

September 10, 2019

Cure RTD supports development of RTD mouse models by Jackson Laboratory (JAX).

Cure RTD is pleased to announce that in partnership with Jackson Laboratories (JAX - www.jax.org) development has started on mouse models of Riboflavin Transporter Deficiency (RTD).

While it is known that riboflavin transporters are responsible for the transport of riboflavin in the body, the exact mechanism by which riboflavin deficiency causes damage in RTD is not understood. The development of this mouse animal model with RTD will enable the investigation of these mechanisms, in an effort to subsequently develop other novel therapeutic strategies for RTD.

Using the latest CRISPR gene editing technology, mice are being created to carry two very common gene mutations causing RTD. This will be the first RTD mouse model to carry genetic mutations that are the human equivalent to the SLC52A2 gene mutations causing RTD type 2.

This mouse model is being created in connection with the RTD gene therapy project headed by Dr. Steven Gray, PhD, at University of Texas (UT) Southwestern in Texas, which is being funded by the Cure RTD Foundation. RTD mice will be used to show the safety and effectiveness of RTD gene therapy, which is a necessary step before human clinical trials can begin.

Cure RTD has committed to funding research by Jackson Laboratories for the validation and study of these RTD mice. The RTD mouse model, once created and validated, will also be made available to researchers around the world interested in studying RTD.

About Jackson Laboratories

Founded in 1929, The Jackson Laboratory (JAX) is a nonprofit biomedical research institution that seeks to understand the human organism — in all its biological and genomic complexity — by using the mouse as a model. JAX is the world's source for more than 8,000 strains of genetically defined mice and is the home of the Mouse Genome Informatics database. Their research breakthroughs have helped form the foundation of modern medicine. Organ and bone marrow transplants, stem cell therapies, and in vitro fertilization all have a foundation in JAX research.