Technology Opportunity

Ref: RDLP# 884

Keywords: Peroxisomes, Autophagy inhibitors, Zellweger syndrome

BACKGROUND: Peroxisome Biogenesis Disorders (PBDs) are a spectrum of diseases ranging from the severe form, Zellweger syndrome, to milder forms, neonatal adrenoleukodystrophy and infantile Refsum disease. These autosomal recessive diseases are derived from dysfunction of peroxisomes and treatment is primarily focused on supportive care, dietary management, symptomatic therapy and treatment strategies involving the use of pharmacological induction of peroxisomes. To date, therapeutic interventions for PBDs have targeted biochemical defects of peroxisomal function in patients. While this approach appears to have some effect on the milder forms of PBDs, it can be highly variable in PBD patients and not effective in patients with Zellweger syndrome. Originally thought to be disorders of peroxisome biogenesis the new hypothesis is that PBDs are caused by the increased rate of peroxisome degradation or autophagy. Currently there are autophagy inhibitors available for the treatment of cancers and other proliferative disorders.

DESCRIPTION OF OPPORTUNITY: Our scientists are suggesting a new use for existing and approved autophagy inhibitors in the treatment of peroxisome biogenesis disorders. Modulation of autophagy activity of peroxisomes offers a viable treatment option to increase the number and normal functioning of peroxisomes, thereby preventing further loss of peroxisome numbers.

POTENTIAL ADVANTAGES/APPLICATIONS: There are currently no approved treatment options to specifically treat peroxisome biogenesis disorders, with the majority of therapy being symptomatic in nature such as cataract surgery and physical therapy. Given the unmet medical need and the possibility that the targets discussed here might be disease altering, it would be important to implement a drug development strategy that is innovative, rapid and lean. Our repurposing strategy will result in decreased development costs and the chance of successful clinical trials is also higher due to the prior wealth of clinical knowledge on the compounds.

DEVELOPMENT STAGE: In vitro data in patient derived cell lines have been generated

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