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Genome engineering using CRISPR

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Summary

Genetic engineering, also called genetic modification, is the direct manipulation of an organism's genes using biotechnology. It is a set of technologies used to change the genetic makeup of cells, including the transfer of genes within and across species boundaries to produce improved or novel organisms. New DNA is obtained by either isolating and copying the genetic material of interest using recombinant DNA methods or by artificially synthesizing the DNA. A construct is usually created and used to insert this DNA into the host organism.[1] Now, a new revolution has seized the scientific community. Within only a few years, research labs worldwide have adopted a new technology that facilitates making specific changes in the DNA of humans, other animals, and plants. This technology is referred to as "CRISPR," and it has changed not only the way basic research is conducted, but also the way we can now think about treating diseases.[2] Now, researchers use the same CRISPR strategy to take on threats like diseases. CRISPR can turn genes on or off, or make them work in a different way, to protect your health.[3]

Introduction

CRISPR is an acronym for Clustered Regularly Interspaced Short Palindromic Repeat. This name refers to the unique organization of short, partially palindromic repeated DNA sequences found in the genomes of bacteria and other microorganisms. While seemingly innocuous, CRISPR sequences are a crucial component of the immune systems of these simple life forms. The immune system is responsible for protecting an organism's health and well-being. Just like us, bacterial cells can be invaded by viruses, which are small, infectious agents. If a viral infection threatens a bacterial cell, the CRISPR immune system can thwart the attack by destroying the genome of the invading virus. The genome of the virus includes genetic material that is necessary for the virus to continue replicating. Thus, by destroying the viral genome, the CRISPR immune system protects bacteria from ongoing viral infection. The specificity of CRISPR-based immunity in recognizing and destroying invading viruses is not just useful for bacteria. Creative applications of this primitive yet elegant defense system have emerged in disciplines as diverse as industry, basic research, and medicine.[2] CRISPR is not the first molecular tool designed to edit DNA, but it gained its fame because it solves some longstanding problems in the field. First, it is highly specific. When properly set up, the molecular scissors that make up the CRISPR system will snip target DNA only where you want them to. It is also incredibly cheap.

Methods and Studies

stunning progress has been made in correcting genetic diseases in the laboratory just over the past few years. for example, muscular dystrophy — a complex and devastating family of diseases characterized by the breakdown of a molecular component of muscle called dystrophin. For some types of muscular dystrophy, the cause of the breakdown is understood at the DNA level. In 2014, researchers at the University of Texas showed that CRISPR could correct mutations associated with muscular dystrophy in isolated fertilized mouse eggs which, after being reimplanted, then grew into healthy mice. By February of this year, a team here at the University of Washington published results of a CRISPR-based gene replacement therapy which largely repaired the effects

of Duchenne muscular dystrophy in adult mice. These mice showed significantly improved muscle strength — approaching normal levels — four months after receiving treatment.[4]

The first clinical trial involving CRISPR started in 2016. It involved removing immune cells from people with lung cancer, using CRISPR to edit out the gene expressed PD-1, then administrating the altered cells back to the same person. 20 other trials were under way or nearly ready, mostly in China, as of 2017.[5]

In 2016 the United States Food and Drug Administration (FDA) approved a clinical trial in which CRISPR would be used to alter T cells extracted from people with different kinds of cancer and then administer those engineered T cells back to the same people.[5-6]

As of 2016 CRISPR had been studied in animal models and cancer cell lines, to learn if it can be used to repair or thwart mutated genes that cause cancer.

In 2017, researchers successfully used CRISPR-Cas9 as a treatment in a mouse model of human genetic deafness, by genetically editing the DNA in some cells in the ears of live mice.[5]

Discussion

CRISPR can be used to correct a mutant gene and reverse disease symptoms in a living animal. By replacing the mutant form of a gene with its correct sequence in adult mice, researchers demonstrated a cure for a rare liver disorder that could be achieved with a single treatment. In addition to treating heritable diseases, CRISPR can be used in the realm of infectious diseases, possibly providing a way to make more specific antibiotics that target only disease-causing bacterial strains while sparing beneficial bacteria. Most experiments use mouse embryos or cells grown in petri dishes in artificial liquid designed to be like blood. Other researchers are modifying stem cells that may then be re-injected into patients to repopulate damaged organs. Only a few labs around the world are actually working with early human embryos. This research is highly regulated and carefully watched. Others work on plant cells, as whole plants can be grown from a few cells.

Conclusion

The story of how a mysterious prokaryotic viral defense system became one of the most powerful and versatile platforms for engineering biology highlights the importance of basic science research. Just as recombinant DNA technology benefited from basic investigation of the restriction enzymes that are central to warfare between phage and bacteria, the latest generation of Cas9-based genome engineering tools are also based on components from the microbial antiphage defense system. It is highly likely that the future solutions for efficient and precise gene modification will be found in as of yet unexplored corners of the rich biological diversity of nature.[7]

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