Method for Delivery to the Brain of Plasmid DNA Encoding a Therapeutic Proteins for Central Nervous System Disorders

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Description

Current gene therapy approaches for brain disorders involve surgical injection of a vector carrying the gene of interest into a patient's brain. Such approaches are highly risky as brain surgery is one of the most invasive procedures. Moreover, such surgeries can have associated deleterious side effects, especially on patients affected with CNS disorders. At the same time, most commonly used vectors (viral vectors) for such procedures are immunogenic, thereby raising potential safety concerns. **This novel approach comprises the use of a plasmid DNA to deliver a gene encoding therapeutic protein of interest to patient's brain.**

Value Proposition

The approach:

• Is safer than past approaches as it enables the use of non-viral vectors (such as plasmid DNA) for gene therapy as compared to unsafe viral vectors used in conventional approaches

• Allows for a controlled and time-limited protein expression

• Allows for an efficient repeated dosage as required

• Allows for a non-invasive gene therapy technique, avoiding surgical needs as observed with prior art approaches

• Provides a renewable, endogenous source of therapeutic protein within the patient's brain

• Enables an efficient intranasal delivery of large, charged molecules such as plasmid DNA by bypassing the blood brain barrier

• Would be a commercially useful gene therapy technique for the treatment of neurodegenerative disorders such as Parkinson's disease, Alzheimer's disease, Huntington's disease, chronic pain, drug addition, mood disorders, schizophrenia, autism, ADHD, and epilepsy

Intellectual Property Status

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