

Primary Immune Deficiency Treatment Consortium

# NEWSLETTER

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*Group photo of the PIDTC Annual Workshop at the JW Marriott in Atlanta.*

## Greetings from Drs. Jennifer Puck, Chris Dvorak, and Elie Haddad, Multi-PIs

Hello PIDTC Members,

This issue presents highlights from the 2024 PIDTC Workshop and Education Day held in Atlanta, GA, hosted by our colleagues from Emory University and Children's Healthcare of Atlanta. It was an exciting meeting that brought into focus how far the PIDTC has come since our first funding announcement back in 2009. Beyond the IRB hassles, data cleaning, manuscript writing and other chores we could all see what a productive and supportive community we have become, providing ever better care for our patients with primary immune disorders.

While we heard from leaders whose fundamental discoveries have formed the basis of our understanding of immunology, we also welcomed many new faces representing immunology, transplantation, genetics, immune regulation, gene therapy and more.

We are confident that the PIDTC's next chapter after the U54 funding that provided our start will be just as exciting and fulfilling.

Cheers!

*Jennifer, Elie, and Chris*



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## PIDTC Education Day & 13<sup>th</sup> Annual Scientific Workshop in Atlanta, GA, April 8 – 11, 2024



This year's PIDTC Education Day and Annual Scientific Workshop were held in Atlanta. We were thrilled to see over 180 in-person attendees this year, our largest gathering ever!

A survey was distributed for feedback from the attendees of the PIDTC Education Day & Annual Scientific Workshop. All respondents found the Workshop to be excellent overall. The great majority of attendees evaluated the workshop as “perfect” regarding the number of speakers and topics (87.2%), overall scientific content (92.3%), and networking opportunities (89.7%).

**Our next workshop will take place from March 10-13, 2025, in St. Petersburg, Florida!**



## Some of the 2024 Workshop Speakers...

The PIDTC would like to thank our speakers for their time, presence, and perspectives.



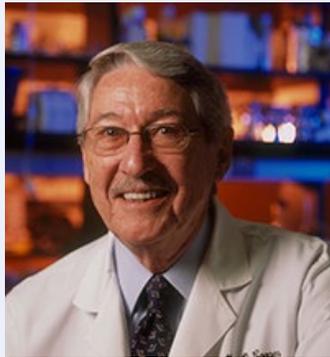
**Dr. Winston Yan** spoke on the potential of bespoke genetic therapies and the need for data sharing and collaboration between research institutions and pharmaceutical companies.



**Dr. Lisa Ott de Bruin** discussed RAG1/2 SCID, covering the biology, vector development, and early outcomes of treatment of RAG1 patients with gene therapy.



**Dr. Michael DeBaun** discussed sickle cell disease outcomes in pediatrics, the factors that influence them, and the implications of this research for pediatric rare disease research.



**Dr. Max Cooper** presented an evolutionary history of the humoral immune system.

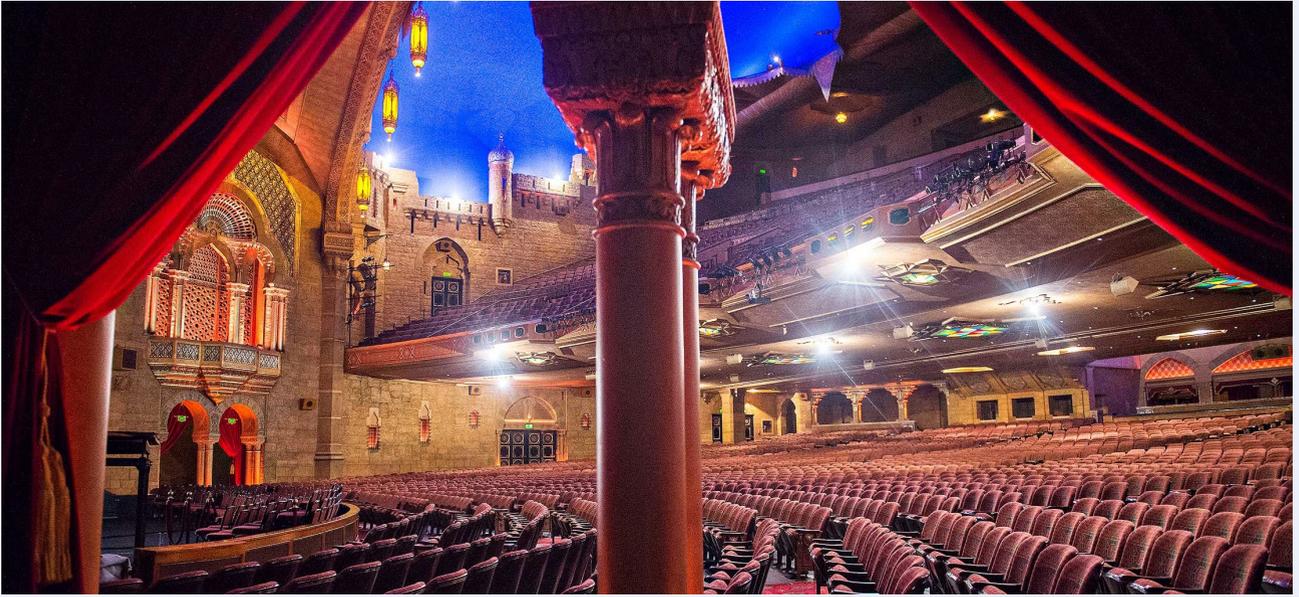


**Dr. Jaap Boelens** spoke on pharmacokinetic targeting of conditioning regimens for non-malignant disorders.



*The 6906 Protocol Leads updated the progress of the PIRD Protocol.*

## Dinner at the historic Fox Theater, Atlanta



A dinner reception was held at the historic Fox Theater in downtown Atlanta, which has hosted many iconic performances including Prince's legendary final concert.

The theater has a long and storied history with the film industry, the preservation of landmarks in Atlanta, and the development of American performance arts.

*(Right): Drs. Shan Chandrakasan and Suhag Parikh welcoming attendees to the Fox Theater.  
(Below): Drs. Jennifer Puck and Mort Cowan posing in Fox Theater chairs.*



## Dr. Harry Malech: Lifetime Achievement Award

The PIDTC was proud to present Dr. Harry Malech with a lifetime achievement award. Dr. Malech is Chief of the Genetic Immunotherapy Section and Deputy Chief of the Laboratory of Clinical Immunology and Microbiology at the National Institute of Allergy and Infectious Diseases intramural program, National Institutes of Health.

Dr. Malech has spent many decades doing groundbreaking basic and clinical research on with chronic granulomatous disease (CGD) and gene therapies. Dr. Malech has led many of the NIH's clinical trials of hematopoietic cell transplantation and gene therapy to treat primary immune disorders. His current endeavors include using CRISPR editing to restore immune function in patients with CGD.



*Dr. Malech received a standing ovation after his award was presented.*

# Protocol Updates

## Severe Combined Immunodeficiency (SCID) – 6901/6902/6907

Protocols 6901 and 6902 are closed to new enrollments and are in the process of final data collection and cleaning. Several papers incorporating data from these legacy protocols are nearing completion, including manuscripts describing outcomes of unrelated cord blood HCTs, matched sibling HCT, racial/ethnic disparities in outcomes, neurodevelopmental outcomes, and differential outcomes by the trigger for diagnosis. Other manuscripts in the works include genotype specific papers (on Artemis, IL2RG/JAK3, and CD3D), outcomes of 2nd transplants and boosts, and the entire 6901 dataset, as well as several other ideas. The SCID working group is always happy to hear new proposals for potential analyses to be performed on this rich dataset.

Protocol 6907 is now open at most centers and is actively accruing all available SCID patients, both newly diagnosed and previously treated, using its carefully-designed case report forms to assemble the premier database on outcomes of patients with SCID. Please continue to enroll patients!

## Chronic Granulomatous Disease (CGD) – 6903/6908

The 6903 overall paper was published in Blood in December 2023. Papers describing transplant outcomes for p47phox-deficient CGD and the intestinal microbiome and metabolome signatures in patients with CGD have also been published. The CGD team is working on several other manuscripts, and additional microbiome papers are also in preparation.

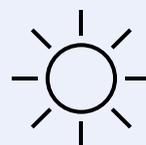
Protocol 6903 has closed to enrollment, and the dataset is actively being cleaned. Please make every effort to complete outstanding data entry for patients enrolled to 6903. Protocol 6908 has been activated or is near activation at the majority of PIDTC centers. Reach out to the CGD program managers (lisa.lim@ucsf.edu and jessica.ni@ucsf.edu) with any questions related to 6903 data completion or 6908 enrollment.

## Wiskott–Aldrich Syndrome (WAS) – 6904

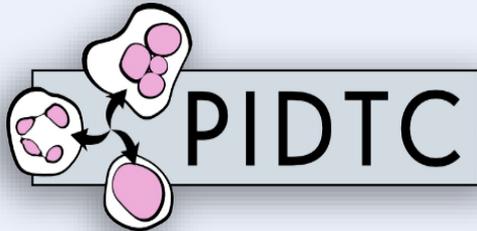
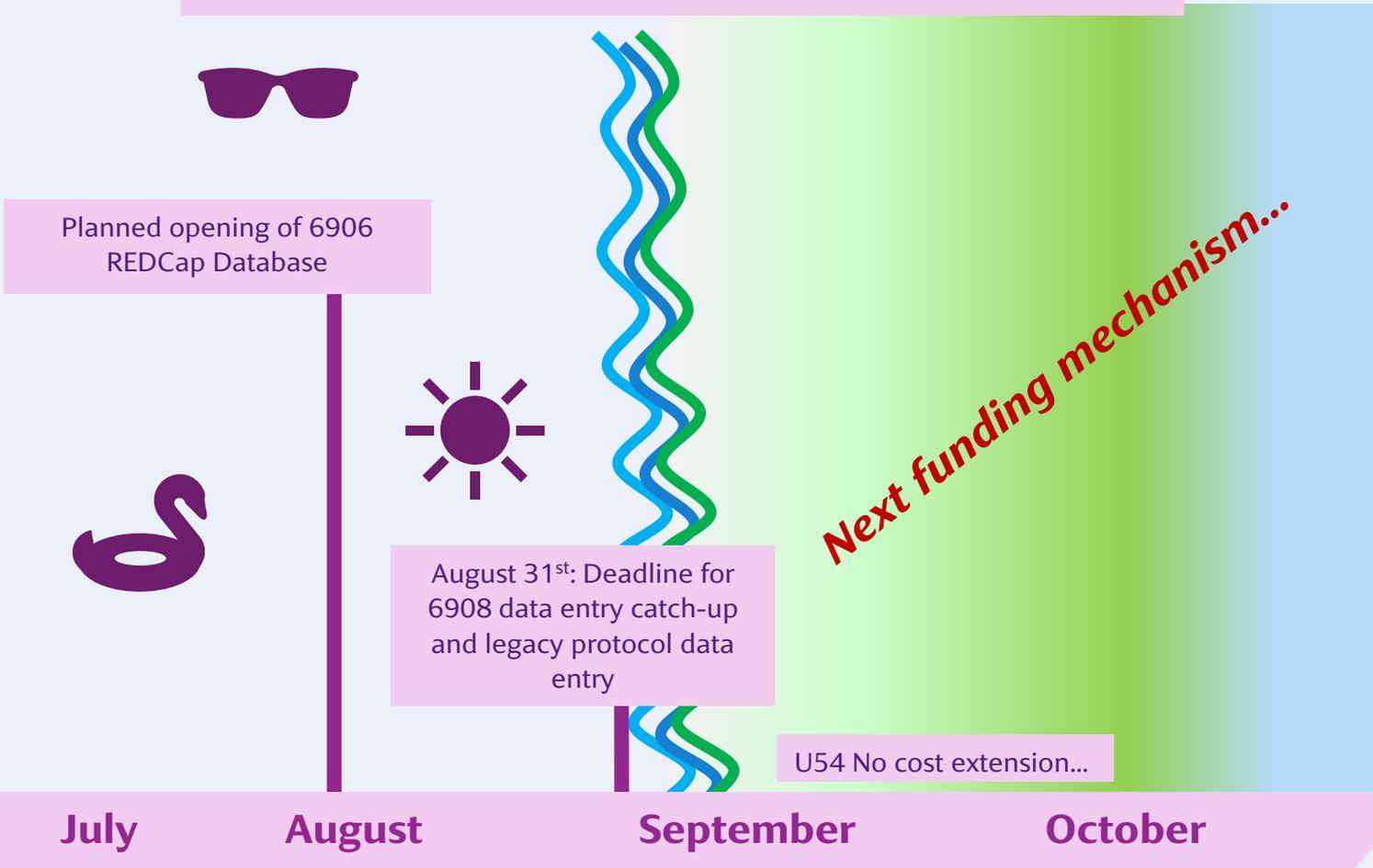
6904 WAS patients can be rolled over onto the 6906 PIRD Protocol. Look out for an email containing a list of eligible rollovers!

## Primary Immune Regulatory Disorders (PIRD) – 6906

The PIRD Protocol currently has 21 activated sites and anticipates that the REDCap database will open in the next few weeks.



## PIDTC: Summer 2024



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

Got announcements?  
Email: [rafael.ricon@ucsf.edu](mailto:rafael.ricon@ucsf.edu)