



ISCT TALKING WITH GIANTS

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

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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.



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Darwin J. Prockop Mentoring Award – Catherine Bollard, MBChB, MD

The International Society for Cell & Gene Therapy is proud to present the inaugural 2021 Darwin J. Prockop Mentoring Award to Catherine Bollard, MBChB, MD, for her dedication and service to her colleagues and the field.

Dr. Catherine Bollard is a world-renowned expert in immunotherapies for cancer, whose work focuses on the deployment of cell therapies to strengthen the immune system against cancer and other inflammatory diseases. In conjunction with her leading-edge work in the lab and clinic, Dr. Bollard has demonstrated a consistent drive to enhance the opportunities and growth of those who work with her through mentorship, and has been a key leader in the cell and gene therapy space through roles across several major organizations including FACT presidency and ISCT past presidency.

But how did she get here?

Dr. Bollard's passion for cell and gene therapy stems from a painful moment, when her best friend in high school had passed away due to complications from EBV+ Hodgkin's lymphoma treatment. Despite entering remission, the treatment options available at the time had difficult side effects and outcomes for patients. The story of her best friend would drive Dr. Bollard to work with cellular therapies, and eventually, to work with several established mentors in the field: Drs. Helen Heslop, Cliona Rooney, and Malcolm Brenner.

Within this team, Dr. Bollard would find early successes, including a novel and successful cell-based therapy for Hodgkin's lymphoma.

Thinking back, she attributes the effectiveness of this team, and the momentum built from her early success, to the selflessness of several mentors who provided advice not only around careers and science, but also around life. "In working with translational science, especially in cell and gene therapy, a cohesive and effective team is critical," she says. "Part of this lies in having a breadth of scientific expertise, but team effectiveness is also built on mutual trust, support, and on mentors being ready to pitch in with 'boots on the ground'."

Dr. Bollard recalls this perspective being driven home by a particularly poignant moment in her life, when her father had passed away just before the deadline of her first POI as a co-principal investigator. During a moment of difficult grief and complex deadlines, her mentors Helen, Malcolm, and Clio stepped in with 'boots on the ground,' working to help edit, write, and offer both project-based and emotional support to ensure that the critical project deadline could be met.

Moments like these continue to inspire Dr. Bollard's approach to mentorship today. Recognizing that early career life comes with balancing commitments in writing, scientific analysis/design, long-term goal setting, and financial and emotional challenges, she adopts a supportive approach based on building trust, being consistently ready to step in, and

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prioritizing the mentee, especially when it comes to career-builders like authorship.

“Watching my mentees (clinical, basic, and translational) give their first talks as grad students/fellows and then seeing their rise to symposium speakers at national and international meetings is so rewarding,” says Dr. Bollard, reflecting on the impacts of being a mentor. “...at a personal level, listening to the struggles we all face in science and medicine and balancing the stressors of home life and work, and just taking the time to listen and supporting them through the process brings true meaning to my role as a mentor.”

Thinking generationally across cell and gene therapy, Dr. Bollard points to the need for mentors and their mentees to stay grounded: “...in our field, it was hard to find a critical mass of (especially) academic physician-scientists in the generation ahead of me who had a durable marriage/life partner relationship, children, NIH-funded laboratories, and clinical responsibilities,” she reflects, thinking towards life around the lab. “While we are still in the minority, there are more of ‘us’ in my generation to build that ‘critical mass,’ which I see as so incredibly important as we groom the next generation of scientists and physician-scientists to develop and lead the next

‘first in human’ cell and gene therapy trials from bench to bedside and beyond.” While the science races ahead, Dr. Bollard stresses that it is critically important not to lose sight of the people behind the profession. For mentors in particular, being mindful of the factors that build resilient lives outside of the lab will mean that teams develop trust, and become more effective in their mission.

She ends with a few words to take away, “No matter what one chooses to do as a career, it is critically important that you are, above all, passionate about what you do and the career choices you have made, as well as your vision for the future. This will get you up in the morning, and hopefully, inspire your mentees to build the same degree of passion and vision for the work! A successful career is always a combination of luck, serendipity, and a lot of hard work and we must always learn to roll with the ‘good’ and the ‘bad’, understanding that sometimes good things may come easily, and other times, they may not. And, for sure, sometimes everything may seem to be depressingly hard, especially around manuscript or grant rejections. But at the end of the day, don’t lose sight of what inspires your passion. And never forget to take the time to sit back, listen, and above all, to have fun.”



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Le Prix Luc Sensebé Innovation Award – Keith Thompson CBE

The International Society for Cell & Gene Therapy is proud to present the inaugural 2021 Le Prix Luc Sensebé Innovation Award to Keith Thompson CBE, for his service to the field by building the groundwork for sustained innovation.

Mr. Keith Thompson is a renowned leader in the cell and gene therapy space, known especially for his contributions as founding CEO of the Cell and Gene Therapy Catapult, based in the United Kingdom. Under his vision, CGT Catapult developed and executed exponentially successful strategies to encourage dialogue that today have established the UK as an international site of cell and gene therapy expertise and standards.

From childhood, Mr. Thompson knew that he wanted to be a scientist or engineer. Driven by curiosity, any gadgets that found their way into his childhood home would be investigated – dismantled, but not always reassembled in time to avoid detection and trouble. This driving motive would bring a young Keith Thompson to develop, a few years into his first industry job, immunoaffinity purification for beta interferon – an innovation he was soon asked to ‘scale up’.

This would launch him into the exciting world of a developmental pilot plant, to him a “veritable palace of stainless steel.” In this space, Mr. Thompson says, “I was hooked. I knew that if you wanted to see these innovations in use, you had to be able to make them

first. Innovation was the practical implementation of ideas that could result in the introduction of new products.”

Over time, Mr. Thompson built a wealth of knowledge as his career progressed. He learned to gauge the practicalities of innovation, especially in a pragmatic market. He saw how regulators, as scientists themselves, would be fascinated by new products, and the ways to prove their efficacy. He began to see the need to change systems, from clinical trials to manufacturing, in order to ensure that innovation could take place.

As his perspective continued to grow, Mr. Thompson would see cell and gene therapies as a field that held the right balance of compelling advancements for treatment – and which had major barriers that could be addressed strategically.

“You can develop a clear mission and strategy that structures activities to systematically attack complex barriers to change, where carefully chosen pathfinder products can clear the way for others,” says Mr. Thompson. “I set up a system at the Catapult to address the three great barriers to the CGT revolution by forming innovation teams, investigating: 1. Clinical/Regulatory; 2. Manufacturing and Cost of Goods; and 3. Health Economics and Business Systems. Each team, led by a highly motivated leader, worked on clinical pathfinder projects designed to flush out the real industry barriers so that we could address them.”

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Mr. Thompson highlights clinical proof of principle as a key factor preceding his strategy. “Prior to founding the catapult, when I was leading the Scottish National Blood Service, we had expanded a cell therapy R&D program established by Mark Turner, and found great success with multiple treatments. That had convinced me around 2010 that cell therapy was the next big thing, leading me to leave to start the Catapult in 2012. The most notable clinical success, however, was the well known Emily Whitehead story with Carl June and his team at the University of Pennsylvania. Not only did CAR-T work – it also provided a framework for many issues in the field to be addressed, alongside a level of interest that lifted the whole industry.”

For Mr. Thompson, clinical success is the key outcome of the innovation cycle – when proof of principle, regulatory approval, and positive health economics evaluation come together to introduce a widespread adoption of a therapy. Alongside this, Mr. Thompson finds enthusiasm in witnessing the expansion of cutting-edge manufacturing, and the correlated opportunities for young professionals to find their path there.

Over the next few years, Mr. Thompson anticipates the need to optimize cell and gene therapies – making them cheaper, faster, and better – as the critical step in making them a ‘pillar’ of pharmaceutical intervention. “The field is simply so enormous so young professionals should have many and broad opportunities presented to them, just like when I started out in Monoclonals over 40 years ago. There will be multiple opportunities for innovation, and it will be a long time until the field settles into an established paradigm.”

Mr. Thompson reflects back on his leadership experience with good humour, and attributes a large part of his success to those around him. “Always hire people who are cleverer than you; in my case, it wasn’t hard,” he laughs. He ends with a few words of wisdom, “try to stand in the future to look back at the problems you are facing. The future is where you will be – so try to shape it to be one that looks good.”