



Gene Therapy for Fatty Acid Metabolism Defects

The University of Florida is seeking companies interested in commercializing an adeno-associated viral vector (rAAV) treatment for fatty acid metabolism defects. Inborn errors of metabolism are associated with a specific enzyme defect in the fatty acid metabolic pathway, resulting in a host of physical maladies including cardiomyopathy, seizures, failure to thrive, neurological disorders, coma, SIDS and even death.

Applications

Treatment and/or prevention of symptoms associated with defects in fatty acid metabolism common in:

- ◆ Cardiomyopathy
- ◆ Hypoglycemia
- ◆ Hypotonia
- ◆ Seizures
- ◆ Developmental delay
- ◆ Failure to thrive
- ◆ Neurological disorders
- ◆ Coma
- ◆ SIDS

Advantages

- ◆ Gene therapy is safer and far less invasive than liver transplantation, a current treatment option for many disorders associated with fatty acid metabolism defects, reducing risk of complications and/or morbidity
- ◆ Treatment would eliminate the cumbersome daily requirements of patients to rigidly monitor food intake, improving daily quality of life

The Technology

This technology will most benefit individuals with genetic mutations that result in low or non-existent levels of enzyme activity. Fatty acid oxidation disorders such as Medium-Chain Acyl-CoA Dehydrogenase Deficiency, Short Chain 3-Hydroxyacyl-CoA Dehydrogenase Deficiency, and Very Long Chain Acyl-Dehydrogenase Deficiency, are inherited metabolic conditions that lead to an accumulation of fatty acids, and a decrease in cell energy metabolism. This protocol should provide long-term treatment, greatly improving quality of life for patients suffering from a variety of rare fatty acid metabolism disorders.

contact

Anita Rao
University of Florida
Office of Technology Licensing
352/392-8929 • email: arao@ufl.edu

Reference UF #10643 ~ Patent pending



UNIVERSITY OF
FLORIDA

Office of Technology Licensing

*Facilitating Technology Transfer
To Serve Faculty and Community*

www.otl.ufl.edu