Innovations
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

Polyamines for the Treatment of Homocystinuria

Product

Spermine and Spermidine

Indication

Homocystinuria

Value Propositions

- Reduced homocysteine serum levels
- Synergistic with existing HCU therapies
- Oral formulation

Market

- $6.5 Million—U.S. HCU therapeutic market in 2017
- Few existing therapeutic competitors

Intellectual Property

- Patent pending*
- Available for licensing

Background on CU5464H

Homocystinuria (HCU) is a rare genetic disease caused by a deficiency in the cystathionine beta-synthase enzyme with resulting buildup of the amino acid homocysteine in the serum and urine. The disease is characterized by intellectual disability, hypercoagulability, scoliosis, nearsightedness, and displaced lenses of the eyes. Currently, few treatments for the disease exist (betaine being the main commercialized therapeutic). Limiting dietary intake of protein (due to methionine's conversion to homocysteine) continues to be the mainstay of treatment even though it is restrictive and difficult to follow.

Technical Innovation

Dr. MacLean and Dr. Jiang have discovered that spermine and spermidine (polyamines) may be novel therapeutics for the treatment of HCU. They have shown that polyamine metabolism is altered in HCU and may contribute to disease pathogenesis. In-vivo studies have demonstrated lowered spermine and spermidine levels in mouse models with HCU phenotype (see Figure). Spermine and spermidine deficiency have been implicated in other disease states that share features with HCU—intellectual disability and scoliosis (in Robinson syndrome) and increased platelet aggregation and thrombus formation (hypercoagulable physiology). Therefore, there is reason to think that spermine and spermidine supplementation may provide clinical benefit in patients with HCU.

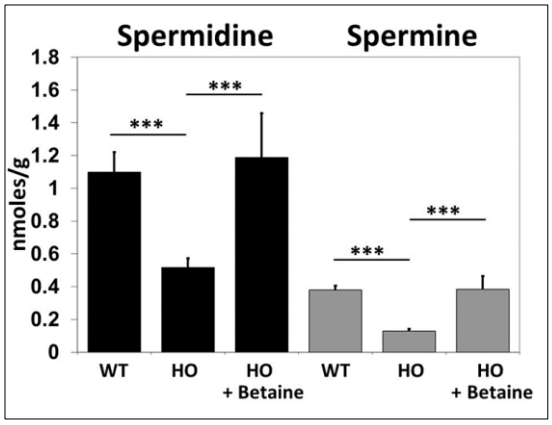

Figure: Spermidine and Spermine levels in mouse models with (HO) and without (WT) HCU phenotype. Higher spermidine and spermine levels are observed after treatment with Betaine.

Resources & Documents:

Maclean et. al. (2021). *Derangement of hepatic polyamine, folate, and methionine cycle metabolism in cystathionine beta-synthase-deficient homocystinuria in the presence and absence of treatment: Possible implications for pathogenesis.* Molecular Genetics and Metabolism, 132(2), 128–138. https://doi.org/10.1016/j.ymgme.2021.01.003

**US Provisional Application—63/109,983—"Compositions and Methods for Treating Homocystinuria and Other Conditions Using polyamines"—filed 11/5/21*

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