

MAN_08 - Technology Roadmap: Delivery systems for RNAi Therapeutics

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Introduction: RNA-based gene therapy is a promising approach for treating genetic diseases. Scientists have developed viral and non-viral delivery systems, such as nanoparticles, to protect RNA and ensure its safe transportation. Creating a technology roadmap can identify investment opportunities in RNA delivery systems.

Objectives: This study explores RNAi delivery in gene therapy using a technology roadmap methodology and highlights the opportunities in the gene therapy market.

Methodology: This study analyzed interference RNA and their delivery platforms via a keyword search in scientific databases. The search was limited to articles between 2018 and 2024. The methodology involved a three-step process and analyzed 100 papers in the Scopus database and 80 issued patents in the Clarivate Derwent Innovation database. The roadmap was developed in Microsoft Excel. The goal was to track the clinical assays of nanocarriers, RNAi molecules, and modern materials used for delivering nucleic acids.

Results: During literature research, a total of 1551 items were found with the majority of them originating from China and the United States. In patent research, 1478 documents were obtained, with most of them coming from the United States and Japan. Alnylam has the most issued patents. Major players invest in lipid-based platforms, while universities study extracellular vesicles, polymers, silica, metal-organic frameworks (MOFs), etc. There is a trend towards a synergistic effect of RNAi therapy with traditional therapies. Co-factors in the delivery mechanism are also being studied. Players in the field are developing new therapies to combat the increasing resistance to current cancer treatments. Alnylam's innovative RNAi therapy approach, along with universities and other players, is driving the market forward and providing hope for those seeking new treatments for cancer, rare and neglected diseases.

Conclusion: The technology roadmap is a fascinating tool that illustrates the trends in market and scientific technologies. Our research has revealed significant findings: the diversity of materials studied to deliver RNAi and how gene therapy is advancing fast. This study is a valuable contribution to the market study and experimental design of materials for nanocarriers and the development of RNAi therapy.

Keywords: Interference RNA; Nanosystems; Technology roadmap