Small molecule therapeutic treats glycogen storage disease independent of patient-specific enzyme deficiencies

Technology #cu16051

This technology is a small molecule therapeutic that can be used to decrease the accumulation of glycogen caused by glycogen storage disease.

Unmet Need: Universal treatment for glycogen storage disease

Glycogen storage diseases (GSD) are a family of diseases that result from abnormal glycogen metabolism, leading to unhealthy glycogen accumulation in the body that can cause growth defects, organ failure, and even death. Current treatments mainly function by replacing deficient enzymes using gene therapy, which carries expensive and labor-intensive development. Additionally, as enzyme deficiencies vary by patient, treatment by gene therapy is not generalizable and must be tailored to each GSD subtype. As such, there is a need for a GSD treatment that reduces glycogen accumulation regardless of a patient’s GSD subtype.

The Technology: Guaiacol is a cost-effective strategy for reducing glycogen production and accumulation

This technology treats GSD by targeting the production of glycogen itself, upstream of respective deficiencies in glycogen metabolism. Using a mouse embryo fibroblast (MEF) cell line that was engineered to accumulate glycogen to simulate GSD, this technology identified a compound called guaiacol that decreases glycogen synthesis and accumulation by 50%. This technology demonstrated that guaiacol reduces glycogen by inhibiting glycogen synthase. As such, unlike labor- and cost-intensive gene therapy, this technology treats GSD irrespective of the specific enzyme deficiency with a simple small molecule therapeutic.

Guaiacol has been demonstrated to cause a significant decrease in glycogen content in the liver in a mouse model of GSD.
Applications:

- Treatment and prevention of Glycogen storage diseases (GSD), including Adult Polyglucosan Body Disease and Lafora Disease
- Research tool for studying GSD

Advantages:

- Guaiacol is readily available and easy to produce
- Targets glycogen production to treat GSD regardless of the specific deficiency in glycogen metabolism
- More cost-effective than expensive gene therapy

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Patent Information:

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Related Publications:


Tech Ventures Reference:

- IR CU16051
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