UNIVERSITY OF FLORIDA *seeks partner to license*

Gene Therapy for Phenylketonuria (PKU)

Phenylketonuria (PKU) is a common human birth defect in which patients lack the enzyme phenylalanine hydroxylase (PAH) needed to breakdown the essential amino acid phenylalanine. Because non-treatment results in mental retardation, all newborns in the United States are screened for PKU. The only existing treatment for PKU is a dietary restriction of protein; however, this diet is expensive, unpleasant and not completely effective. Even with strict compliance with the diet, stress and disease can cause residual problems due to generation of excess phenylalanine levels. University of Florida researchers have developed a novel gene therapy treatment for PKU which may provide an alternative to the PKU diet and result in a lifelong cure for patients.

Applications

Potential treatment for phenylketonuria (PKU)

Advantages

- Allows for long-term control of all sources of phenylalanine, allowing patients to live a normal life style
- Eliminates the need for expensive and unpleasant dietary restriction, saving patients money and reducing inconveniences
- Prevents problems associated with excess serum phenylalanine, reducing the risk of mental retardation and illness

The Technology

University of Florida researchers have developed a novel gene therapy vector for treatment of PKU. Researchers created a recombinant adeno-associated virus (rAAV) vector which carries a copy of the gene for PAH. Using a mouse model for PKU, researchers delivered the vector into the liver of male mice. The vector normalized serum phenylalanine levels, and resulted in a long-term cure for the disease in treated male mice. Further studies are necessary; however, this invention provides hope for a gene-replacement cure for those suffering from PKU.

contact

Elizabeth Garami
University of Florida
Office of Technology Licensing
352/392-8929 • email: egarami@ufl.edu

Reference UF #10834 ~ Patent pending

UNIVERSITY OF FLORIDA

Office of Technology Licensing

Facilitating Technology Transfer To Serve Faculty and Community

www.otl.ufl.edu