineering has been used as one major field in the study of biomedical. Genome engineering is the part of genetic engineering that allow the addition, removal and modification of genes in living cells. Now it has been used as the economical and practical technogly. Many unattained aims have been now achieved with this.

**Conclusion**

Scientists with genome editing can change an organism's DNA. They can not only add but also remove and change the genome at specific locations in the genome. They use different approaches for this purpose. One is the use of CRISPR-Cas9 whisch is not only faster but also more accurate as compared to existing genome editing methods. On the basis of the discussion above it is safe to say that the application of CRISPR/Cas Systems in Medicine is quite vast and it will help in designing drugs that will be target specific and also efficient. CRISPR)/CRISPR-associated nuclease 9 (Cas9) system has sucessfuly been used in HIV-1/AIDS prevention and drop in human cells and animal replica. This is also helpful in case of plants. Ecological and evolutionary genetics are using this for different purposes. The reason behind the use of this is its abilty to instantaneously generate modification to genes. In this process it does not create any t-DNA insertion. The promising nature of this technoglky is enforing scititiits to study it in details and allow the use of this technogly for its all potential results and outcomes.

**References**

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