Primary Immune Deficiency Treatment Consortium

NEWSLETTER

Spring 2022 | Issue 15

Read about Arturo Garcia and his family's experience with the Jeffrey Modell Foundation's Roots & Wings Program on Page 3.

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

We look forward to opening our three new protocols at ALL sites. The SCID protocol (6907) is being resubmitted to the UCSF IRB for Version 2.0. Importantly, we are making revisions to the SCID diagnostic criteria. The CGD (6908) and PIRD (6906) protocols were submitted to the UCSF IRB and are currently waiting approval.

We had several accepted presentations and posters based on PIDTC data that were accepted to the Clinical Immunology Society (CIS) Meeting early April, and we are preparing for the ASTCT/CIBMTR Tandem Meetings in later April as well.

We are also holding a Leadership Meeting soon to prepare for grant submissions in the next 2 years before the end of our U54 funding cycle – it's been a great run, but we are no longer eligible for future U54 funding.

With appreciation,
Jennifer, Elie, and Chris

IN THIS ISSUE

“Roots & Wings” is a humanitarian program created to assist children worldwide who have been diagnosed with a serious or life-threatening Primary Immunodeficiency. The “Roots & Wings” Program provides travel (the “Wings”) and related support (the “Roots”) for families whose child is in need of a stem cell transplant, gene therapy or other lifesaving treatments, and have to travel a great distance for the procedure. Families often have to stay in a Ronald McDonald House or similar housing for many months, and they may not have the funds to do so. We would like to make life easier for these families, reduce the burden, and enable the child to have a chance at life.

There is no fee of any kind. In order to apply please see provided link:

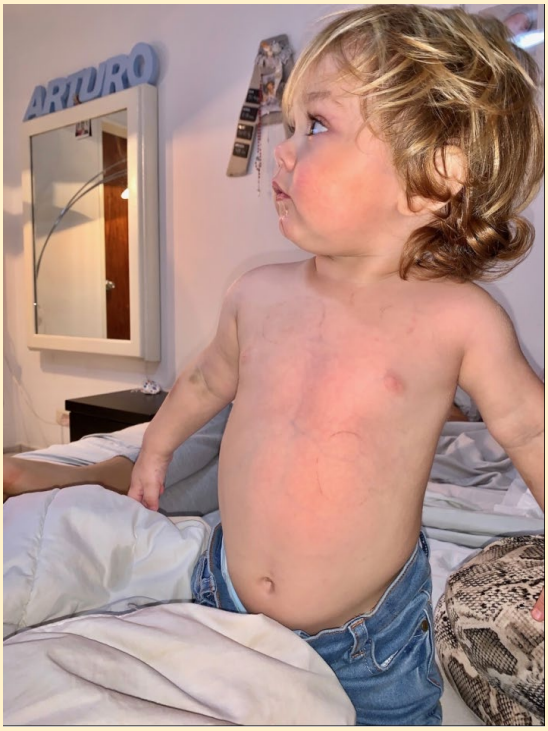
http://jmfworld.com/airport/roots-and-wings

Note that the application request for Personal and Medical information needs to be filled out by a physician.

Arturo Garcia and Family’s Experience with Jeffrey Modell Foundation’s Roots & Wings Program

by Fiorela Jaimes, Mother of Arturo Garcia

Please meet my son, Arturo…these are my words about our experience with the Jeffrey Modell Foundation’s Roots & Wings Program.



Arturo Garcia is currently two and a half years old. He was diagnosed in January 2020 with a congenital disease called Chronic Granulomatous (CGD). In his first months of life, he was a healthy baby, although early he showed some symptoms of what later became a problem for his health. It was until he was 17 months old when he had his first long hospitalization. After 2 months with complications with a severe inflammatory condition and several infections, the doctors were able to identify that the cause of everything that was happening was caused by a Primary Immunodeficiency. Of which we did not know in our family, that we were carriers.

CGD is caused by a genetic mutation in the gene, CYBB, that Arturo carries. CGD patients have a compromised immune system and are susceptible to serious, invasive, and life-threatening infections with bacteria and fungi. CGD patients are also susceptible to life-threatening autoimmune and autoinflammatory conditions. Arturo has already suffered from the effects of CGD, a universally fatal disease, that without proper medical care, can require lifelong treatment with antibiotics and antifungal medications to prevent infection.

Dr. Donald Kohn and Arturo Garcia at UCLA.

This last reason led us to make drastic decisions as a family, to get Arturo out of Venezuela (the medical system of our country does not have what is necessary to attend to these cases) and give him the emergency care he needed, since this disease was unknown to us as a family.

We started looking for someone to turn to and a lot of the information we found was related to the Jeffrey Modell Foundation. So looking on their website is where we got the contact information of a Venezuelan immunologist listed by the Foundation, and we contacted her and shared Arturo's medical records with her. She kindly received our request for guidance that we solicited, and she helped us reach out and share our medical request with Mrs. Vicki Modell. In one or two days we had already made a connection as a family with the Foundation.

The Jeffrey Modell Foundation has been an invaluable support in carrying out Arturo's medical process. In January 2020 when they received the case, they connected our family with different specialists in the United States who could attend to the baby. We received a prompt and positive response from Dr. Jennifer Leiding, formerly from Johns Hopkins All Children's Hospital in St. Petersburg, Florida. She offered us the possibility of transferring not only Arturo, but the entire family from Caracas-Venezuela to Miami-Florida.

- That is how in a couple of weeks everything was prepared, and thanks to the Jeffrey Modell Foundation we were able to transfer Arturo (who had delicate health at the time) from Venezuela to the United States on February 3, 2020.

- Arturo was seen for the first time in Dr. Leiding's office on February 7, 2020. He was hospitalized at Johns Hopkins for a week, where Dr. Leiding oversaw all the medical procedures and genetic tests that would determine his health status and helped to confirm the diagnosis that had been identified in Venezuela.

- The therapeutic option to correct Arturo's CGD required a 10/10 compatible bone marrow transplant. After a long search we did not have a 10/10 donor for Arturo, so the options changed to perform a haploidentical transplant with one of his sisters, who is 50 % compatible with him, or through gene therapy. These two therapeutic options do not guarantee the success of correcting the pathology in his neutrophils, but we had to try. Again, without knowing what path to take, we turned to the advice of the Jeffrey Modell Foundation who encouraged us to trust the medical team and who considered that Arturo was being cared for by the most recognized specialists for these medical cases.

- Replacing the immune system with genetically modified stem cells (called gene therapy), as part of a clinical trial, has been completed as a curative option. This gene therapy is only available in 2 centers in the United States and Arturo is currently enrolled in the UCLA clinical trial, led by Dr. Donald Kohn. It is now just over 4 months since the infusion of gene-modified stem cells after a high-dose chemotherapy. His gene therapy has been complicated by the delayed engraftment of his white blood cells, as well as, by multiple infections, requiring readmission to hospital for further support and management. He is currently hospitalized again with a relapse in count of his white blood cells.

- It is in Arturo's best medical interest to continue close outpatient follow-up for study time, point evaluations (including specialized assay tests) and ongoing support for his comprehensive development, including management of acquired oral aversion that prevents him from eating by himself. At this point, the family has already exhausted their economic resources to maintain their stay in Los Angeles, California. The full-time condition that Arturo's care merits, coupled with the unavailability of permits to work in the country, does not allow us as a family to generate income. Under these conditions we found ourselves in the forced situation of returning to Venezuela with Arturo, but thanks to the Jeffrey Modell Foundation, which has set up a fund to help the family pay the rent to stay in Los Angeles, Arturo will be able to continue the course of his treatment and medical care by Dr. Donald Kohn at the Ronald Reagan UCLA hospital.

We thank God for all these efforts that have been achieved up to now, and for allowing the joint collaboration between the Jeffrey Modell Foundation, the medical team and our family, which we hope will result in the best and most anticipated result by us, the total healing of Arturo.

On behalf of Arturo's family
Fiorela Jaimes
Mother

Research Training Grant Announcement

A one-year Research Training Grant in primary immune deficiencies is available from the PIDTC.

Two Training Grant awards are made each year to encourage research in transplantation, gene therapy, or outcomes of therapies for primary immune deficiencies (PID); however, applications may be on any aspect of PID.

Eligibility: Trainees or Junior Faculty with two years or less as a faculty member from an eligible PIDTC institution may apply. Applicants from Intramural NIH are not eligible. Successful recipients who remain in eligible trainee status can apply for one additional year of funding immediately following the first year, with a one-page progress report in addition to a renewal application documenting accomplishments made on the research topic. Please inquire about the additional year by 8 months after the award start date to: sharon.kidd@ucsf.edu.

Format: The application packet must be sent as a single pdf file and must include:

- Research proposal in NIH grant format with Title and Contact Information, Specific Aims, Background, and Research Plan/Strategy. This should be limited to 3 pages (not counting references), single spaced, Arial 11 font, 0.5 inch margins).
- Letters of support from: Mentor; Training Program Director (if trainee); and Department Chair (documenting sufficient resources and time commitment to complete the project).
- NIH format Biosketch with Other Support.
- NIH biosketch of mentor with Other Support.
- Budget with justification, effort related to project, other commitments, and timeline for completion of studies (1 page).

Link to application:
https://www1.rarediseasesnetwork.org/cms/Portals/PIDTC/PIDTC%20Research%20Training%20Grant%20Announcement%202022.pdf

Please review application for more details and dates regarding the timeline for application process.

Deadline: **April 25, 2022**

Pilot Project Announcement

Please note that we will not be offering the Pilot Project Program this year.

Research from the PIDTC …

Dr. Geoff Cuvelier from Cancer Care Manitoba has submitted a paper on outcomes after definitive therapy for ADA Deficiency to *Blood* using data from two of our SCID protocols

Abstract from Outcomes After Hematopoietic Cell Transplant and Gene Therapy for Adenosine Deaminase (ADA) Deficiency: A Combined Analysis from the Primary Immune Deficiency Treatment Consortium (PIDTC) 6901 and 6902 Studies

Authors: Cuvelier, G, Logan B, Prockop SE *et. al*…other PIDTC investigators

To Be Presented at the American Transplant and Cellular Therapy Tandem Meetings,
Salt Lake City, April 25, 2022

Background: ADA is a rare enzyme deficiency causing ~13% of cases of severe combined immune deficiency (SCID). Treatments include enzyme replacement therapy (ERT), hematopoietic cell transplant (HCT), or gene therapy (GT). We sought to understand differences in outcome between HCT and GT.

Methods: 120 ADA patients diagnosed between 1982-2017 and enrolled on PIDTC 6901 (prospective) or 6902 (retrospective and cross-sectional) studies at 27 centers were included.

Results: First definitive cellular therapy (FDCT) included 56 receiving HCT without ERT (HCT), 31 HCT preceded by ERT (ERT-HCT), and 33 GT preceded by ERT (GT).

5-yr OS for the entire cohort from FDCT was 72.5% for HCT (95% Confidence Interval (CI): 57.7-82.8%) vs 79.6% for ERT-HCT (CI: 60.0-90.3%) vs 100% for GT (CI: 100-100%) (**Fig 1a**, p=0.01). 5-yr EFS (alive, no need for further ERT or cellular therapy) was 49.5% for HCT (CI: 34.9-62.5%) vs 73% for ERT-HCT (CI: 53.1-85.5%) vs 75.3% for GT (CI: 34.4-92.7%) (**Fig 1b**, p<0.01). 5-yr cumulative incidence of subsequent treatment was 30% for HCT (CI: 17.7-43.5%), 10.4% for ERT-HCT (CI: 2.5-24.7%) vs 24.7% for GT (CI: 3.3-56.4%) (p=0.14). 2-yr cumulative incidence of chronic GVHD was 3.8% for HCT vs 12.7% for ERT-HCT (p=0.31). 5-yr OS was significantly better in patients who underwent HCT at <3.5 months of age (91.6% vs 68% if ≥3.5 months, p=0.02) although EFS was similar (71.6% vs 51.9% if ≥3.5 months, p=0.18). Active infection at the time of HCT resulted in decreased 5-yr OS (64.7% vs 82.3%, p=0.02) and EFS (33.1% vs 68.2%, p<0.01). OS (p<0.01) and EFS (p<0.01) were better for matched sibling / related donors (MSD) vs mismatched related donors (MMRD). OS was better for MSD vs matched unrelated (MUD) / cord (UCB) (p=0.03). EFS was better for MUD vs MMRD (p=0.02) (**Fig 2**). Conditioning did not impact OS/EFS.

Initial groups were unbalanced, with greater proportions of patients who received unconditioned MMRD HCT in earlier eras and with active infections, whereas all GT patients were uninfected at the time of GT. To evaluate outcomes in the contemporary era, therefore, we compared patients receiving any HCT or GT as FDCT after the year 2000 and without an active infection (HCT n=33; GT n=33). HCT patients included 15/33 with ERT-HCT and a variety of donors, grafts, and conditioning regimens. 5-yr OS was 90.9% (CI: 74.4-97%) for HCT vs 100% (CI: 100-100%) for GT (**Fig 3a**, p=0.09) (Fig 3). 5-yr EFS was 75.3% (CI: 56.5-86.8%) for HCT vs 75.3% (CI: 34.4-92.7%) for GT (**Fig 3b**, p=0.29).

Conclusions: Overall survival after GT and MSD HCT for ADA was 100% and only a minority needed to restart ERT or undergo subsequent HCT. In the contemporary era, HCT in uninfected patients led to OS/EFS comparable to GT. ERT before FDCT did not decrease survival. Survival benefits were apparent when HCT occurred <3.5 months of age and without infection, supporting SCID newborn screening to identify patients early in life.

PAG Updates

Immune Deficiency Foundation:

SCID Compass
An educational program of the Immune Deficiency Foundation

Immune Deficiency Foundation

Supporting Siblings of Children with Chronic Illness

A diagnosis of severe combined immunodeficiency (SCID) is overwhelming, not only for parents but also for the siblings of the baby diagnosed. As parents immerse themselves in navigating a life-threatening medical emergency for their baby, siblings' needs go unmet, their routines are interrupted, and they experience anxiety, said Samantha Childs, during her recent SCID Compass Lunch & Learn presentation, "Supporting Siblings of Children with Chronic Illness."

For more details regarding this talk click on the link below:
https://scidcompass.org/news/siblings-need-support-after-scid-diagnosis

If you are interested in viewing previous Lunch & Learn presentations, see the following:

- Date: March 9th, 2022 (past)
- Focus of this lunch and learn was: Hyper IgM
- Link: https://primaryimmune.org/video/idf-lunch-learn-hyper-igm-march-9-2022

CGD Association of America:

IDF provides informative podcasts titled “Have You Herd?” Watch the latest podcast which focuses on living with CGD and discussions about BMT.

In order to watch latest podcast, which focuses on living with CGD and discussions about BMT, click on this link:

https://primaryimmune.org/video/living-with-cgd-discussion-about-bmt-diagnosis-specific-episode

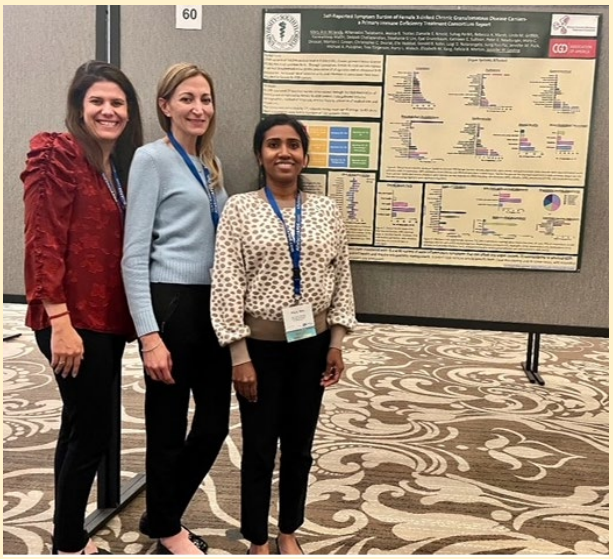
Chronic Granulomatous Disease or CGD, one of the rare forms of primary immunodeficiency, causes an increased susceptibility to infections caused by certain bacteria and fungi. In today’s diagnosis-specific episode, we will be exploring treatment options, particularly Bone Marrow Transplant, or BMT for CGD with Felicia Morton and Dr. Vinod Prasad who is the Site PI at Duke University Medical Center for our 6903/6908 CGD Protocol.

Abstract from Research Collaboration on Female Carriers of X-Linked CGD Presented at 2022 Clinical Immunology Society Meeting

The CGD Association of America is delighted to announce that the abstract titled "Self-Reported Symptom Burden of Female X-linked Chronic Granulomatous Disease Carriers - a PIDTC Report," was accepted for a poster presentation at the 2022 Clinical Immunology Society meeting in Charlotte, North Carolina.

The IRB-approved research project, led by Dr. Jennifer Leiding, is a collaboration with the CGD Association of America and the Primary Immune Deficiency Treatment Consortium. This important research effort aims to better understand the health issues and ailments that CGD carriers can experience. Thank you to Horizon for supporting this research effort.

For further details click on this link:
https://cgdaa.org/f/cgd-research-presented-at-22-clinical-immunology-society-meeting



Dr. Jennifer Leiding, Attending Physician, Arnold Palmer Hospital for Children and Adjunct Associate Professor, Johns Hopkins University, Felicia B. Morton, Executive Director, CGDAA, and Dr. Mary Ann Miranda, Fellow Physician, Division of Allergy and Immunology, University of South Florida.

Jeffrey Modell Foundation:

Meet JMF's new Chief Operating Officer and Executive Vice President:

Margaret Caspler

The Jeffrey Modell Foundation is thrilled to announce that Margaret A. Caspler (formerly Cianci), a veteran non-profit leader and most recently Executive Director of The Endometriosis Foundation of America, has been appointed as the nonprofit's first Chief Operating Officer and Executive Vice President. Please join us in welcoming Margaret to the team, as we work together to accelerate research and early diagnosis and improve treatment and access to care for primary immunodeficiency diseases.

Hyper IgM Foundation:

Research Grants

- Website Link: https://hyperigm.org/hyper-igm-foundation-research-grants/
- The intent of these grants is to accelerate scientific work focused on improving the treatment, quality of life, and long-term outlook for patients with Hyper IgM.
- Researchers interested in the advancement of a cure for Hyper IgM Syndrome can apply.
- Award value: One-time grants of up to $100,000
- Application deadline: **May 15, 2022**

Virtual 5K Fundraiser

- In honor of National Primary Immunodeficiency Month and World PI Week (April 22nd - April 29th, 2022), the Hyper IgM Foundation will be hosting a Virtual 5K
- Website Link: https://hyperigm.networkforgood.com/events/40166-hyper-igm-foundation-virtual-5k-for-world-pi-week

SCID Angels for Life:

Grant Award Announcement

SCID Angels for Life Foundation launched its first call for grant proposals in October 2021 with funding from AMDA Biologics Inc. During the review period an anonymous donor stepped forward with a matching grant, allowing SCID Angels the ability to fund a second proposal.

Congratulations to the 2022 Grant Awardees who are both PIDTC Members!

One Year Grant for - New and Innovative Curative Treatment Approach

"IL7Rα gene editing to treat IL7Rα-SCID"

Dr. Donald B. Kohn, MD
University of California, Los Angeles

One Year Grant for - Innovative Approach Targeting a Marginalized Community

"Predicting the clinical phenotype and increasing awareness of a novel RAG1 p.C176F founder variant causing atypical SCID with variable immune dysregulation in U.S. Mennonite communities"

Dr. Jolan Walter, MD, PhD
University of South Florida

To find more details regarding the proposals click on this website link:
http://www.scidangelsforlife.com/2022-grant-award-announcement/

Aisha Chaudhary Educational Scholarship Program

- SCID patients that will be attending college or a trade school are welcomed to apply
- three awardees will receive $5,000 each
- Website link:
  http://www.scidangelsforlife.com/2019/12/aisha-chaudhary-educational-scholarship-program/

WAS Foundation:

WAS Carrier Survey

- The WAS Carrier Survey is now closed.
- Dr. Suhag Parikh and Dr. Shanmuganathan Chandrakasan from Emory University School of Medicine will be analyzing the data.
- The abstract for the WAS Carrier Survey will be presented at the 3rd International WAS Symposium which will take place on Friday June 17th, 2022 in Munich, Germany.
- Results of the WAS Carrier Survey were presented at the IDF Lunch & Learn on April 20th, 2022

Thank you to all our PAGS!

Protocol Updates

Severe Combined Immune Deficiency (SCID) – 6901/6902/6907

Updates:
Thank you to our Protocol PIs, **Drs. Chris Dvorak**, **Elie Haddad** and **Jen Heimall** for leading the SCID team finalizing the new 6907 protocol and overseeing the numerous manuscripts that are in the works. We thank our outstanding statistics team, led by **Dr. Brent Logan,** for its efforts pulling together this data.

The modified consents are UCSF IRB–approved and we began the process of onboarding many of our PIDTC sites in January. UCSF Benioff Children's Hospital has onboarded to the 6907 protocol. Nine sites (CancerCare Manitoba Winnipeg Children's Hospital, Children's Hospital of Pittsburgh, Children's of Alabama, Children's National Hospital, Hackensack University Medical Center, Maria Fareri Children's Hospital, Rady Children's Hospital, St. Louis Children's Hospital, and Stanford University) have been submitted for IRB review.

Goals: Do not miss enrolling your 6901 Prospective SCID patients (while awaiting onboarding) during the DMCC transition period! Enter your eligibility data into the CRFs in the South Florida database and then email Elizabeth Dunn at Elizabeth.dunn@ucsf.edu, to finalize patient eligibility via email.

Chronic Granulomatous Disease (CGD) – 6903/6908

Updates: The 6908 protocol review which was led by Protocol PIs **Drs. Jen Leiding**, **Harry Malech**, and **Dani Arnold** has been submitted to the UCSF IRB and currently awaiting approval. The entire 6903 team, especially **Drs. Elizabeth Kang, Suhag Parikh, Stephanie Si, Kanwal Malhi, Deepak Chellapandian and Rebecca Marsh,** have been busy preparing for a manuscript using the overall data. Thank you to our statisticians, **Rachel Wu** and **Dr. Brent Logan,** for all their efforts!

Enrollment: Do not miss enrolling your 6903 Prospective CGD patients. Enter in your Eligibility data into the South Florida database and then email Elizabeth Dunn at Elizabeth.dunn@ucsf.edu to finalize patient eligibility.

Wiskott–Aldrich Syndrome (WAS) – 6904

Updates. The WAS team is now working on data analysis and manuscript writing for the second 6904 paper with a larger "N" of patients. We especially want to thank investigators **Drs. Lauri Burroughs, David Shyr**, **Blachy Davila Saldana, Jessie Barnum**, and **Ami Shah** and our talented statisticians **Dr. Ruta Brazauskas**, and **Joy Liu**. We would also like to thank **Dr. Sumathi Iyengar** for her advice and active participation in our protocol calls.

Primary Immune Dysregulation Disorder – 6906

Updates: The PIRD team led by Protocol PIs Drs. Troy Torgerson, Alice Chan and Rosa Bachetta, are awaiting approval of the IRB submission. Meanwhile, plans for networking with Adult Immunology providers is underway to capture many adults with PIRD that might otherwise be missed.

Ongoing Clinical Studies

Lentiviral gene transfer for SCID-X1 with low dose targeted Busulfan conditioning

This trial is open and enrolling at Boston Children's Hospital and Mattel Children's Hospital UCLA, as well as at Great Ormond Street Hospital in London. For eligibility or more information about the study, please contact: Overall PI: **Sung-Yun Pai, MD** (sung-yun.pai@childrens.harvard.edu); Los Angeles PI: **Donald Kohn, MD** (dkohn1@mednet.ucla. edu); Sponsor: **David A. Williams, MD** (david.williams2@childrens.harvard.edu).

LAD-I gene therapy trial

This Leukocyte Adhesion Deficiency Type I (LAD-I) gene therapy trial is currently enrolling patients at UCLA (US). Additional treatment centers will include UCL/GOSH (UK) and Hospital Infantil Universitario Niño de Jesús (Spain). The trial is sponsored by Rocket Pharmaceuticals, Inc., and funded by the California Institute of Regenerative Medicine (CIRM). For more information, please contact LADclinicaltrial@rocketpharma.com or visit https://clinicaltrials.gov/ct2/show/NCT03812263?term=NCT03812263&rank=1 or https://www.rocketpharma.com/lad-i-clinical- trial-for-health-care-providers/

Gene Therapy Trial to Treat X-linked Severe Combined Immunodeficiency

This trial is currently enrolling at St. Jude's, Seattle, and UCSF Benioff Children's Hospital. In this research study, boys with SCID-X1 will receive a treatment called "lentiviral gene transfer," also called "gene therapy." This method inserts a normal copy of the SCID-X1 gene into blood-forming cells or "stem cells" from bone marrow that grow and develop into all blood cell types. The inserted gene will provide correct instructions to the defective stem cells in SCID-X1 so that functioning lymphocytes can develop.

For eligibility or more information about the study, please visit: stjude.org/LVXSCID-ND, or contact **Ewelina Mamcarz, MD** (ewelina.mamcarz@stjude.org), **Aleksandra Petrovic, MD** (Aleksandra.Petrovic@seattlechildrens.org), or **Mort Cowan, MD** (Mort.Cowan@ucsf.edu)

UCSF Artemis SCID Gene Therapy

In this trial, newly diagnosed or previously treated patients with insufficient immunity due to ART-SCID receive "lentiviral gene transfer," also called "gene therapy." A normal copy of the DCLRE1C gene is inserted into blood-forming stem cells that grow and develop into all blood lineages. The inserted gene provides correct instructions to the defective stem cells so that functioning T and B lymphocytes can develop. So far 10 patients have been treated.

For eligibility or more information about the study, please contact: **Mort Cowan, MD** (Mort.Cowan@ucsf.edu) or **Jennifer Puck, MD** (Jennifer.Puck@ucsf.edu).

A Study of Mavorixafor in Participants With Severe Congenital Neutropenia and Chronic Neutropenia Disorders

This Phase 1b study will determine the safety and tolerability of mavorixafor in participants with severe chronic idiopathic neutropenia (CIN) and selected congenital neutropenia disorders. The anticipated enrollment is up to 25 participants.

For questions regarding the trial, please email clinicaltrialinfo@x4pharma.com or call 857-529-5779.

Website: https://www.x4pharma.com/patients/chronic-neutropenia/

Viral CTL Consortium (VIRCTLC)

Principal Investigator Mitchell S. Cairo, MD and Study Chairs Julie Talano, MD and Nancy Bunin, MD, are studying (funding by the FDA) the safety, efficacy and biology of viral CTLS derived from related donors by the Cytokine Capture System using the Prodigy device in patients with immunodeficiencies either secondary to HSCT or primary immunodeficiencies with refractory CMV, ADV and/or EBV or intolerant to anti-viral therapy. If you and your institution are interested in participating in this clinical trial, please contact **Dr. Mitchell S. Cairo** (Mitchell_cairo@nymc.edu).

Anti-c-KIT (JSP191) Transplant Protocol

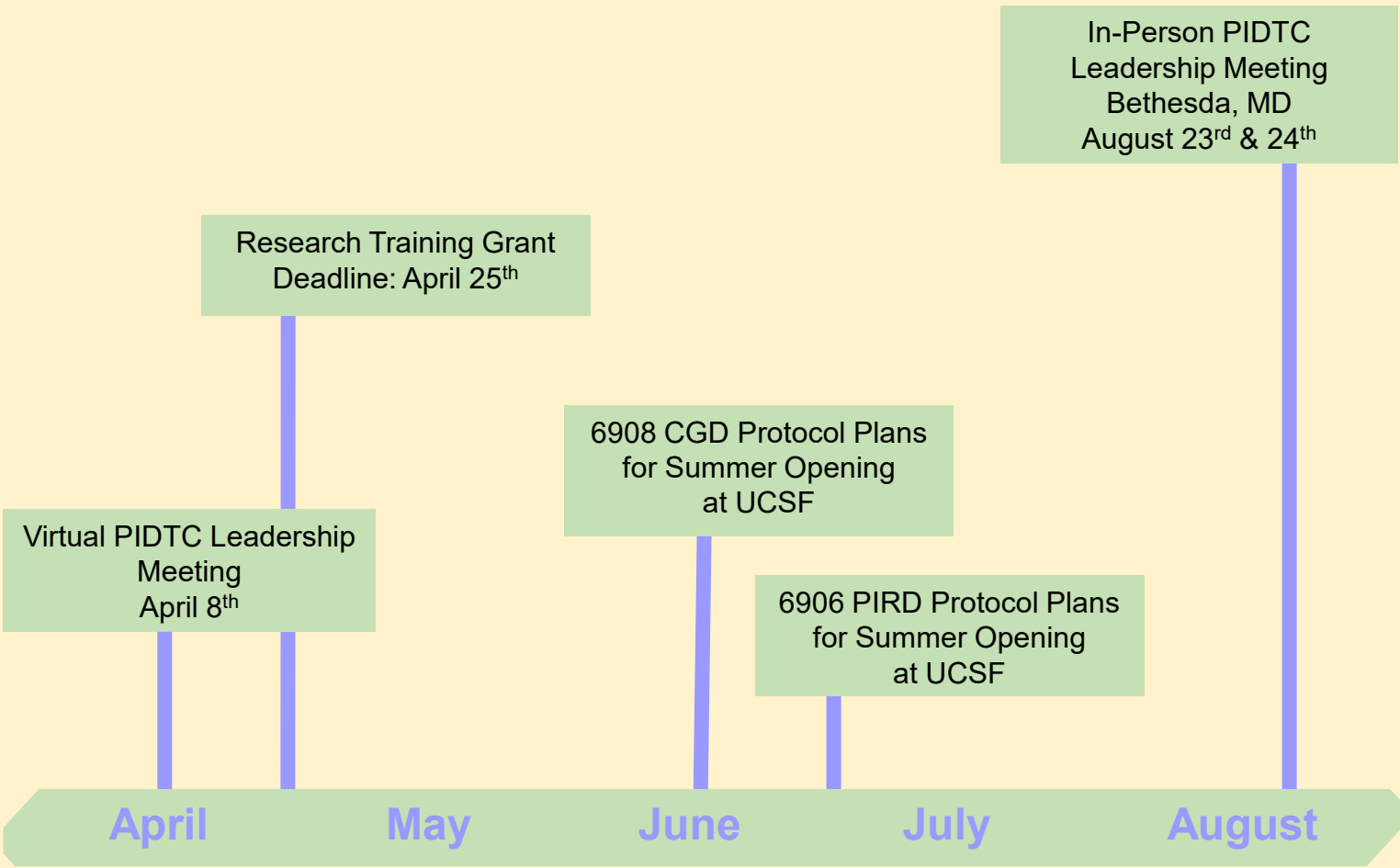
This Phase I study is a single arm, open label, dose escalation trial being conducted at multiple PIDTC centers, including: UCSF Benioff Children's Hospital, Lucile Packard Children's Hospital at Stanford and Memorial Sloan Kettering Cancer Center in New York. The study objective is to evaluate the safety and tolerability of allogeneic CD34+ human stem cells (HSC) in patients with Severe Combined Immune Deficiencies (SCID) conditioned for transplantation with JSP191, a monoclonal antibody that targets human CD117. The trial is open for both patients in need of repeat HCT as well as newly- diagnosed patients undergoing first HCT.

For questions regarding the trial please contact Wendy Pang (wpang@Jaspertherapeutics.com).

C-SIDE

CSIDE is open to enrollment 34 sites and 13 patients have been enrolled to date. More centers are currently being activated! If you have any questions about getting your site on board, please email **Sung-Yun Pai, MD** (sung-yun.pai@nih.gov), **Mike Pulsipher** (mpulsipher@chla.usc.edu), and **Jenny Vogel** (jvogel@nmdp.org).

PIDTC: Spring/Summer 2022



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

Got announcements?
Email: kiana.soriano@ucsf.edu