

## Translational Research Program Recipients

The Translational Research Program provides support for innovative and novel investigations in Primary Immunodeficiency. We aim to encourage researchers and physicians from all over the world to apply to enhance the understanding and knowledge of the immune system.

The Jeffrey Modell Foundation has long been a global advocate for the awareness and education of Primary Immunodeficiency. We are a cross-continental team, made up of a vast group of brilliant people, each with their own master craft. In order to keep our team as forward-thinking as possible, we created the Translation Research Program to further strengthen our community.

Grants are peer-reviewed and scored by the JMF Research Committee, which consists of physicians and scientists who have an extensive depth of knowledge of Immunology.

All of our awardees are wonderful additions with remarkable talent whose contributions are sure to be invaluable and to bring us one step closer to more meaningful treatments and cures for Primary Immunodeficiency. Below are the recipients and awarded proposals from the program to date:

### Cycle One Recipients:

- One physician from Descartes University Laboratory of Human Genetics of Infectious Diseases, Hôpital Necker-Enfants Malade in Paris, France, has been honored with an award for the proposal titled *"Novel Primary Immunodeficiencies Underlying Invasive Fungal Disease"*.
- Another physician from Harvard Medical School, Children's Hospital Boston in Boston, Massachusetts, will also receive an award for the proposal titled *"Development of a Genomic Platform for the Sensitive Diagnosis of Primary Immunodeficiencies"*.

### Cycle Two Recipients:

- One physician from Pediatrics Clinic and Institute for Molecular Medicine, in Brescia, Italy, will conduct work based on the proposal entitled *"Genotype-phenotype correlation and functional in vitro studies in XLA patients: evaluation of predictive parameters for clinical outcome and prognosis"*.
- Another physician from General Pediatric Department, Pediatric Immunology Services, "Edmond and Lilly Safra" Children's Hospital, Sheba Medical Center, Tel Hashomer, Israel was awarded for the proposal titled *"Understanding the genetics and pathomechanisms of primary Immunodeficiencies of neutrophils in Israeli and Palestinian patients – bridging between two cultures"*.
- The third honoree, from Children's Research Institute in Washington D.C., USA, will be conducting research based on the proposal titled *"Adoptive Immunotherapy for Treatment of Viral Infections in Primary Immunodeficiency Disorders"*.

### Cycle Three Recipients:

- One physician from the Experimental Laboratory Immunology, Department of Microbiology and Immunology, Catholic University of Leuven, Belgium will conduct research based on the proposal *"Unraveling defects in the Toll-like receptor and NF- $\kappa$ B pathway"*.
- The second physician from Baylor College of Medicine, Texas, USA was awarded for the proposal titled *"An Emerging Paradigm: Novel Primary Immunodeficiency Diseases Caused By Two-Gene Defects"*.

### Cycle Four Recipients:

- One physician from the Cincinnati Children's Hospital in Ohio, USA, will conduct research based on the proposal *"Mechanisms of LRBA-mediated immune regulation"*.

- The second award was presented to physicians from CeMM in Vienna, Austria, and Erasmus MC in Rotterdam, Netherlands will focus on *“New insights into DNA repair disorders: integrating genomics and functional studies for developing diagnostic and therapeutic approaches”*.

**Cycle Five Recipient:**

- This honoree from Bellvitge Biomedical Research Institute (IDIBELL) from Barcelona, Spain will conduct research based on the proposal entitled *“Assessing Epigenomic Heterogeneity and its Pathological Consequences in Common Variable Immunodeficiency”*.

**Cycle Six Recipient:**

- The cycle six honoree from the University of Colorado, Denver, Children’s Hospital of Colorado in Aurora, Colorado will conduct research based on the proposal, *“Understanding genetic and immune cellular-signaling defects in common variable immunodeficiency (CVID) with granulomatous lymphocytic interstitial lung disease (GLILD)”*.

**Cycle Seven Recipients:**

- One physician from San Raffaele Telethon Institute for Gene Therapy, in Milan, Italy, will conduct work based on their proposal entitled *“Preclinical Development of a Hematopoietic Stem Cell Gene Therapy for Adenosine Deaminase 2 Deficiency”*.
- The second honoree from Children's Hospital of Philadelphia in the United States was awarded for their proposal titled *“Dissecting B-cell hematopoiesis in PU.1-mutated agammaglobulinemia patients”*.

**Cycle Eight Recipient:**

- One physician from Washington University School of Medicine in the United States was awarded for their proposal entitled *“Immune mechanisms of a novel primary immunodeficiency associated with TLR8 gain-of function”*.

**Cycle Nine Recipients:**

- One physician from University of California, Los Angeles (UCLA) in the United States was awarded for their proposal entitled *“Gene Therapy for CD3delta Severe Combined Immune Deficiency”*.
- The second award was presented to physicians from NIH and Columbia University Irving Medical Center in the United States, and The University of British Columbia in Canada on *“Broadening our collective understanding of inborn errors of immunity—a new disorder caused by loss-of-function variants in OSMR”*.

**Cycle Ten Recipients:**

- One physician from New York University Grossman School of Medicine in the United States was awarded for their proposal entitled *“Mechanistic and Therapeutic Analyses into UBA1 and VEXAS Syndrome”*.
- The second award was presented to physicians from Bar-Ilan University and Edmond and Lily Safra Children’s Hospital in Israel, and University of South Florida at Johns Hopkins All Children’s Hospital and Clinic for Special Children in the United States on *“From clinical and molecular characterization to CRISPR genome-editing therapy of RAG1 combined immunodeficiency”*.

**Cycle Eleven Recipients:**

- Erasmus University Medical Center, Rotterdam, Netherlands, *“Towards targeted treatment for granulomatous lymphocytic interstitial lung disease”*
- Johns Hopkins University School of Medicine, Baltimore, MD, USA, *“Expanding the genetic landscape of inborn errors of immunity (IEIs) using novel genomics strategies”*

- UCL Great Ormond Street Institute of Child Health, London, UK, “A better donor system to mediate targeted integration in hematopoietic stem cells of a therapeutic gene for the treatment of X-linked Agammaglobulinemia”

**Cycle Twelve Recipients:**

- The Regents of the University of California, San Francisco, San Francisco, CA, USA, “Elucidating a Novel PIRD Gene”
- The Children's Hospital of Philadelphia, Philadelphia, PA, USA “Non-coding DNA and antibody deficiency diseases”

If you would like any additional information about the JMF Translational Research Program, please contact the Jeffrey Modell Foundation’s Scientific Director at [research@jmfworld.org](mailto:research@jmfworld.org).