ISCT TALKING WITH GIANTS

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International Society ISCT Cell & Gene Therapy®

ISCT Career Achievement Award – Donald Kohn, MD

The International Society for Cell & Gene Therapy is proud to present the 2021 Career Achievement Award to Donald Kohn, MD, for his service to the field throughout a dedicated career.

Dr. Donald Kohn is a world-recognized expert in blood stem cell treatments and gene therapy. Through decades of work as a clinical leader and scientific investigator, he has made significant contributions that have advanced the state of gene therapy, establishing landmarks in proof-of-concept for treatment in genetic blood diseases.

Dr. Kohn is credited with pioneering innovative clinical methods to treat genetic blood diseases, through modified blood stem cells, establishing a proven standard of care for Severe Combined Immunodeficiency (SCID), otherwise known as "Bubble Baby" disease. "We are now treating infants and children with SCID using gene therapy and most are appreciating greatly improved well-being and are growing up healthy. That's about as exciting as scientific research can get." Beyond drastic improvements in the outcomes of over 50 pediatric patients, Dr. Kohn's work has provided the clinical world with the groundwork to develop further blood-based gene therapies. Indeed, Dr. Kohn's research continues to progress the field through trials targeting x-linked chronic granulomatous disease, and sickle cell disease – two conditions that have severe and lifelong effects on patients.

Looking back, Dr. Kohn describes the scientific advancements that enabled his groundbreaking research. "It's important to recognize how many things come together to allow for a successful therapy. Leading up to my own work, I look back to discoveries in molecular biology – from the fundamentals in DNA structure to more recent innovations in gene cloning; retroviruses – including their identification, elucidation of provirus integration, and the development of viable vectors; hematopoietic stem cells – from identification to transplantation, transduction, and processing; and gene therapy – from the earliest theoreticians and practitioners – these advancements enabled the success of my team's clinical research." It goes without saying that developing the expertise needed to deploy such methods requires a breadth of experience.

Dr. Kohn's early career aligned with the inception of gene therapy as a field. His early experiences included the combination of a residency focused on treatments in pediatric immunology, under the guidance of Dr. Richard Hong, and a fellowship in 1985 within the lab of Dr. Mike Blaese, which, coincidentally, was in the process of spearheading a research collaboration focused on SCID. Within this environment, Dr. Kohn would find himself working hands-on with one of the first retroviral vectors with the ability to effect gene editing in a T-cell line – resulting in a publication showcasing the early promise of gene therapy. Though there was no functional treatment yet developed, this early proof of concept would help build the groundwork for Dr. Kohn's breakthrough years later.

Diversity of experience, Dr. Kohn notes, is key. For young professionals, he observes, "Attaining multidisciplinary training in cell biology, molecular biology, cell therapy, biostatistics and bioinformatics, bioengineering, clinical research, and so on, is a great way to prepare to get your hands on performing this kind of novel research." Over the coming years, Dr. Kohn anticipates key breakthroughs in the precision and effectiveness for cell and gene-based therapeutics, perhaps stemming from developments in Cas9, Base Editors, and Primer Editors, among others. To those joining the field, he says, "Work hard, be nice to those around you, and enjoy it." Dr. Kohn's own early experiences demonstrate that finding new connections not only in scientific data, but also in between scientists themselves, and the teams they form, can build novel outcomes.

Beyond his research, Dr. Kohn's leadership in the field has demonstrated a consistent passion for the science, and for giving back to the community. Working across the field, Dr. Kohn has mentored over 50 early-career professionals, many of whom now have become key opinion leaders in their own right. He has held an extensive set of volunteer roles in multiple scientific societies, including serving as past president of ASGCT, the American Society of Gene and Cell Therapy, and serving on the ISCT Immuno & Gene Therapy Committee.

Collaboration and selflessness have long been at the heart of Dr. Kohn's work, and the great influence of his career on our global community speaks for itself. His dedicated contributions to his patients, colleagues, and to the field at large upholds the scientific spirit of giving back to the world.

For his contributions to scientific innovation, groundbreaking research, dedicated volunteer service, and devoted mentorship, ISCT is proud to present Dr. Donald Kohn with the 2021 ISCT Career Achievement Award.