TECHNOLOGY

Targeted Delivery System for Therapeutic RNAs

OVERVIEW

Background

RNA interference (RNAi) is a powerful tool that has allowed researchers to exert greater control over the expression of genes within cells. With this experimental control over gene expression, researchers have uncovered the mechanisms for numerous diseases that led to the conclusion that RNAi could be a powerful therapeutic method to combat many genetic diseases. In experimental cell culture, researchers directly transport RNAi constructs into the cell using common transfection reagents or techniques that disrupt the cell membrane to allow passive diffusion of RNAi constructs into the cell. Unfortunately, researchers cannot use these techniques in vivo as the transfection mechanisms cannot gain direct access to the cells of interest within the body, and unprotected RNAi constructs generally do not survive transport through the body to the cells of interest. To be able to harness the full potential of RNAi as a therapeutic, a method for safely delivering RNAi constructs to select subpopulations of cells and then transporting the RNAi into those cells efficiently is needed.

Innovative Technology

Researchers at the University of Maryland have developed a new method to deliver RNAi constructs to cells. This delivery system uses recombinant proteins to protect the RNAi message from being degraded as it travels through the body to the target cell. Once reaching the target cell, the proteins uses the cell's own natural process for incorporating material into the cell, preserving membrane integrity. This active transport across the cell membrane is more efficient than passive diffusion lowering the amount of RNAi material needed, and shortening the time needed for transfection. The delivery system can be modified to only transport to a select population of cells, allowing for targeted treatment of diseases.

APPLICATIONS

· In vitro genetic research
· Delivery of therapeutic RNA for treatment of genetic diseases

ADVANTAGES

· Uses natural cellular systems to insert RNAi constructs into the cell increasing cell viability
· Achieves effective gene silencing in hours instead of days

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Additional Information

INSTITUTION

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LICENSE STATUS

Contact OTC for licensing information

CATEGORIES

- Biologics
- Drug delivery devices

EXTERNAL RESOURCES

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