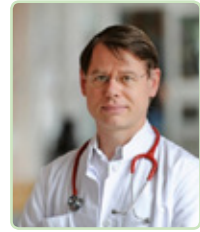


Second Laureate Applied Research

- **Research Work Title:** From therapeutic orphans to pioneers of personalized medicine – international alliance for children with rare diseases of the immune system
- **Researcher:** Prof. Christoph Klein
- **Country:** Germany
- **Field:** Pediatric
- **Scientific Affiliation:** Ludwig Maximilians University, Munich



Abstract:

In collaboration with Iranian pediatric centers, our lab is engaged in studying rare human variants of the immune system to provide novel insights into mechanisms governing inflammation and leukemogenesis. We propose to analyze children suffering from congenital neutropenia and to determine underlying genetic defects. In a complementary approach, we plan to use a genome-wide RNA-interference screen in an attempt to unravel critical factors controlling endoplasmatic reticulum stress in myeloid cells, a fundamental aspect of increased cellular death during myeloid cell differentiation in severe congenital neutropenia. Finally, we will mechanistically explore a novel genetic factor causing congenital neutropenia in human patients by studying a new murine model system. Overall, our studies will continue to build scientific and clinical bridges between Germany and various Iranian centers of excellence by unraveling as yet unknown pathways governing the differentiation and function of the innate immune system, a prerequisite for the development of personalized therapies. These studies will contribute to our global care-for-rare alliance, aiming to support the mission that no child should die of its rare disease, regardless of national and ethnic and financial considerations.

Biography:

Professor Christoph Klein, MD PhD, is Director of the Dr. von Hauner Children's Hospital at the Ludwig-Maximilians-Universität München, where he oversees an extensive clinical and research portfolio devoted to preventing, diagnosing, and treating children with rare and common diseases. After completing subspecialty training in pediatric immunology (Hôpital Necker Enfants Malades, Paris) and pediatric hematology/oncology (Boston Children's Hospital, Harvard Medical School), he held faculty appointments at Harvard Medical School and Hannover Medical School before being nominated chair- man of the Department of Pediatrics at LMU. Christoph Klein and his team have made seminal contributions to the understanding of how blood and immune cells develop and control immunity and tolerance as well as to the development of novel cell- and gene-based therapies. He is a member of many scientific societies and recipient of numerous prestigious national and international awards. He is the founding spokesman of the German research networks on rare diseases as well as co-founder and principal architect of the international Care-for-Rare Foundation.