



## Exploring the Potential of RNAi in Personalized Medicine and Gene Therapy

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### DESCRIPTION

RNA Interference (RNAi) is a highly conserved biological mechanism in eukaryotic cells that regulates gene expression by silencing specific messenger RNA (mRNA) molecules. Discovered in the late 1990s, RNAi has revolutionized molecular biology, providing powerful tools for functional genomics, therapeutic development, and biotechnology. This post-transcriptional gene silencing mechanism enables cells to control protein production, defend against viral infections, and maintain genomic stability.

The RNAi pathway is initiated by Double-Stranded RNA (dsRNA), which is processed into small RNA molecules, primarily Small Interfering RNAs (siRNAs) or MicroRNAs (miRNAs), by the enzyme Dicer. These small RNAs are then incorporated into the RNA-Induced Silencing Complex (RISC), guiding the complex to complementary target mRNAs. Upon binding, RISC either degrades the target mRNA or inhibits its translation, effectively silencing the gene. This specificity allows precise regulation of gene expression, making RNAi an invaluable research and therapeutic tool.

SiRNAs are typically exogenous or experimentally introduced double-stranded RNAs that trigger the degradation of target mRNAs with perfect sequence complementarity. They are widely used in laboratory settings to knock down the expression of genes, helping researchers understand gene function and molecular pathways. In contrast, miRNAs are endogenous, naturally occurring small RNAs that regulate multiple genes through partial complementarity, fine-tuning gene expression in processes such as development, cell differentiation, and apoptosis.

RNAi has extensive applications in functional genomics. By selectively silencing genes, researchers can study the effects of gene loss and identify critical pathways involved in disease, metabolism, and cellular signaling. High-throughput RNAi screens enable systematic analysis of gene function across the genome, accelerating target identification for drug discovery and biomarker development. In plants, RNAi plays an important

role in antiviral defense and has been harnessed to develop disease-resistant crops.

In therapeutics, RNAi offers a novel approach to treating diseases caused by aberrant gene expression, including cancer, viral infections, and genetic disorders. Synthetic siRNAs or Short Hairpin RNAs (shRNAs) can be designed to target disease-associated genes, reducing pathogenic protein levels. For example, RNAi-based therapies are being developed to inhibit genes driving tumor growth or viral replication. The specificity of RNAi reduces off-target effects compared to conventional drugs, making it a promising modality for precision medicine. Delivery of RNAi molecules remains a challenge, with strategies such as lipid nanoparticles, viral vectors, and conjugated carriers being explored to ensure stability and cellular uptake.

RNAi also contributes to the understanding of epigenetic regulation. Certain small RNAs can direct chromatin modifications, influencing gene transcription and genomic architecture. This highlights RNAi's role not only in post-transcriptional regulation but also in broader gene regulatory networks. Moreover, the discovery of RNAi has led to the development of CRISPR-Cas-based technologies, which share conceptual similarities in sequence-specific targeting and gene modulation. Despite its potential, RNAi research must address limitations such as off-target effects, immune responses, and efficient delivery to target tissues. Advances in chemical modification of RNA molecules, nanotechnology-based carriers, and improved targeting strategies are helping overcome these obstacles. Ongoing clinical trials are evaluating RNAi therapeutics for a range of conditions, marking a significant step toward clinical translation.

In conclusion, RNA interference is a transformative mechanism in molecular biology that enables precise gene silencing. Its applications span functional genomics, therapeutics, and biotechnology, offering new avenues for understanding and manipulating gene expression. As research continues to refine delivery methods and enhance specificity, RNAi holds immense potential for advancing personalized medicine and innovative treatments.

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