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Original Article

A Review on RNA-Based Therapeutics: Mechanisms, Applications, and Future Prospects

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ABSTRACT

RNA-based therapeutics have gained considerable attention in recent years due to their ability to regulate disease processes at the molecular level. Unlike traditional pharmacological agents that primarily interact with proteins, these therapies function by influencing gene expression and RNA translation pathways. Various RNA modalities, including messenger RNA (mRNA), small interfering RNA (siRNA), antisense oligonucleotides, and microRNA, have been explored for therapeutic applications. RNA-based medicines have demonstrated promising outcomes in the treatment of genetic disorders, cancer, viral infections, and metabolic diseases. Advances in chemical modification techniques and delivery systems such as lipid nanoparticles have significantly improved the stability, bioavailability, and safety of RNA molecules, thereby enhancing their clinical potential. However, certain limitations including off-target effects, immune stimulation, and efficient tissue-specific delivery remain major challenges. This review presents a concise overview of RNA-based therapeutic strategies, their mechanisms of action, advantages, and current limitations. Additionally, it highlights recent developments and future prospects of RNA therapeutics in modern drug discovery. With continued research and technological innovation, RNA-based therapies are expected to play an increasingly important role in personalized and precision medicine.

Keywords: RNA therapeutics, Gene regulation, mRNA therapy, siRNA technology, Drug delivery systems

INTRODUCTION

Recent progress in molecular biology and pharmaceutical sciences has led to the development of novel therapeutic strategies that target diseases at the genetic level. Among these approaches, RNA-based therapeutics have emerged as a promising class of medicines capable of regulating gene expression and cellular functions. [1,3] Unlike conventional drugs that mainly interact with proteins or receptors, RNA-based therapies act by modifying RNA translation or interfering with specific genetic sequences, thereby offering a more precise mode of treatment. [4,5] Different forms of therapeutic RNA, including messenger RNA (mRNA), small interfering RNA (siRNA), antisense oligonucleotides, and microRNA, have been extensively investigated for their potential applications in various diseases. [2,6,7] These molecules can either enhance the production of therapeutic proteins or suppress the expression of disease-causing genes. RNA-based therapeutics have shown encouraging results in the management of genetic disorders, cancer, infectious diseases, and metabolic conditions. [3,4,10] The clinical success of RNA-based medicines has been supported by significant advancements in RNA stabilization techniques and targeted delivery systems, such as lipid nanoparticles and polymer-based carriers. [6,3] These innovations have improved the safety, efficacy, and bioavailability of RNA molecules. However, challenges related to immune responses, off-target effects, and efficient tissue-specific delivery continue to limit their widespread applications. [4,5,8]

2. Types of RNA-Based Therapeutics

1. Small Interfering RNA (siRNA)

- Short, double-stranded RNA molecules designed to target specific mRNA sequences.
- Bind to complementary mRNA and induce its degradation, resulting in gene silencing.

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- Prevents the synthesis of disease-causing proteins.
- Widely explored in cancer therapy, viral infections, and genetic disorders. [2,8]

2. MicroRNA (miRNA)

- Endogenous, single-stranded RNA molecules involved in post-transcriptional regulation of genes.
- Therapeutic approaches aim to replace deficient miRNAs or inhibit overexpressed miRNAs.
- Suppress protein production by regulating mRNA translation rather than complete degradation.
- Show potential in cancer, cardiovascular, and neurological diseases. [2,4]

3. Antisense Oligonucleotides (ASOs)

- Short, synthetic single-stranded nucleic acid sequences complementary to target mRNA.
- Bind to mRNA and block its function or promote degradation.
- Reduce abnormal protein expression with high sequence specificity.
- Used in the management of genetic, inflammatory, and rare inherited disorders. [1,8]

4. Messenger RNA (mRNA) Therapeutics

- Involve delivery of synthetic mRNA into cells to produce therapeutic proteins.
- Utilize the cell's natural translational machinery.
- Do not integrate into the host genome, ensuring a reversible and safe approach.
- Applied in vaccine development, cancer immunotherapy, and protein replacement therapy. [6,3]

5. RNA Aptamers

- Short, single-stranded RNA sequences that fold into unique three-dimensional structures.
- Bind specifically to target proteins or receptors with high affinity.
- Function similarly to antibodies but with lower immunogenicity.
- Used in targeted therapy, diagnostics, and drug delivery systems. [5,7]

3. Mechanism of Action of RNA-Based Therapeutics

RNA-based therapeutics work by regulating gene expression at the RNA level. These therapies mainly act by controlling the production of proteins involved in disease processes. The general mechanism is based on specific interaction between therapeutic RNA molecules and their target messenger RNA (mRNA).

3.1General Mechanisms

- RNA-based therapeutics are delivered into target cells using suitable delivery systems.
- Once inside the cell, the therapeutic RNA recognizes and binds to a specific mRNA sequence.
- This binding interferes with normal mRNA function.
- As a result, the production of disease-causing proteins is reduced or a required therapeutic protein is produced.
- The overall effect leads to suppression or correction of abnormal cellular activity. [4,8]

3.2 Type of Mechanism

1. Small Interfering RNA (siRNA)

- siRNA binds to complementary mRNA inside the cell.
- The bound mRNA is degraded, preventing protein synthesis.

2. MicroRNA (miRNA)

- miRNA interacts with target mRNA and suppresses its translation.
- This results in reduced protein expression without complete mRNA degradation.

3. Antisense Oligonucleotides (ASOs)

- ASOs bind directly to target mRNA sequences.
- This binding block mRNA function or promotes its breakdown.

4. Messenger RNA (mRNA) Therapeutics

- Synthetic mRNA enters the cell and uses cellular machinery to produce therapeutic proteins. The produced protein helps in disease treatment without altering the genome. [2,6,8,9]

4. Applications and Therapeutic Uses of RNA-Based Therapeutics

1. Cancer Therapy

RNA-based therapeutics are used to suppress the expression of oncogenes responsible for uncontrolled cell growth. By targeting specific mRNA involved in tumor progression, these therapies help reduce cancer cell proliferation. They are also investigated in combination with chemotherapy and immunotherapy to enhance treatment effectiveness. [3,4,10]

2. Genetic Disorders

Many genetic diseases are caused by mutations that lead to abnormal protein production. RNA-based therapeutics help control or correct these defects by silencing faulty genes or by producing missing functional proteins. This approach offers a targeted and personalized treatment option for inherited disorders. [1,4,10]

3. Viral Infections

RNA-based therapeutics are effective in inhibiting viral replication by targeting viral RNA or host factors required for viral growth. By blocking the synthesis of viral proteins, these therapies help limit infection and disease progression. They are being actively studied for various viral diseases. [2,3]

4. Neurological Disorders

In neurological conditions, abnormal gene expression plays a key role in disease development. RNA-based therapeutics help regulate proteins involved in neuronal damage and degeneration. This approach shows potential in treating neurodegenerative and rare neurological disorders. [2,4]

5. Cardiovascular Diseases

RNA-based therapeutics are explored for the regulation of genes associated with cholesterol metabolism, inflammation, and vascular function. By modulating these pathways, they contribute to improved cardiovascular health and disease management. [4,3]

5. Current Status and Future Prospects

- RNA-based therapeutics have progressed from lab research to clinical evaluation.
- Several RNA drugs and vaccines are already approved for human use.
- Ongoing clinical trials are exploring treatments for cancer, genetic disorders, viral infections, and neurological diseases.
- Current challenges include stability, delivery, and off-target effects.
- Advances in delivery systems, chemical modifications, and personalized approaches are expected to improve safety and effectiveness.
- RNA therapeutics are likely to play an important role in modern medicine with more precise and targeted treatments in the future. [3,4,6,10]

DISCUSSION

RNA-based therapeutics represent a new and promising approach for treating a wide range of diseases. This review shows that different types of RNA molecules, such as siRNA, miRNA, antisense oligonucleotides, mRNA, and aptamers, can

specifically target disease-causing genes and regulate protein expression. Compared to traditional drugs, RNA therapeutics offer high specificity, allowing precise control of gene expression with minimal side effects.

CONCLUSION

RNA-based therapeutics have emerged as a powerful and versatile tool for treating a wide range of diseases. By targeting specific genes and regulating protein production, these therapies offer high precision and reduced side effects compared to conventional treatments. Different types of RNA molecules, including siRNA, miRNA, antisense oligonucleotides, mRNA, provide multiple strategies for addressing cancer, genetic disorders, viral infections, neurological conditions, and more. Although challenges like stability, delivery, and off-target effects remain, advances in delivery technologies, chemical modifications, and personalized approaches are expected to overcome these limitations. The current progress in clinical trials and approved therapies highlights the potential of RNA-based treatments to transform modern medicine. Overall, RNA therapeutics represent a promising frontier in precision medicine, offering targeted, effective, and safer solutions for diseases that were previously difficult to treat.

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