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Lysosomal Storage Disease Enzyme Delivery System (20100076)

IP Status: Issued US Patent; **Application #:** 14/103,597

Intranasal Delivery of Therapeutic Enzymes

A new technology rapidly delivers therapeutic enzymes via the nasal passage to the central nervous system (CNS). Such enzymes are critical for treating lysosomal storage diseases, in particular, Hurler syndrome. Intranasal delivery is a non-invasive method able to bypass the blood-brain barrier (BBB) to rapidly target therapeutics directly to the CNS along the olfactory and trigeminal nerve pathways.

Hurler Syndrome Treatment Requires Critical Enzymes

Hurler syndrome is one of the most severe versions of lysosomal storages diseases (mucopolysaccharidoses, or MPSs). MPS diseases occur when too many large molecules, called glycosaminoglycans (GAGs), accumulate (i.e. are stored) in a cell because the patient lacks the enzymes required to break them down. When such materials accumulate in the brain, a number of devastating neurological effects occur. Delivering the missing enzymes directly to the CNS may alleviate or even prevent these adverse effects by preventing the accumulation of storage materials. Existing therapies do not address CNS symptoms of MPSs because they cannot penetrate the BBB.

Fast Delivery

This intranasal method not only provides a novel route of administering FDA-approved therapeutic enzymes to the brain, but it does so quickly: in only 20 minutes, enzyme activity in mice was detected throughout the brain. In addition, this method could be used much earlier (i.e. in neonates), expanding the patient population that could benefit from treatment.

BENEFITS AND FEATURES OF INTRANASAL DELIVERY OF ENZYMES FOR LYSOSOMAL STORAGE DISEASES:

- Enzymes bypass the blood-brain barrier to reach the CNS, protecting the brain from damage and alleviating symptoms
- Fast delivery – enzyme activity in the brain occurs within 20 minutes
- Lower risk of infection – intranasal delivery is non-invasive
- Intranasal delivery could be used in addition to IV delivery
- Enzymes may prevent accumulation of glycosaminoglycans (GAGs)

Phase of Development - In Vivo/Animal Studies

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Life Sciences/Pharmaceuticals
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