INNOVATION PARTNERSHIPS
UNIVERSITY OF MICHIGAN

Ultrasound Assisted Gene Therapy

Technology number: 2023-335

Technology ID
2023-335

Category
Therapeutics and Vaccines
Life Sciences

Inventor
Michael Passineau
Isabelle Lombaert

Further information
Megan Griffin
mrost@umich.edu

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OVERVIEW

Ultrasound-assisted gene therapy using non-viral plasmid and microbubbles

- No immune response, allows multiple dosing tailored to patient needs
- Can treat genetic disorders, cancer, and radiation-induced hyposalivation

BACKGROUND

Gene therapy has shown promise in treating various genetic disorders and certain cancers but often faces challenges related to delivery mechanisms, particularly those involving viral vectors, which can trigger immune responses and limit repeat dosing. Adenoassociated and adenoviral vectors, commonly used in gene therapy, have these limitations, making them less ideal for tailored treatments. Historically, non-viral plasmids have been explored but struggled with efficient delivery and cellular uptake. There is a critical need for a more effective, repeatable, and non-immunogenic delivery method. Ultrasound-assisted gene therapy offers a potential solution by using microbubbles to enhance plasmid delivery, thereby enabling more personalized therapeutic approaches.

INNOVATION

Investigators have created a product that combines a non-viral plasmid, an FDA-approved ultrasound machine, and microbubbles to achieve efficient gene delivery without eliciting an

immune response. The plasmid is delivered using a GE ultrasound machine working in conjunction with FDA-approved Lantheus microbubbles. This method allows for multiple dosing, enabling tailored treatment protocols based on patient needs. Originally designed to treat radiation-induced hyposalivation, this approach has broader applications, including treating various genetic disorders and cancers. Key advancements include the non-immunogenic delivery system, the ability for repeat dosing, and a potential new patent surrounding these innovations. Real-world applications encompass personalized gene therapy for genetic disorders, cancer treatments, and conditions arising from radiation therapy, demonstrating a significant leap forward in gene therapy delivery methods.