Intranasal Delivery of Oligonucleotides for Neurodegenerative Diseases

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BRIEF DESCRIPTION

Delivery of oligonucleotide therapy to the central nervous system remains challenging. Neurodegenerative diseases, such as Huntington’s disease and Spinal Muscular Atrophy, can require intrusive and regular treatments, therefore a non-invasive delivery system would be very beneficial to patients. UC Irvine researchers have proposed a new method of therapeutic delivery utilizing a SARS-CoV-2 pseudovirus. Delivered intranasally, this system has the ability to bypass the blood brain barrier, making it an exciting approach to decrease risk for patients and ease the treatment process.

SUGGESTED USES

• Intranasal delivery of oligonucleotide therapeutics for neurodegenerative diseases
• Cross the blood brain barrier and provide delivery to the brain without invasive procedure

FEATURES/BENEFITS

• Less invasive: Nasal injections offer more flexibility and less pain for treatments
• Decreased risk: Minimize side effects due to lumbar injections and easier to tolerate for those with motor impairments
• Efficient: The delivery system would allow large amounts of therapeutic to be delivered on as frequent of a schedule as needed

TECHNOLOGY DESCRIPTION

Neurodegenerative diseases, such as Alzheimer’s disease, Parkinson’s disease, amyotrophic lateral sclerosis, and Huntington’s (HD), ultimately lead to neuronal loss and death. These are progressive disorders, many of which are characterized by behavioral changes, aberrant movements, and cognitive decline. Several neurodegenerative diseases are caused by a single, well-characterized genetic mutation. Unfortunately, many of them, such as HD, lack appropriate treatments. New medications based on RNAi or antisense oligonucleotide therapies are being developed that require intensive and painful lumbar punctures or brain surgery. Scientists at UC Irvine have proposed a new drug delivery tool utilizing a derivative of the SARS-CoV-2 virus for the delivery of drugs including RNAi or antisense oligonucleotide reagents. This method is highly beneficial as it can be delivered intranasally, is capable of crossing the blood-brain barrier, and through the use of aSARS-CoV-2 pseudovirus, can deliver oligonucleotides to neuronal cells. Nasal delivery is far less invasive than brain surgery or lumbar puncture, and allows for therapeutic delivery as regularly as needed. These qualities make this delivery technique a valuable approach to decrease risk for patients and ease the treatment process.

STATE OF DEVELOPMENT

Scientists have constructed a SARS-CoV-2 pseudovirus and shown in vitro infection of human neuronal cultures. Mouse studies to demonstrate efficacy in vivo are planned.