Primary Immune Deficiency Treatment Consortium

NEWSLETTER

Summer 2026 | Issue 24

Group Photo at the 15th Annual PIDTC Scientific Workshop in Salt Lake City, Utah

Greetings from Drs. Rebecca Marsh, Chris Dvorak, Elie Haddad, and Troy Torgerson, Multi-PIs

Hello PIDTC Members,

We hope that everyone is gearing up for some well-deserved time off over the upcoming summer months.

I think all would agree that our annual meeting in Salt Lake City was an enormous success and much thanks to Mike Pulsipher and our Utah hosts. We are already starting to plan an exciting meeting for next spring in uptown New York City, hosted by Joe Oved.

We've also been busy with modernizing knowledge transfer at PIDTC. Our PIDTC video podcast, hosted by Jack McDonnell (Cleveland Clinic) and Elie Haddad (St. Justine), has debuted on the PIDTC YouTube channel – with 7 fun and interesting episodes to date! Take some time to check them out!

Last, huge thanks to everyone for continuing to enroll patients. We are very fortunate that our community remains strong and collaborative.

Cheers!

Rebecca, Chris, Elie, and Troy

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PIDTC Education Day & 15th Annual Scientific Workshop in Salt Lake City, Utah from March 17th - 20th, 2026

The PIDTC **Education Day** was held on March 17th-18th, where we once again explored unique aspects of diagnosis and management of primary immunodeficiencies with leaders in the field.

This was followed by our **Scientific Workshop** from March 18th-20th. The workshop was a valuable experience, with the vast majority of attendees rating the content as perfectly balanced between topics and overall “excellent” (100% of respondents).

We are excited to announce that next year’s 16th Annual PIDTC Education Day and Scientific Workshop will be held on March 9-12th, 2027 in New York City, NY. Planning is already underway, more information to come.

Speakers and Presenters

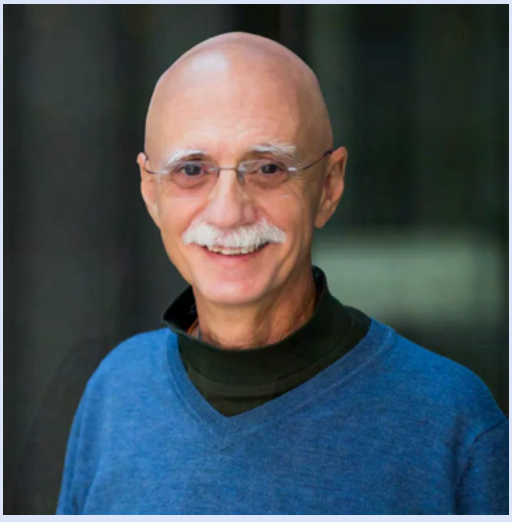
The PIDTC was honored to welcome distinguished leaders in immunology and transplantation who shared their expertise, reflected on major advances in the field, and were recognized for their longstanding contributions to improving outcomes for patients with inborn errors of immunity.

Workshop host Dr. Michael Pulsipher welcomed attendees to the 15th Annual PIDTC Scientific Workshop in Salt Lake City, Utah.

Lifetime Achievement Award

The PIDTC Lifetime Achievement Award recognizes individuals whose sustained leadership, scientific contributions, and dedication have advanced the field of inborn errors of immunity and improved patient care worldwide.

Dr. Luigi ("Gigi") Notarangelo



Dr. Mort Cowan

The PIDTC proudly recognized Dr. Luigi (“Gigi”) Notarangelo and Dr. Mort Cowan as recipients of its 2026 Lifetime Achievement Award, honoring their roles as co-founders of PIDTC and their extraordinary leadership, scientific contributions, mentorship, and enduring impact on the field of inborn errors of immunity.

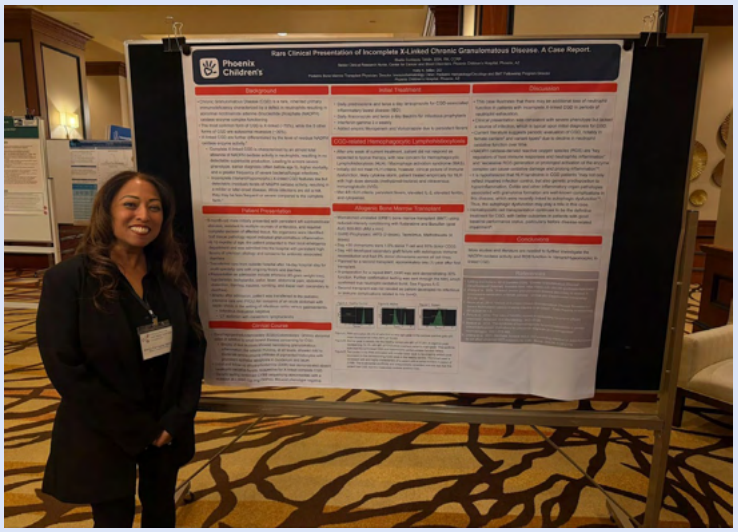
Speakers and Presenters

The PIDTC welcomed distinguished international experts whose contributions highlighted the importance of global collaboration in advancing care for patients with inborn errors of immunity.

Dr. Maria Ester Bernardo, MD, PhD
Associate Professor of Pediatrics, Vita-Salute San Raffaele University
Italy

Dr. Mary Slatter
Associate Clinical Lecturer, Newcastle University
United Kingdom

PIDTC Clinical Research Coordinators attending the 15th Annual PIDTC Scientific Workshop in Salt Lake City, Utah.



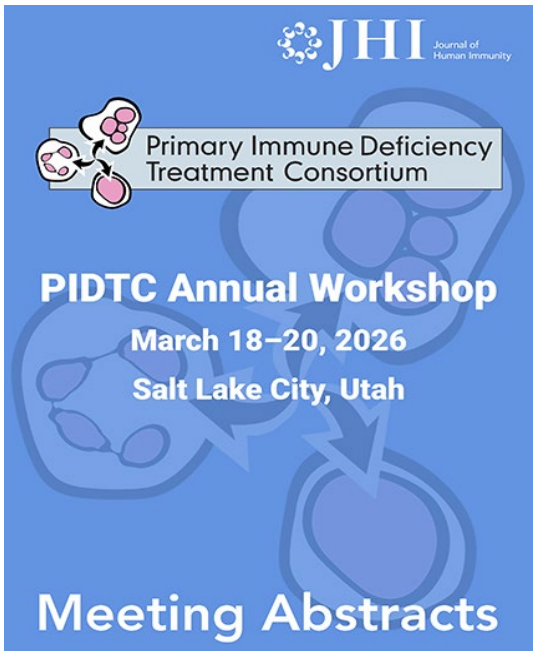
Sheila Contapay-Tabilay, BSN, RN, CCRP, presented her research during the workshop poster session, highlighting the vital contributions of clinical research professionals to advancing patient care and research.

Dinner at the Natural History Museum of Utah

A dinner reception was held at the Natural History Museum of Utah, which houses over two million objects including artifacts, relics, and over 20,000 catalogued dinosaur fossils.

2026 PIDTC Workshop Abstracts Now Available Online

The PIDTC meeting abstracts website is now live through the Journal of Human Immunity, providing online access to the 2026 Workshop abstract collection.



JHI cover for the 2026 PIDTC Workshop Meeting Abstracts

JHI Journal of Human Immunity

Published through the Journal of Human Immunity

About JHI

The Journal of Human Immunity (JHI) is the official journal of the International Alliance for Primary Immunodeficiency Societies (IAPIDS).

JHI is published by Rockefeller University Press.

About the Abstract Collection

The collection includes abstracts presented at the PIDTC Annual Scientific Workshop held March 18–20, 2026, in Salt Lake City, Utah.

Featured work spans case reports, clinical trial outcomes, preclinical studies, and mechanistic investigations in primary immune deficiencies and inborn errors of immunity.

All abstracts were reviewed and approved by the PIDTC program committee.

Explore the PIDTC Workshop Abstracts
https://rupress.org/jhi/issue/2/PIDTC2026

PIDTC New Affiliate Site Members

The PIDTC proudly welcomes its newest Affiliate Site Members. These institutions participate in PIDTC research initiatives, educational activities, and annual scientific workshops through collaboration with established PIDTC member sites. Affiliate Sites help broaden the consortium's reach and support our shared mission of advancing research, education, and patient care for individuals with inborn errors of immunity.

Arkansas Children's
HOSPITALS · RESEARCH · FOUNDATION

JOHNS HOPKINS
CHILDREN'S CENTER

HealthCare
KENTUCKY CHILDREN'S HOSPITAL

CHOC Children's

Monroe Carell Jr.
Children's Hospital
at Vanderbilt®

Mansoura University

HAWAI'I PACIFIC HEALTH
KAPI'OLANI MEDICAL CENTER FOR WOMEN & CHILDREN

UVA Health

University Hospitals
Rainbow Babies & Children's
The Science of Health. *The Art of Compassion.*

OHSU
DOERNBECHER CHILDREN'S *Hospital*

The Children's Hospital at Montefiore

MUSC
Children's Hospital®

UCDAVIS HEALTH | CHILDREN'S HOSPITAL

Acknowledging Amanda Zablocki

PIDTF Board Member | Leadership, Service, and Advocacy for the PIDTC Community

Amanda Zablocki
PIDTF Board Member

About Amanda

Amanda Zablocki, J.D. is a healthcare attorney and co-leader of the Healthcare Industry team at Sheppard, Mullin, Richter & Hampton LLP. Following her son Idan's diagnosis with X-linked Hyper IgM syndrome, Amanda and her husband co-founded the Hyper IgM Foundation, where she serves as Secretary. Her family's experience inspired her commitment to advancing research, advocacy, and care for individuals with inborn errors of immunity.

Supporting the PIDTC Mission

Amanda has generously provided *pro bono* legal guidance to help establish the **Primary Immune Deficiency Treatment Foundation (PIDTF)** as a nonprofit organization dedicated to supporting research and education for the PIDTC and the broader clinical immunology community. Her expertise, leadership, and unwavering commitment have been instrumental in building a strong foundation for the PIDTF's future. On behalf of the PIDTF Board of Directors and the entire PIDTC community, we extend our sincere appreciation and gratitude for Amanda's outstanding leadership, service, and dedication.

Thank you, Amanda, for your dedication to the PIDTF and PIDTC Community.

Learn more at PIDTF.org

PIDTC Podcast *Immune Matters*

Episode 7: Gene Therapy for Inborn Errors of Immunity

YouTube

7 episodes available

Featured Guest
Dr. Donald Kohn

Hosts Jack McDonnell and Elie Haddad with featured guest Dr. Donald Kohn

Episode Highlights

- Advances from early safety concerns to modern lentiviral vectors.
- Emerging tools including CRISPR, base editing, and prime editing.
- Gene therapy applications in ADA-SCID, Wiskott-Aldrich syndrome, CGD, and LAD.
- Barriers to broader access, including cost, regulation, insurance, and the limited commercial market.

About the Channel

The PIDTC YouTube channel features the Immune Matters podcast series, with expert conversations on research advances, clinical care, transplantation, and emerging therapies.

Watch Episode 7

https://youtu.be/7qPAEEY6sn0
Hosted by Jack McDonnell and Elie Haddad

Episode 7 explores how gene therapy is reshaping care for patients with inborn errors of immunity.

Honoring Dr. Neena Kapoor's Legacy

Celebrating a distinguished career in transplantation, immunology, mentorship, and service

Dr. Neena Kapoor

A Lifetime of Contributions

Dr. Neena Kapoor earned her medical degree from Himachal Pradesh Medical College and completed her residency at Rhode Island Hospital/Brown University. She then pursued fellowship training in immunology at Memorial Sloan Kettering Cancer Center and Cornell University under Drs. Robert Good and Richard O'Reilly, where she developed her interest in caring for patients with severe combined immunodeficiency.

Dr. Kapoor played an important role in advancing T-cell depletion approaches for transplantation in SCID. Her landmark work included the use of soybean lectin and sheep red blood cell agglutination for T-cell depletion of haploidentical grafts. In 1981, she was the first to report that a radiation-free busulfan and cyclophosphamide regimen could correct both the immune and platelet defects of Wiskott-Aldrich syndrome, establishing a conditioning approach used for decades.

After helping develop transplant programs at several institutions, Dr. Kapoor joined Children's Hospital Los Angeles, where she spent more than 30 years as Director of the Blood and Marrow Transplant Laboratory and Professor of Clinical Pediatrics at the Keck School of Medicine of USC. She also helped establish CHLA as a leading center in TCR alpha/beta and CD19-depleted transplantation.

A longstanding PIDTC member and site principal investigator, Dr. Kapoor has made lasting contributions to the consortium's mission. We wish her the very best in retirement and look forward to seeing her at future PIDTC meetings.

PIDTC Working Group Updates

The PIDTC Working Groups continue to advance collaborative research through growing enrollment, protocol development, mechanistic studies, and manuscript preparation. The following highlights summarize recent progress across PIDTC 6906, 6907, and 6908.

PIDTC 6906 – Primary Immune Regulatory Disorders

190	27	32	21
Patients Enrolled	Participating Sites	Genetic Diagnoses	No Identified Gene

Enrollment continues to grow across a diverse cohort of patients with primary immune regulatory disorders. These data strengthen the consortium's ability to characterize the clinical and genetic spectrum of PIRD.

- Initial regulatory T-cell mechanistic studies are underway, with additional studies planned.
- The first manuscript describing the clinical characteristics of the initial cohort is in preparation.
- A protocol amendment is being developed to expand eligibility and facilitate enrollment of patients who have been difficult to include under the current criteria.

PIDTC 6907 – Severe Combined Immunodeficiency

274	22	80	141	31
Eligible Participants	Cohort 1	Cohort 2	Cohort 3 Rollovers	Cohort 4

Protocol and Study Updates

The study committee is developing an amendment to simplify consenting, with the goal of allowing enrollment through a single consent whenever possible.

- **Closed sub-studies:** CMV maternal-fetal transmission, TCRVβ/TREC, and T-cell exhaustion. Sites should no longer send samples for these projects.
- **Open studies:** mechanistic evaluation of intrinsic thymic defects in genetically undefined SCID and pharmacokinetic/pharmacogenetic studies of conditioning agents in young infants.
- **Enrollment priority:** eligible Cohort 3 rollover participants previously enrolled in PIDTC 6901 or 6902.

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Recent Publications

Winestone L, Logan B, Liu X, et al. "Newborn Screening Reduces Survival Disparities in SCID After Stem Cell Transplant: A PIDTC Report." *Journal of Human Immunology* (2026), accepted manuscript.

Rayes A, Logan BR, Liu X, et al. "Outcomes Following Matched Sibling Donor Hematopoietic Cell Transplantation for Severe Combined Immunodeficiency: A Report from the PIDTC." *Blood Advances* (2026). PMID: 41289158

PIDTC Working Group Updates

PIDTC 6908 – Chronic Granulomatous Disease

Protocol Development

The working group is reviewing the current protocol in preparation for a coordinated amendment. Areas under review include outdated language, institutional affiliations, study procedures, reporting expectations, and other clarifications needed to align the protocol with current practice and support consistent implementation across sites.

Research and Manuscript Progress

- The HLA-mismatched donor manuscript is undergoing final review before submission.
- The clinical outcomes manuscript is nearing completion.
- The microbiome manuscript draft will circulate shortly.
- The CGDAA manuscript is being prepared for Steering Committee review.
- A second-transplant outcomes project remains in development.
- A quality-of-life manuscript is planned for publication in Frontiers.

Enrollment and Reporting

Enrollment reporting is being refined to distinguish cohort, site, rollover status, eligibility status, and true enrollment from placeholder records. Cohort 1 enrollment remains the highest priority, as the protocol is powered for 80 participants. Revised reporting will help guide targeted outreach to sites and better identify true accrual gaps.

Looking Ahead and Site Collaboration

The working group will use revised enrollment reports to tailor site outreach, clarify cohort-specific priorities, and support completion of historical and prospective data. Continued collaboration among investigators, coordinators, and site teams remains essential to advancing CGD research, completing active manuscripts, and strengthening the long-term value of the 6908 registry.

Thank You

The PIDTC sincerely thanks all participating investigators, clinical research coordinators, study coordinators, central reviewers, and participating sites for their continued efforts in identifying eligible patients, completing study forms and eligibility reviews, and supporting this collaborative research network.

PAG Updates

Jeffrey Modell Foundation

Advancing Artificial Intelligence and Global Collaboration
The Jeffrey Modell Foundation (JMF) continues to lead global efforts to improve the diagnosis and care of individuals with primary immunodeficiencies through education, research, artificial intelligence (AI), and international collaboration.

The Foundation recently announced the publication of the **Proceedings of the Second Artificial Intelligence in Primary Immunodeficiency (AI4PI) Meeting** in the *Journal of Allergy and Clinical Immunology (JACI)*. The publication highlights collaboration among more than 30 experts from 16 countries and 30 U.S. states and explores how AI can support earlier diagnosis, clinical decision-making, and improved patient outcomes.

Building on this work, JMF continues to expand its **SPIRIT (Sequencing, Population Health, Intelligence, and Research Integration Technology) Platform**, which integrates genomic, electronic health record, and healthcare claims data to identify individuals at risk for primary immunodeficiencies. These efforts aim to reduce diagnostic delays and accelerate access to genetic testing and specialized care.

Education and Professional Development
JMF continues to provide educational opportunities through its internationally recognized **JMF Speaker Series**. Recent presentations have focused on advances in the diagnosis and treatment of inborn errors of immunity, bringing together experts to share current research, clinical experience, and evolving standards of care.

JMF has also launched a new **Artificial Intelligence Speaker Series**, highlighting applications of AI in immunology, precision medicine, and rare disease diagnosis. The series promotes collaboration among clinicians, researchers, and technology experts while exploring how machine learning can support diagnostic accuracy and clinical decision-making.

Supporting Patients and Families Worldwide
Beyond its educational programming and AI initiatives, JMF continues to expand patient and family engagement through its **Genetic Sequencing Program**, **World PI Week** activities, and the newly established **Patient Family Advisory Board**. These efforts raise awareness, encourage earlier recognition of warning signs, and ensure that patient and family perspectives inform research, education, and advocacy.

As JMF continues to expand its global initiatives, the Foundation remains committed to fostering collaboration among clinicians, researchers, patient advocacy organizations, and families. Through innovation, education, and the responsible application of artificial intelligence, JMF is helping shape the future of primary immunodeficiency diagnosis, research, and patient care.

For more information about JMF click here: https://www.info4pi.org/

Wiskott-Aldrich Foundation

First-ever International Study on the Clinical Spectrum of WAS Carriers

First-Ever International Study on the Clinical Spectrum of WAS Carriers
The Wiskott-Aldrich Foundation is proud to share the publication of the first-ever international study examining the clinical spectrum of Wiskott-Aldrich Syndrome (WAS) carriers. Conducted in collaboration with the PIDTC and the Immune Deficiency Foundation (IDF), this landmark study provides important insight into the health experiences of carriers and establishes a foundation for future research.

The Foundation extends its gratitude to the carriers who participated in the survey and helped develop and refine the study. While this publication represents an important step forward, additional research is needed to better understand long-term health outcomes, identify potential risks, and determine best practices for evaluating carriers as potential stem cell donors. https://doi.org/10.1016/j.clim.2025.110658.

FDA Approval of Waskyra™ Gene Therapy
The FDA recently approved Waskyra™, the first gene therapy approved in the United States for Wiskott-Aldrich Syndrome. This historic achievement represents decades of scientific innovation, clinical research, and collaboration among investigators, clinicians, advocacy organizations, patients, and families.

The Foundation recognizes the contributions of Fondazione Telethon, SR-TIGET, Dr. Alessandro Aiuti, Dr. Francesca Ferrua, and the many patients and families who participated in clinical trials. Their dedication helped make this breakthrough possible and marks a major advancement in treatment options for individuals living with WAS.
https://www.wiskott.org/About-WAS/treatment-of-was/gene-therapy

Looking Ahead: Expanding Research and Global Collaboration
Building upon these milestones, the Foundation continues to explore future research initiatives focused on WAS carriers and long-term outcomes. Current discussions include the development of an international patient registry in collaboration with the European Society for Immunodeficiencies (ESID), as well as opportunities to incorporate Global Patient ID systems to strengthen data sharing and research coordination across institutions and countries.

Through continued collaboration, advocacy, and patient-centered research, the Foundation remains committed to improving understanding of Wiskott-Aldrich Syndrome and advancing care for affected individuals and families worldwide.

For more information about the WA Foundation click here: https://www.wiskott.org/

Immune Deficiency Foundation

2026 IDF National Conference
The Immune Deficiency Foundation (IDF) successfully hosted its 2026 National Conference in San Antonio, Texas, welcoming nearly 1,000 patients, families, clinicians, researchers, and advocates from across the primary immunodeficiency community. Centered around the theme of diversity, resilience, and community, the conference featured educational sessions, expert presentations, networking opportunities, and patient engagement activities. Feedback from attendees has been overwhelmingly positive, reflecting the conference's continued importance as the largest annual gathering dedicated to primary immunodeficiency.

Research, Education, and Patient Resources
IDF continues to expand educational resources for patients, families, and healthcare professionals. The Foundation recently released a comprehensive **Prior Authorization Resource**, developed in partnership with the IDF Nurse Advisory Committee, to assist providers with insurance approvals, documentation, appeals, peer-to-peer reviews, and demonstrating medical necessity for primary immunodeficiency therapies.

A preview of the **7th Edition of the Patient & Family Handbook for Primary Immunodeficiency** is now available, with additional chapters continuing to be released ahead of the complete edition. The handbook remains one of the Foundation's most widely used educational resources for patients, caregivers, and healthcare professionals.

Supporting Research and Community Engagement
IDF continues to support research through partnerships with investigators and advocacy organizations. As part of these efforts, the Foundation is hosting recruitment for the **MIRACLE Study**, a multicenter research initiative dedicated to understanding fertility, pregnancy, and reproductive health in individuals with genetically driven immune disorders. The study brings together immunologists, obstetrician-gynecologists, maternal-fetal medicine specialists, geneticists, and nurse practitioners from institutions across the United States.

The Foundation also recently welcomed **Dr. Paula Henao** as its Medical Director and continues expanding educational opportunities for patients, families, and healthcare professionals. Planning is underway for the next **Teen & Tween Escape Program**, anticipated for Summer 2027, which provides young people living with primary immunodeficiencies opportunities to build friendships, develop leadership skills, and connect with peers from around the country.

Advocacy for Patient Access
Advocacy remains central to IDF's mission. The Foundation continues working alongside patients, caregivers, clinicians, researchers, and policymakers to improve access to life-saving immunoglobulin therapies and other essential treatments while promoting earlier diagnosis, strengthening patient education, supporting research, and improving health outcomes for individuals and families affected by primary immunodeficiencies.

For more information about the IDF: https://primaryimmune.org/

CGD Association of America

Advancing Standards of Care and Education for the CGD Community
Landmark CGD Clinical Protocol Published
The CGD Association of America (CGDAA) is proud to announce a historic milestone in the clinical management of Chronic Granulomatous Disease (CGD). On April 16, 2026, the first-ever comprehensive clinical protocol for the diagnosis and treatment of CGD was published in the *Journal of Human Immunity*.

Developed through a collaboration between CGDAA and leading members of the Primary Immune Deficiency Treatment Consortium (PIDTC), the publication, *"How I Treat Chronic Granulomatous Disease,"* provides a consensus-based guide for clinicians, patients, and families navigating this rare inborn error of immunity. As the first comprehensive consensus document dedicated specifically to CGD, the protocol represents an important advancement toward standardizing care, promoting evidence-based treatment approaches, and improving outcomes for individuals affected by CGD worldwide.
https://rupress.org/jhi/article/2/3/e20250162/281765/How-I-treat-chronic-granulomatous-diseaseHow-I

Education and Community Engagement
CGDAA continues to support patients and families through educational programming and outreach initiatives. Earlier this year, the organization hosted a highly attended **Coffee & Clinicians** session featuring Dr. Christopher Chang in collaboration with the Lupus Foundation of America. The discussion focused on X-linked carrier experiences, lupus-like symptoms, management strategies, and the role of rheumatologists in diagnosis and long-term care.

These educational events provide valuable opportunities for patients, caregivers, and healthcare professionals to learn from experts and engage in meaningful discussions about CGD-related care and research.
https://youtu.be/BjGxjvz0H2E?si=EStmKBirWZpX-AiC

CGDAA at the 2026 IDF National Conference
CGDAA participated in the 2026 Immune Deficiency Foundation National Conference in San Antonio, Texas, where Dr. Jennifer Leiding of Johns Hopkins All Children's Hospital presented current standards of care for CGD and discussed the emerging role of gene therapy and gene editing as future treatment approaches. The session concluded with an interactive question-and-answer discussion moderated by CGDAA Executive Director Felicia Morton and provided attendees with an opportunity to discuss recent advances in clinical care and research.

Through continued advocacy, education, research, and collaboration with organizations such as PIDTC, CGDAA remains committed to advancing care and improving the lives of individuals and families affected by Chronic Granulomatous Disease.

For more information about the CGDAA: https://cgdaa.org/

SCID Foundation

Education, Research, and Support for SCID Families
The SCID Foundation continues to support individuals and families affected by Severe Combined Immunodeficiency (SCID) through education, advocacy, research, and community engagement. By connecting patients, caregivers, healthcare professionals, and researchers, the Foundation remains dedicated to improving outcomes and quality of life for individuals living with SCID.

Connecting and Supporting the SCID Community
The Foundation's digital newsletter provides educational resources, patient stories, treatment updates, and community news for families and healthcare professionals. Recent issues have featured stories on leaky SCID, treatment for genetically undefined forms of SCID, and experiences from newly diagnosed families. Monthly virtual meetups provide opportunities for families to connect, share experiences, and learn from others navigating similar challenges.

Path Forward Scholarship Program
The SCID Foundation's Path Forward Scholarship Program continues to provide financial support to families navigating treatment for Severe Combined Immune Deficiency. Families undergoing bone marrow transplantation, hematopoietic stem cell transplantation, gene therapy, or receiving Revcovi while awaiting treatment may be eligible for assistance.
To date, more than 20 scholarships have been awarded, helping families offset treatment, travel, and caregiving expenses. The Foundation remains grateful to the donors and sponsors who make this program possible.

SCID Foundation Research Grant Program
The **2026 SCID Foundation Research Grant Program** is now accepting applications. The Foundation will award **one grant of up to $25,000** to support a one-year research project focused on improving the diagnosis, treatment, management, or long-term outcomes of individuals affected by SCID. The application deadline is **September 15, 2026**.

Priority areas include early diagnosis, access to care, definitive treatment, long-term follow-up, reduction of late complications, and strategies to improve outcomes before definitive therapy. Special consideration will also be given to projects addressing **Cytomegalovirus (CMV) transmission through breastfeeding in newborns with SCID** while preserving the benefits of breastfeeding whenever possible.

The Foundation hopes this seed funding will support innovative projects led by fellows, postdoctoral researchers, and junior faculty while advancing meaningful improvements in SCID research and patient care.

For more information about the SCID Foundation, visit: www.scidfoundation.org

IPEX Foundation

Advancing Advocacy, Research, and Family Support for the IPEX Community
The IPEX Foundation continues to expand its efforts to support individuals and families affected by Immune Dysregulation, Polyendocrinopathy, Enteropathy, X-linked (IPEX) syndrome. Through advocacy, education, fundraising, and support for clinical research, the Foundation remains committed to improving outcomes and quality of life for individuals living with IPEX syndrome worldwide.

Leadership, Advocacy, and Awareness
Under the leadership of President Missy Ramirez and Executive Director Julian, the Foundation has entered a period of renewed growth and engagement. Earlier this year, Foundation representatives participated in NIH Rare Disease Day and presented at Stanford Medicine, sharing the patient and family perspective on rare disease research and clinical trial participation. These efforts help increase awareness of IPEX syndrome while highlighting the importance of continued investment in research and patient-centered care.

Supporting Research and Clinical Trials
The Foundation continues to support families participating in Stanford's IPEX clinical trial program. Current efforts focus on helping bridge support and funding gaps while assisting families who travel significant distances to participate in research studies. By working closely with patients, families, clinicians, and researchers, the Foundation helps ensure that individuals affected by IPEX have opportunities to contribute to advances in treatment and care.

Education and Family Support
To improve recognition and diagnosis of IPEX syndrome, the Foundation has partnered with Code Rare to develop educational resources and training materials for healthcare providers. These initiatives aim to increase awareness of IPEX signs and symptoms, promote earlier diagnosis, and improve access to specialized care and support services.
Fundraising efforts continue to play an important role in advancing the Foundation's mission. Through community-driven initiatives and donor support, the Foundation has raised nearly $60,000 to support research programs, patient services, and financial assistance for families facing treatment- and travel-related challenges.

As the Foundation continues to grow, its focus remains on supporting families, advancing research, promoting earlier diagnosis, and strengthening partnerships throughout the rare disease community.

For more information about the IPEX Foundation, visit: www.ipex.foundation

ImmUnity Canada

Advancing Access, Advocacy, and Education for Canadians Living with Primary Immunodeficiency

ImmUnity Canada continues to advocate for improved care, education, and support for individuals living with primary immunodeficiency diseases across Canada. Through policy initiatives, healthcare professional development, educational programming, and patient engagement efforts, the organization remains committed to improving access to care and strengthening support networks for patients and families nationwide.

Improving Access to Care Across Canada

One of ImmUnity Canada's most significant recent initiatives was the release of *Rare Disorders, Remote Realities: Mapping the Gaps in Care in Rural and Remote Canada*. The report highlights the challenges faced by patients living in underserved regions and outlines recommendations for improving access to specialized care, treatment, and healthcare services throughout the country. By bringing attention to the unique barriers experienced by patients in rural and remote communities, ImmUnity Canada continues to advocate for equitable access to care regardless of geographic location.

Supporting Healthcare Professionals

To strengthen immunology care capacity across Canada, ImmUnity Canada recently launched a national immunology nurse mentorship program. This initiative supports healthcare professionals who are establishing immunology clinics and services while encouraging collaboration and knowledge sharing among nurses working in the field of primary immunodeficiency.

The program represents an important investment in the future of immunology care and aims to improve patient access to knowledgeable healthcare providers across Canada.

Education and Community Engagement

Education remains a central component of ImmUnity Canada's mission. The organization continues to offer educational webinars and virtual events covering topics such as primary immunodeficiency, autoimmunity, patient advocacy, and patient engagement in healthcare decision-making.

Its popular *Slice of PI Podcast* features conversations with patients, caregivers, clinicians, researchers, and advocates from across the immunodeficiency community. Through personal stories and expert perspectives, the podcast explores topics related to diagnosis, treatment, advocacy, and everyday life with primary immunodeficiency while helping foster connection and awareness throughout the community.

For more information about ImmUnity Canada, visit: www.immunitycanada.org

Hyper IgM Foundation

Advancing Research, Collaboration, and Patient-Centered Outcomes
The Hyper IgM Foundation continues to support research, education, and collaboration aimed at improving outcomes for individuals and families affected by Hyper IgM syndromes. Through partnerships with researchers, clinicians, patient advocacy organizations, and families, the Foundation remains committed to advancing scientific discovery while ensuring that patient perspectives remain central to future research and clinical care.

Advancing Research Through Grant Funding
The Foundation continues to invest in innovative research to accelerate new therapies for Hyper IgM syndromes. This year, two competitive research grants were awarded to support projects with the potential to improve diagnosis and treatment.

A **$100,000 research grant** was awarded to **Dr. Caroline Kuo (UCLA)** to support the preclinical development of a **prime editing–based therapy for X-linked Hyper IgM syndrome**, generating critical data needed for future FDA engagement and IND-enabling studies.

A **$25,000 research grant** was awarded to **Dr. Rui Yang (Baylor College of Medicine)** to develop **"An Atlas of Variant Effects in CD40 and CD40LG,"** a project designed to better understand the functional impact of genetic variants associated with Hyper IgM syndromes and improve interpretation of genetic testing.

Education and Community Engagement
The Foundation recently participated in the **2026 Immune Deficiency Foundation National Conference**, hosting two educational sessions dedicated to Hyper IgM syndrome. Presentations featured **Dr. Francesca Ferrua**, who discussed the upcoming gene-editing clinical trial in Milan, and **Chris Simpson**, who shared additional perspectives on patient care and emerging research.

The Foundation also continues collaborating with patient advocacy organizations, clinicians, and researchers to develop shared educational resources and improve long-term outcomes research. These efforts help strengthen collaboration across the rare disease community while ensuring that the experiences and priorities of individuals living with Hyper IgM remain well represented.

Supporting Families and Future Research
Beyond research funding, the Foundation continues to strengthen patient engagement through community initiatives, including **Idan's 10-Year Transplantaversary Fundraiser**, which celebrates long-term transplant success while raising awareness and supporting future research for individuals living with Hyper IgM syndromes.

Through continued investment in research, education, and patient-centered collaboration, the Hyper IgM Foundation remains committed to advancing new therapies and improving the lives of individuals and families affected by these rare immune disorders.

For more information about the Hyper IgM Foundation, visit:
www.hyperigm.org

In Memoriam: Paul Veys

The following tribute was shared by Professor Persis Amrolia.

Dear friends,

It is with great sadness that I have to let you all know that Paul Veys, Director of the BMT unit at GOS from 1994–2020, passed away last Saturday. He had been battling bravely with cancer for the last year but there comes a time when the suffering is too much. He died at home peacefully with his family around him.

Paul was a colossus in the field of paediatric BMT who built and led the GOS BMT unit. He was a superb clinician who was totally committed to doing the best for children under his care and inspired a whole generation of transplanters both within the UK and Europe. One of Paul's greatest qualities is his leadership. He had a vision to make transplant safer and better: he pioneered reduced intensity conditioning in children with non-malignant disease, was an early advocate for cord blood transplant and led the first trial of allogeneic CAR T cell therapy. That took enormous vision, courage and self-sacrifice. He was the most natural leader I have ever known with his winning smile, boundless enthusiasm and unconditional support where he led, it was a privilege to follow. He genuinely believed in the power of collaboration and played a leading role in the UK Paediatric BMT Group, EBMT Inborn Errors and Paediatric Diseases Working Parties and PIDTC. Outside work Paul loved playing football, skiing and astronomy but above all his family. He was a kind, generous man and a wonderful mentor to so many of us, inspiring us to continue his mission. He touched the lives of so many, including over 2000 children transplanted on the unit and was loved by everyone who knew him. A life well lived. For those attending the IEWP-EBMT Meeting in Zurich this September, a keynote session will be presented in honour of Paul's remarkable career and life.

A great light has passed but Paul leaves a remarkable legacy in the unit he built and loved, the lives he saved and in our hearts.

Take care,
Persis

Newsletter brought to you by the PIDTC Program Management Team. Thank you to our partners at the RDCRN/DMCC!

Got announcements?
Email:abdullah.ahmed@ucsf.edu