TECHNOLOGY

Compositions and Methods for Delivering Nucleic Acids to Cells

OVERVIEW

Summary

This invention provides a breakthrough in gene therapy and molecular biology, featuring a non-viral vector system for delivering nucleic acids to cells. It utilizes a polyplex particle with acetylated polyethylenimine (Ac-PEI) and an anionic biomaterial envelope, reducing cytotoxicity and enhancing gene delivery efficiency. The technology is important for gene therapy, immunotherapy, and vaccine development. The gene therapy market is rapidly growing, projected to reach USD 35.1 billion by 2032, and this innovation addresses key market needs, including safety and efficacy in gene therapy treatments.

Technology

This technology represents a significant leap in the field of gene therapy and molecular biology. It is the development of a non-viral vector system for the delivery of nucleic acids to cells, a challenge that has been a significant bottleneck in therapeutic applications. This system uses a non-viral polyplex particle, which includes polyethylenimine (PEI) complexed with nucleic acid and an envelope of an anionic biomaterial. This invention could surmount the limitations of existing viral and non-viral methods, offering a more efficient and less toxic pathway for gene delivery.

Innovation in this domain is vital, given the potential for gene therapy to treat a broad range of diseases by correcting genetic defects, silencing detrimental genes, or providing new functions to cells. The invention uses acetylated polyethylenimine (Ac-PEI), which reduces the cytotoxicity associated with conventional PEI, thereby improving its suitability for in vivo applications. This adjustment to PEI also enhances its ability to release the nucleic acids once inside the target cells, a critical step for successful gene expression.

Key features of this technology include the ability to maintain the stability of the polyplexes in the presence of serum, a critical component of the in vivo environment that has historically impeded gene delivery. Additionally, the modular nature of the polyplexes allows for the control of cellular interactions, enabling tailored applications to specific cell types or disease states.

Technical advantages are manifold. The polyplexes exhibit a lower toxicity profile compared to their non-acetylated counterparts, which is a substantial benefit for clinical applications. Furthermore, the system demonstrates enhanced transfection efficiency, particularly within immune cells, an area of great interest for immunotherapy applications.

The primary application of this technology is in gene therapy, including the treatment of genetic disorders, cancers, and infectious diseases. Secondary applications could extend into vaccine development, where genetic material from pathogens can be delivered to cells, prompting an immune response without the use of weakened pathogens.

Market

The gene therapy market is rapidly expanding, with projections indicating a growth from USD 7.7 billion in 2022 to USD 35.1 billion by 2032, at a CAGR of 16.4%. Another study estimates the market to reach USD 23.9 billion by 2028. This growth is fueled by a substantial pipeline of transformative therapies. This new technology aligns with the escalating demand for efficient and safer gene delivery methods, addressing key market gaps such as reducing the toxicity of gene delivery vectors and enhancing transfection efficiency, particularly in immune cells.

The commercial potential is underpinned by a record number of gene therapy approvals for treating rare diseases and cancers. Regulatory bodies are also modernizing frameworks to accommodate these innovations, which is critical for market growth.

This technology could impact the gene therapy market segment, addressing unmet needs by improving the safety profile and efficacy of gene therapy treatments. The technology's ability to offer a modular approach to specifically control cellular interactions of the polyplexes enhances its commercial appeal, positioning it to capitalize on market trends towards personalized and precise medical treatments.

References

- "Gene Therapy Market Size to Reach USD 35.1 Billion By 2032 CAGR: 16.4%," DataHorizzon Research. https://finance.yahoo.com/news/gene-therapy-market-size-reach-004000569.html
- "The Cell And Gene Therapy Sector In 2023: A Wave Is Coming – Are We Ready?" In Vivo. https://invivo.citeline.com/IV146781/The-Cell-And-Gene-Therapy-Sector-In-2023-A-Wave-Is-Coming--Are-We-Ready

Additional Information

- Potential Fields of Application: Gene therapy, immunotherapy, vaccine development, genetic research, molecular biology.
- Keywords: Non-viral vector, gene delivery, polyplex particle, polyethylenimine (PEI), acetylated PEI, nucleic acid transfection, anionic biomaterial envelope, cytotoxicity reduction, immune cell transfection.
- Advantages: Reduced toxicity, improved stability in serum, enhanced transfection efficiency, controlled cellular interactions, modular design, extended duration of gene expression.

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- Biologics
- Methods of Treatment

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RP-2020-012