



Precise Gene Manipulation Using RNA Interference

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Description

Ribonucleic Acid (RNA) interference (RNA interference/RNAi) is a powerful and versatile technology that enables precise gene manipulation at the molecular level. It is a naturally occurring cellular process that involves the degradation or silencing of specific messenger RNA (mRNA) molecules, resulting in the inhibition of protein production from the corresponding genes. RNAi has emerged as a revolutionary tool in molecular biology, genetics, and biomedicine, with a wide range of applications including gene knockdown, gene silencing, and gene editing.

Mechanisms of RNA interference

RNAi is initiated by small RNA molecules, known as small interfering RNAs (siRNAs) or microRNAs (miRNAs), which are complementary to the target mRNA molecules. These small RNAs are incorporated into a protein complex called the RNA-Induced Silencing Complex (RISC), which guides them to the complementary mRNA molecules. The RISC complex cleaves or destroys the mRNA, preventing it from being translated into a protein. Alternatively, miRNAs can also inhibit translation by binding to the target mRNA and preventing its association with ribosomes, thereby blocking protein synthesis.

Advancements in RNA interference technology

Over the years, RNAi technology has undergone significant advancements, enabling more precise and efficient gene manipulation. Some of the notable advancements include:

siRNA design and delivery: Designing highly effective siRNAs is crucial for successful gene knockdown. Advances in siRNA design algorithms and chemical modifications have improved siRNA stability, specificity, and delivery efficiency. Novel delivery methods,

such as lipid-based nanoparticles, viral vectors, and exosome-mediated delivery, have also been developed to enhance the delivery of siRNAs to target cells and tissues.

Gene editing with CRISPR-Cas9 and RNAi: The revolutionary CRISPR-Cas9 gene editing system has been combined with RNAi technology to achieve precise gene manipulation. RNAi can be used to specifically silence the expression of a gene of interest, followed by CRISPR-Cas9-mediated gene editing to precisely introduce desired genetic modifications. This combination allows for highly specific and efficient gene editing, opening up new possibilities for precise gene manipulation in research and therapy.

Tissue-specific gene knockdown: RNAi can be used to achieve tissue-specific gene knockdown by designing siRNAs that target tissue-specific mRNA molecules. This allows for the selective silencing of genes in specific tissues or organs, enabling researchers to study gene function in a tissue-specific manner and potentially develop tissue-specific gene therapies for diseases that affect specific organs.

Therapeutic applications: RNAi has shown promising potential for therapeutic applications, including the treatment of genetic diseases, viral infections, and cancer. RNAi-based drugs, such as small interfering RNA (siRNA) therapeutics and Antisense Oligonucleotides (ASOs), are being developed as a new class of drugs for targeted gene knockdown in various diseases. Clinical trials for RNAi-based therapies are underway, with some already approved for the treatment of rare genetic diseases.

Functional genomics and drug discovery: RNAi has revolutionized functional genomics by enabling high-throughput screening of gene function in a systematic and efficient manner. RNAi-based screens have been widely used for identifying novel drug targets, understanding gene function, and discovering new therapeutic interventions. RNAi technology has also been employed in drug discovery to validate drug targets, assess drug efficacy, and predict drug responses.

Conclusion

RNA interference has emerged as a powerful tool for precise gene manipulation in various research and therapeutic applications. Advancements in siRNA design, delivery, gene editing with CRISPR-Cas9, tissue-specific gene knockdown, and therapeutic applications have significantly enhanced the precision and efficiency of gene manipulation using RNAi technology. This has opened up new avenues for understanding gene function, identifying drug targets, and developing novel therapies for various diseases. However, challenges still remain, such as off-target effects, delivery efficiency, and potential off-target effects.

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