



Primary Immune Deficiency Treatment Consortium

PIDTC Newsletter

01 April 2016
Issue #2

PICTURED ABOVE: ANNALOU (SEE PG 2)

IN THIS ISSUE

Quarterly Update from Dr. Cowan

As we enter our next quarter of year seven I want to recap this year's goals for the 6902 cross-sectional study, and the 6903 and 6904 retrospective studies. Many centers have made a significant effort in getting the ball rolling with these studies, but I would like to see this momentum continue to pick up going forward. Our progress to date:



16 of 100

16 Additional **6902 Cross-sectional** visits of our goal of 100 visits by August 31, 2016



40 of 145

40 **6903 CGD** retrospective patients completed of this year's goal of 145 total patients by August 31, 2016



29 of 157

29 **6904 WAS** retrospective patients completed of this year's goal of 157 total patients by August 31, 2016

I cannot emphasize enough the importance of capturing these 6902 cross-sectional patients. I thank all centers who have been working hard to track these patients down and bring them in for visits! I ask that centers

be making a strong effort over the next year and a half to reconnect with these patients, and remember we will be reimbursing centers \$750 per patient brought in for a visit either directly or remotely!

I want to encourage all centers to start the retrospective enrollments for 6903 and 6904 as soon as they can. Getting these patients through the enrollment process sooner rather than later will help centers significantly to meet the deadlines for these studies. I have full confidence that with every center's dedication to these studies that we can achieve these goals. Thank you to all for your hard work!

As all of you are aware, we are in the process of implementing a central IRB across centers for our PIDTC studies. Centers are currently reviewing this with their institutions' IRB, and I am pleased to report that six centers have already signed the reliance agreement with UCSF!

Again, thank you all for your contribution to these studies and I am looking forwards to seeing you all at the PIDTC Scientific Workshop in Los Angeles on May 19th to the 21st!

Thank you,

Dr. Mort Cowan

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See you all May 19th at the Workshop!

This year CHLA and UCLA are hosting the PIDTC Workshop in Los Angeles at the Marina Del Rey Marriott pictured above! If you are attending and have not registered please do ASAP with Katia Dahan at katia.dahan@gmail.com

PAG Update

We are pleased to inform you that the WAS Quality of Life Survey poster will be presented at the CIS meeting in May, and the manuscript is in preparation.

Michael Keller and the Immune Deficiency Foundation have sent out a survey recently to all PIDTC SPC Members to assess providers' perceptions regarding mental health screening for caregivers of patients with primary immunodeficiencies. Thank you to all of those who participated, and if you have not yet and would like to participate please follow the link below to complete the survey:

<https://www.research.net/r/GVYJRHL>

6901 Prospective SCID

Our First 100 patients abstract, *Poor T cell reconstitution at 100 days after T cell-replete hematopoietic cell transplantation (HCT) for SCID is associated with later risk of death or need for 2nd transplant in the 6901 Prospective Study of the Primary Immune Deficiency Treatment Consortium*, was presented at ASBMT this year as one of the best pediatric abstracts. The manuscript is currently in preparation.

Any questions regarding 6901 and 6902 matters, please contact Tara Bani at tara.bani@ucsf.edu

6902 Retrospective and Cross-sectional SCID Update

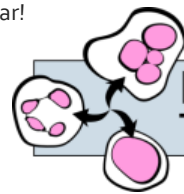
Our 6902 Manuscript is currently underway! **Dr. Elie Haddad** and **Dr. Brent Logan**, along with our 6902 protocol team, have been working diligently through our data. Thank you for all of your efforts and thank you to all sites that have been working hard to respond to the recent queries! Sites that received queries for the Stratum A-subsequent transplants, this round of queries will be due April 15, 2016. Tara Bani will be sending out notices of future query deadlines.

We would like to remind sites that we are still actively recruiting patients for the cross sectional cohort. Our goal is to bring in an additional 200 patients for a cross-sectional visit over the next 2 years, and complete half of that by August 31, 2016. Sites will receive a reimbursement of \$750 per patient brought in for a cross-sectional visit. Sites are welcome to use this reimbursement however they choose, but reimbursements to patients for travel expenses are highly encouraged. Please be on the lookout for 6902 Cross-sectional updates and deadlines from Tara Bani, over the next year!

6903 CGD and 6904 WAS Update

We just completed our first deadline for the retrospective cohorts of both 6903 and 6904. Thank you to all sites that were able to meet this deadline; we really appreciate your hard work! We have seen a decent rate of enrollment, but there is room for improvement going into the next deadline! We hope to enroll and complete CRF data entry for half of our expected retrospective patients for both studies by August of this year. Our project manager, Megan Murnane, has sent out a notice to each site regarding the next deadline: **May 30, 2016**. This deadline is for the enrollment and CRF completion for 1/6th of the total retrospective CGD and WAS patients at your center. Please remember to submit eligibilities as soon as possible, as the review process may take time. We hope to see an upward trend in participation from centers this deadline! Sites will receive a reimbursement of \$800 per patient upon the completion of all CRFs for that patient.

Regarding the 6903 and 6904 studies and the retrospective deadline, please contact Megan Murnane at megan.murnane@ucsf.edu.



Primary Immune Deficiency Treatment Consortium

PIDTC Patient Highlight

UCSF'S ANNALOU



Annalou is California's first patient with SCID diagnosed by the New Born Screening program. She received a haplocompatible CD34+ HCT from her mother without any conditioning and has full T cell and B cell reconstitution. She is now 5 years old and still sporting that cute smile!

Here she is pictured with Dr. Puck and Dr. Cowan!

CURRENT ENROLLMENTS:

	6901	6902	6903	6904
Enrolling Sites	30	33	24	27
Current Year Enrollments (Since Aug 2015)	19	1	48	38
Total Enrollments	190	728	128	80

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

Announcements and Deadlines

2016 PIDTC Scientific Workshop and Education Day

Scientific Workshop: **May 19th - May 21st 2016**

Education Day: May 18th 2016

Los Angeles, CA

PIDTC Pilot Project

May 31, 2016:

Submit and optional letter of intent (500 words) that describes specific aims, hypothesis, and approach. If you are concerned about the appropriateness of the topic please submit your letter of intent in advance of starting the application to Elizabeth Dunn at Elizabeth.Dunn@ucsf.edu.

June 30, 2016:

Submit application as an electronic pdf file to Elizabeth Dunn, PIDTC Project Manager at Elizabeth.Dunn@ucsf.edu. The application must include: a letter of support from the PIDTC site PI, a Biosketch, any other additional support, and the application- no more than 3 pages excluding references (single spaced, Ariel 11 font, 0.5 inch margins). The application should be completed in NIH grant format including: specific aims, significance, innovation, and approach.

August 22, 2016:

Notification of award.

Deadlines

