Gene Editing in Utero via Non-Viral, Lipid Nanoparticle Delivery of mRNA Complexes

Tech ID: 32822 / UC Case 2022-508-0

ABSTRACT

Researchers at the University of California, Davis have developed a new method of in utero gene editing through lipid nanoparticle delivery of mRNA gene editors.

FULL DESCRIPTION

Gene editing therapies have the potential to treat a variety of devastating diseases, but they currently have a narrow range of suitable applications due to limitations in their delivery methods. Typically, lipid nanoparticle (LNP) or viral delivery of mRNA editors are administered, but these substances are too large to bypass the vascular system and have difficulties in reaching the desired organ system. Consequently, these methods of delivery are only suitable to treat diseases that affect phagocytic organs, such as the liver. Many genetic disorders can be identified in relatively early stages of fetal development, and ongoing research suggests that one of the most effective treatments for severe disorders involves genetic editing in utero. Implementing this approach may alleviate some of the shortcoming of traditional gene editing therapies.

Researchers at the University of California Davis have developed a novel method of in utero gene editing through a non-viral LNP delivery of mRNA complexes. During fetal development there is a high rate of cell division, stem cell proliferation, and angiogenesis, allowing gene editors to more easily transfer from blood to organ tissue. Therefore, this approach can target a variety of major organ systems, including the heart, liver, kidneys, lungs, GI tract, and brain. Unlike adults, the fetal immune system doesn't generate an antagonistic response against gene editing enzymes which further improves the efficiency of gene editing in this stage. LNP delivery is preferred over vial delivery since it generally features more specific targeting and doesn’t trigger immune responses as frequently. In utero gene editing also avoids the ethical issue of germline editing, since these genetic alterations can’t be passed down from one generation to the next. This novel approach of in utero editing can be performed at low cost without advanced equipment, making this an important discovery in the treatment of deadly genetic diseases.

APPLICATIONS

▶ Treatment of genetic disorders affecting major organ groups such as the heart, liver, kidneys, lungs, GI tract, and brain
▶ Gene therapy accomplished through gene editing in utero

FEATURES/BENEFITS

▶ Can target a wider range of organ systems compared to existing gene therapies
▶ Doesn’t generate an antagonistic immune response to gene editing enzymes, allowing for more efficient editing
▶ Low cost and accessible treatment

PATENT STATUS

Patent Pending

RELATED CASES

2022-508-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

▶ Exosome-Mimicking Nanovesicles