Cincinnati Children's | Innovation Ventures

Drug Delivery Therapeutic

Therapeutic Protein Delivery Across the Blood-Brain Barrier

Brief Description of Technology

A delivery mechanism which allows large molecule therapeutics to trancytose the blood-brain barrier for treatment of neurological disorders.

TECHNOLOGY ID

2010-1203

BUSINESS OPPORTUNITY

Exclusive License

TECHNOLOGY TYPE

Natural/Modified Protein

PATENT INFORMATION

Nationalized

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Technology Overview

The blood-brain barrier prevents large molecular weight substances from trancytosing into the CNS. This technology surmounts this barrier by fusing the receptor-binding domain (Rb) of apolipoprotein E with potentially therapeutic proteins that can bind to LDL receptors on the BBB. Proof of concept was demonstrated using lysosomal enzyme α-L-iduronidase (IDUA) in a mouse model. Testing showed desirable receptor-mediated binding, endocytosis, and transendothelial transport of the fusion protein, as well as appropriate lysosomal enzyme flow and biological function, and led to 2% to 3% of normal brain IDUA activities 5 months after long-term delivery. The technology can be used to deliver therapeutic treatment for lysosomal storage diseases, with potential application in other brain diseases.

Applications

- Drug delivery mechanism that allows protein therapeutics to cross the blood-brain barrier
- Proof of concept tested in Hurler Syndrome
- Cell therapies
- Protein therapies

Advantages

- Non-invasive
- Global delivery of protein therapeutics to brain and central nervous system tissue
- Only limited activity required for significant benefit

Market Overview

There are over 50 lysosomal storage diseases (LSD), and enzyme replacement therapy has been largely unsuccessful in improving CNS manifestations of LSDs due to difficulty penetrating the blood-brain barrier.

Investigator Overview

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