Public Announcement

In commemoration of Rare Disease Day on February 28th, we are excited to share that our international research team is the recipient of the prestigious global Jeffrey Modell Foundation Translational Research Program Award (Cycle 10). The study entitled, "From clinical and molecular characterization to CRISPR genome-editing therapy of RAG1 combined immunodeficiency", is a close collaboration between the teams of Dr. Ayal Hendel at Bar-Ilan University and Dr. Raz Somech at Edmond and Lily Safra Children's Hospital in Israel, and Dr. Jolan Walter University of South Florida at Johns Hopkins All Children's Hospital and Dr. Kevin Strauss at Clinic for Special Children in the United States.

Our research concerns developing a gene editing technique in the laboratory for patients inheriting a combined immune deficiency due to a defective recombination activating gene 1 (RAG1). This gene functions in the normal development of T and B cells of the immune system. Patients with RAG1 deficiency may have prolonged or recurrent infections, and/or immune dysregulation appearing as autoimmune or inflammatory disorders, which could be fatal if not treated. Restoring the function of this gene in the laboratory could eventually lead to marked improvement or even a cure in patients with this serious genetic immune deficiency.

At the University of South Florida, Joseph Dasso, MD, PhD, has spearheaded the grant writing process with Jolan Walter, MD, PhD, and Krisztian Csomos, PhD, will lead B cell receptor repertoire investigations. The team of Ayal Hendel, PhD, will perform genome editing of RAG1 combined immunodeficiency, whereas that of Raz Somech, MD, PhD, including Yu Nee Lee, PhD, will investigate T cell receptor repertoires. The clinical team of Kevin Strauss, MD at the Clinic for Special Children will work with patients from the Mennonite and Amish communities in the United States. Collaborators at other national and international institutions may also contribute patients to our study, which could not be possible without many clinicians and scientists working together around the globe. We thank the families, our research and clinical team members, and the Jeffrey Modell Foundation.

See the latest publication of Drs. Hendel and Somech related to the technical approach in our study.
https://www.sciencedirect.com/science/article/pii/S2162253122003237?via%3Dihub