



USE OF TIDEGLUSIB FOR TREATMENT OF MUCOPOLYSACCHARIDOSIS TYPE II

(Below is limited to 1-page only; be careful not to disclose vital technology content. Please delete these words when the document is finished)

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Experience:

https://www.mc.ntu.edu.tw/medgenpro/Vcard.action?q_type=-1&q_itemCode=380

Market Needs:

Patients with Mucopolysaccharidosis (MPS) type II currently rely on enzyme replacement therapy (ERT) using human recombinant IDS administered intravenously, which cannot cross the blood-brain barrier to treat the central nervous system.

Our Technology:

Our technique utilizes iPSC-derived neuronal models from MPS II patients to elucidate key degenerative mechanisms and assess potential drug therapies. We found that the small molecule drug Tideglusib effectively corrects disrupted signaling pathways and enhances neuronal survival and function.

Strength:

The small molecule drug Tideglusib can cross the blood-brain barrier and operates through a mechanism distinct from current therapies. With known pharmacokinetics and pharmacodynamics, Tideglusib holds potential as an adjunct treatment for patients with mucopolysaccharidosis type II, particularly those with severe forms involving neurodegeneration, and may advance more quickly into clinical trials.

Competing Products:

JCR Pharmaceuticals Co., Ltd. has developed JR-141, a recombinant fusion protein combining an anti-transferrin receptor antibody with IDS. Short-term clinical trials indicate some patients show cognitive improvement, but the long-term effects remain unknown. JCR has agreed to grant Takeda exclusive commercial rights to JCR-141 in the U.S. after phase 3 studies are completed.

Intellectual Properties:

- (1) This technology has been granted a U.S. provisional patent with application number: 63/617,433.
- (2) Our research team has 30 years of research experience.

Contact (do not need to fill out):

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