

## Primary Immune Deficiency Treatment Consortium



PICTURED ABOVE: EVERETT (SEE PG 2)

# PIDTC Newsletter

01 August 2016  
Issue #3

### IN THIS ISSUE

## Year Seven Recap from Dr. Cowan

As we conclude year seven, I want to congratulate you all on a very successful year! Our accomplishments this year include:

- Revised protocols for 6901/6902: out by end of June.
- Started enrollment/CRF completion for 6903/6904
- Completed dataset cleanup for 6902 Stratum A & (almost) stratum B with stratum C to follow shortly
- Funded 2 fellows
- Accounted for 9 publications
- Completed international QoL survey of WAS patients/families with IDF and WAF; poster at CIS; manuscript in preparation.
- Activated 3 European centers for CGD Protocol 6903: **Newcastle, London and Zurich**
- One of best pediatric abstracts at ASBMT – 6901 “1<sup>st</sup> 100”
- Initiated Central IRB for PIDTC: 13 centers signing reliance agreement, 9 still reviewing, 16 declined (6 Privacy Board, 4 only cede new protocols, 1 federally-funded research, 1 no benefit, 4 no reason).
- Initiated monthly CRF alerts and “real time” data cleanup
- Submitted Ro1 for 6905: Prospective study of low dose Bu for newly dx’d SCID –scored but not funded (yet): **Sung Yun Pai**
- Started planning for multicenter study of virus-specific cytotoxic T cells: **Michael Keller** and **Catherine Bollard**
- Increased PIDTC membership with the addition of **St. Jude’s Children’s Hospital** and the **University of Alberta!**
- A very successful PIDTC workshop this past May, thanks to UCLA + CHLA!

It goes without saying that all of these have only been made possible through the hard work contributed by each center. As has been discussed, we have been working to reduce this workload on the centers through various methods. These include shortening the CRFs for each study, initiating more frequent data clean ups, initiating a central IRB, centralizing our contracting this September, and implementing prospective intervention studies and hoping to provide more money for staff through these efforts.

It is through the continuation of these efforts that I am confident we will have a just as successful year 8. That said, I wish to conclude with our goals for the next year:

- 6903/6904: enroll & complete CRFs for 50% by Aug 2016 and 100% of retrospective patients (by May 2017)
  - Submit initial papers on 50% of patients (Jan 2017)
- Resubmit 6905 application to NIH or ITN
- Publish paper from WAF/IDF/PIDTC QoL survey of WAS patients/families
- Complete parent survey of non-medical support for SCID, CGD and WAS pts (IDF, WAF, CGDA) & publish manuscript
- Plan for renewal (spring 2018): new disease(s) and intervention studies at the PIDTC Leadership meeting in Bethesda in the Fall 2016

Again, thank you all for all your hard work this year, and I look forward to another great year for you and the PIDTC!

Hoping you are having a great summer,

*Mort*

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A big thanks to IDF and SCID Angels! *Page 2*

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### Cheers to the PIDTC’s Seventh Year!!

Dr. Cowan, Dr. Puck, and the PIDTC Management Team cheers to another great year while aboard the surprise yacht at this year’s PIDTC Workshop!

## PAG Update

We would like to extend a huge thank you to the Patient Advocacy Groups: SCID Angels, the IDF, and WAF for their huge efforts to connect PID patients and families with our studies. We have had a huge influx of families signing up on our contact registry, and are in the process of connecting many of these families with PIDTC centers for enrollment to our studies! A huge thanks to **Barb Ballard** with the IDF and **Heather Smith** with SCID Angels!

This past workshop **Sumathi Iyengar** at the Wiskott-Aldrich Foundation and **Rob Sokolic** presented the results from the Cross-sectional Quality of Life Study in Patients and Families with Wiskott - Aldrich syndrome and X-Linked Thrombocytopenia. This study found that both patients with WAS and their families show decreased QOL scores, and are similar to those scores of patients with other PIDs. However, QOL scores are significantly higher in patients who have had a HCT. This study will serve as a tool to help improve the Quality of Life of patients with WAS and help both families and physicians make management decisions. Thank you **Sumathi** and **Rob**!



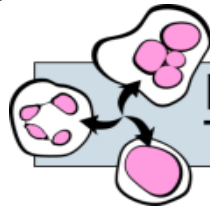
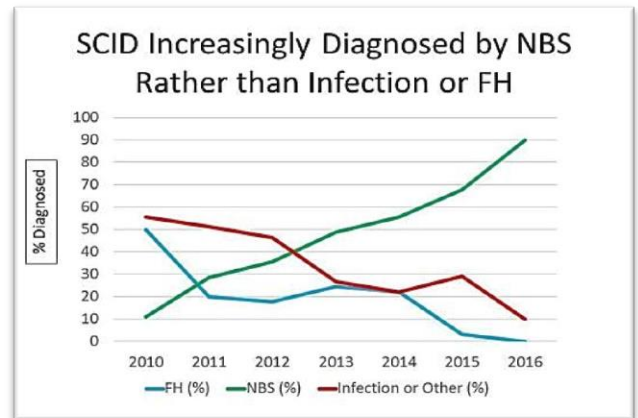
## 6901 Prospective SCID Update

Our First 100 patients manuscript is currently underway and the first draft should be released soon. We would like to recognize **Dr. Jennifer Heimall** of CHOP and **Dr. Chris Dvorak** of UCSF for all of their efforts on both the abstract presented at ASBMT and the manuscript. Thank you to the both of you! Thank you to all the centers who contributed patients to the first 100!

We recently implemented "**Infectious Disease Assessment and Monitoring**" forms. Thank you to those centers who have completed these forms for patients included in the First 100. Centers should bear in mind that they should be going back and completing these for all 6901 patients.

We would also like to remind centers of the importance of completing the **Quality of Life surveys** particularly for prospective patients. We would like to see an improvement in the completion rate of these forms over the next year! Any questions regarding these or other 6901 inquiries please contact [Tara.Bani@ucsf.edu](mailto:Tara.Bani@ucsf.edu).

Dr. Dvorak, our 6901 PI, recently shared some interesting findings regarding the increased incidence of SCID diagnosis by New Born Screening rather than infection or family history (FH) amongst our 6901 patients since the implementation of the Newborn Screening Program in 2010.



## Primary Immune Deficiency Treatment Consortium

## PIDTC Patient Highlight

UCSF'S EVERETT



Everett is a SCID patient cared for at UCSF. Here he is with his dad playing with bubbles at UCSF Benioff Children Hospital's PID bubble day!

The PIDTC wants to hear about your patients! If you would like one of your patients featured in next quarter's issue, Please send a photo and a brief blurb to [Megan.Murnane@ucsf.edu](mailto:Megan.Murnane@ucsf.edu)

### ENROLLMENT GOALS AND CURRENT ACCRUALS:

28% of goal

28 **6902** Cross-sectional visits to reach our goal of 100 additional visits in year 7

43% of goal

114 **6903** CGD retrospective patients enrolled and completed of 260 total patients by year 7 end

43% of goal

66 **6904** WAS retrospective patients enrolled and completed of 155 total patients by year 7 end



	6901	6902	6903	6904
<b>Accruing Sites</b>	30	33	31	32
<b>Current Year (Since Aug 2015)</b>	37	5	97	69
<b>Total</b>	208	732	189	123

For any inquiries regarding PIDTC patient accrual or DMCC related inquiries please contact the DMCC project manager, Rosalie Holland at [Rosalie.Holland@epi.usf.edu](mailto:Rosalie.Holland@epi.usf.edu)

## 6902 Retrospective and Cross-sectional SCID Update

The 6902 retrospective study was closed this year and the data sets for all strati are almost complete. With the conclusion of this extensive data clean up, thanks to **Brent Logan**, **Elie Haddad**, and **Tara Bani**, and the rest of the 6902 protocol team, the manuscript is underway! We would like to thank all of these people for their relentless efforts over the past year on this study!

### **REMINDER:** Sites should be recruiting for the 6902 Cross Sectional Study!

We are still actively recruiting patients for the cross sectional cohorts, and we ask that sites be trying their hardest to get in touch with their older SCID patients to bring them back in. Our goal is to bring in an additional 200 patients for a cross-sectional visit through this year and next by August 2017. Sites will receive a reimbursement of \$750 per patient brought in for a cross-sectional visit. Please contact **Tara Bani** at [Tara.Bani@ucsf.edu](mailto:Tara.Bani@ucsf.edu) for 6902 inquiries.

## 6903 Chronic Granulomatous Disease & 6904 Wiskott - Aldrich syndrome Update

Our 6903 and 6904 studies were initiated this year at a majority of sites, with the aim to enroll at least half of our projected WAS and CGD patients in the retrospective cohorts. We have had 3 deadlines this year, and are approaching our final one for year 7 one at the end of the month, **August 31, 2016**. These deadlines will continue through year 8, and require a percentage of your site's retrospective patient's CRFs to be completed for both 6903 and 6904. Our project manager, Megan Murnane, has sent out notice to each site regarding the **August 31<sup>st</sup> Deadline**. A majority of our centers are behind on these deadlines, we ask that all centers dedicate time to these enrollments and CRFs as soon as possible.

Sites will receive a reimbursement of \$800 per retrospective patient upon the completion of all CRFs for that patient. From prospective patients, only enrollment is required for reimbursement.

For questions concerning the 6903 and 6904 studies and the retrospective deadline, please contact Megan Murnane at [megan.murnane@ucsf.edu](mailto:megan.murnane@ucsf.edu).

## 2016 Annual PIDTC Scientific Workshop and Education Day Recap

HOSTED BY UCLA AND CHLA IN LOS ANGELES, CA

We would like to extend a special thanks to the 2016 workshop committee:



Don Kohn, Chair



Caroline Kuo, Co-Chair



Mike Pulsipher, Co-Chair



Neena Kapoor, Co-Chair



Katia Dahan



and Serge Dahan, coordinators

As well as all of our PIDTC PIs:

**Morton J Cowan, Luigi Notarangelo, Elie Haddad, Chris Dvorak, Sung-yun Pai, Marcia Boyle, Lauri Burroughs, Elizabeth Kang**

This year's annual Scientific Workshop was held in Los Angeles at the beautiful Marina Del Rey Marriott. We had over 120 attendees at the workshop and about 22 Education Day Participants. Everyone in attendance posed rooftop style during Friday's lunch on the roof of the Marina Del Rey Marriot overlooking the beachy scenery (pictured below)! We would also like to thank all of our speakers! During the 3 day event, we heard some great talks from: **Maria Grazia-Roncarolo, Michael Keller, Maria Cavazzana, Art Weiss, Stephen Smale, Ellen Rothenberg, Sumathi Iyengar, Rob Sokolic, Christopher Scalchunes, Heather Smith, Ingrid Caras, Troy Torgerson, Andrew Gennery, Alice Chan, Satiro De Oliveira, Harry Malech, John Levine, Marie Bleakley, Jacques Galipeau, and Gay M Crooks.**

We would further like to acknowledge our top abstract winners who gave presentations at the workshop: **Rebecca Marsh, Sushmita Roy, Andrew Gennery, and Rosa Bacchetta**, as well as our fellowship awardees: **Caroline Kuo, Teresa Tarrant, and Julia Chu.**

We concluded our talks on Friday with a lovely surprise sunset dinner cruise aboard the Dandean Mega Yacht as we circled the Marina Del Rey Harbor! What a treat!

If you were an attendee at this year's Workshop or Education Day and have not completed the follow up surveys, please do so as soon as you can. Contact Megan Murnane at [megan.murnane@ucsf.edu](mailto:megan.murnane@ucsf.edu) if you need the surveys resent to you. These are very important!

The next PIDTC Scientific Workshop will be held May 24-26, 2017 in Bethesda, MD on at the NIH with Education day taking place May 23, 2017. See you next year!



# Announcements

## *Congratulations to our new Fellowship Awardees!*

This year's Fellowship Awardees were **Catherine Biggs** from Boston Children's and **Caroline Kuo** from UCLA!



Catherine Biggs, MD



Caroline Kuo, MD

## *The PIDTC is on Facebook!*



Like our page on Facebook!

The PIDTC recently joined the Facebook community as a resource to stay connected with our patients and provide them with useful updates on our research! Check us out at: <https://www.facebook.com/RDCRN.PIDTC/>

## *The PIDTC Pilot Project winner announced soon!*

The winner of the Pilot Project is currently being selected and will be announced on:

**August 22, 2016**

## *If you are a patient and would like to participate...*



### **Join the RDCRN PIDTC Contact Registry!**

The Contact Registry is a way for patients with primary immune deficiency and their family members to learn about PIDTC research studies and find out if they may be eligible to participate on one of our studies. Registration is completely voluntary and you may choose to withdraw at any time. There is no cost to join the Contact Registry.

Follow the link to join today:

<https://www.rarediseasesnetwork.org/cms/pidtc/Get-Involved/ContactRegistry>

## RDCRN/ DMCC Update

BY ROSALIE HOLLAND

Over the past year there were many exciting additions to the RDCRN website. In the beginning of this year we were able to successfully implement the eReg binder, which allows for electronic storage of all regulatory items for the PIDTC and will allow the DMCC, UCSF, and sites to coherently track regulatory status. The project managers and I would like to thank all of the research coordinators at every site for their hard work in making this process happen!

We also launched our new public website: <https://www.rarediseasesnetwork.org/cms/PIDTC>. A big thanks to **Dr. Suhag Parikh** and **Dr. Geoff Cuvelier** for your work on the website! If you have not provided the contact for a transplant and an immunologist for your site, please do so ASAP. The website is serving as a great resource for PID patients and families particularly through the use of our contact registry which allows us to connect our patients with PIDTC centers and our studies!

Additionally, we implemented new monthly CRF alerts to all sites and "real time" data cleanup, that allows every center to more efficiently track their CRF completion.

Lastly, we are currently working on the initiation of a central IRB for PIDTC. We have distributed the reliance agreement to all centers and so far 13 centers have signed, 9 are still reviewing it, and 16 have declined.

It has been a pleasure working with everyone through all of these changes and I look forward to the benefits these advancements will reap in year 8!

For any DMCC related inquiries please contact me, Rosalie Holland at: [Rosalie.Holland@epi.usf.edu](mailto:Rosalie.Holland@epi.usf.edu)



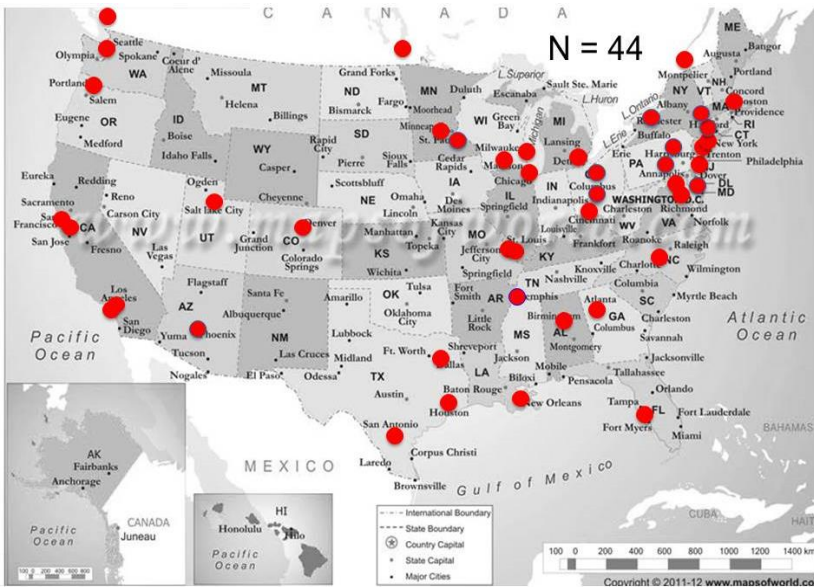
*The PIDTC is part of Rare Diseases Clinical Research Network (RDCRN), an initiative of the Office of Rare Diseases Research (ORDR), NCATS. The PIDTC is funded through collaboration between NCATS, and the NIH/NIAID*

*The PIDTC has expanded!*

We wish to welcome **St. Jude's Children's Hospital** who will be joining us on all of our studies!  
Here is a map of the PIDTC's total landscape!

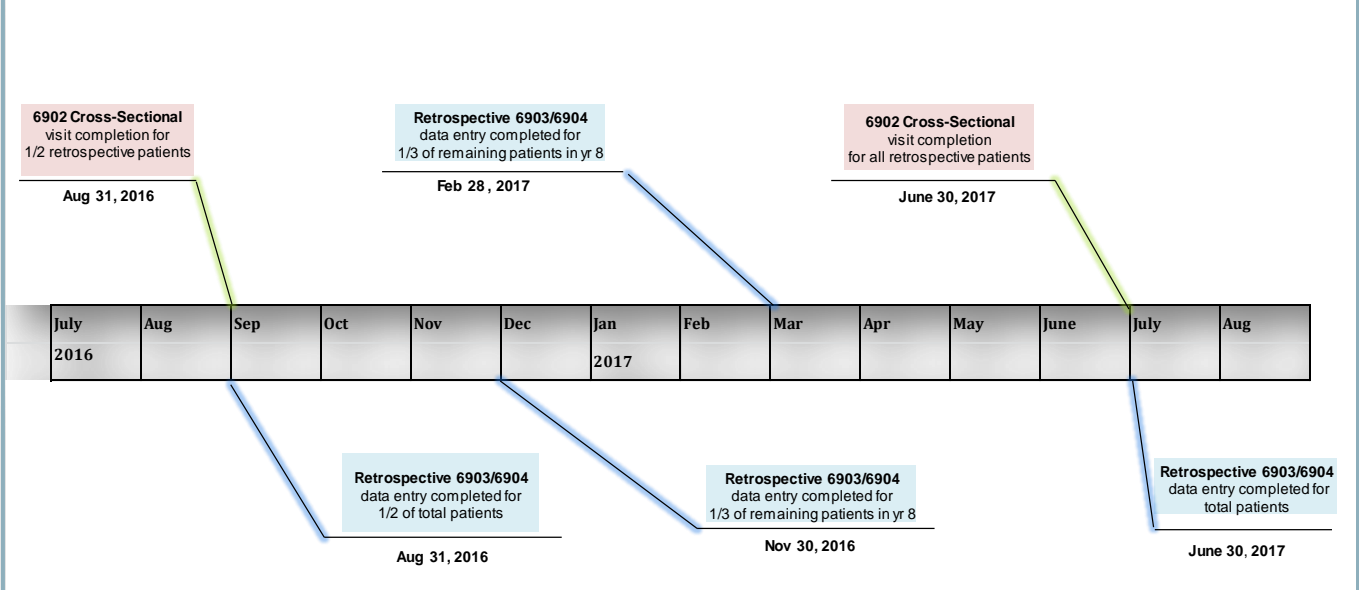
We are also pleased to announce that this year we added three international centers to our 6903 protocol:

- **Great Ormond Street in London**
- **The University of Zurich**
- **Newcastle**



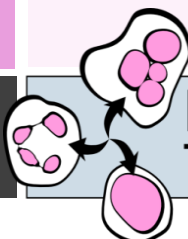
# Deadlines

## Project Timeline



**PIDTC Newsletter**

Brought to you by Megan Murnane and the PIDTC Management Team



**Primary Immune Deficiency Treatment Consortium**

For questions about PIDTC Newsletter, please contact:  
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