Quarterly Update from Dr. Cowan

As we begin year seven, I am pleased to announce some exciting new additions to the PIDTC! As most of you are aware, our 6903 and 6904 studies are now activated and running at most sites. Many sites have already begun enrolling CGD and WAS patients at a commendable rate. Be on the lookout for notices from our project management team as we aim to accomplish our 6903 enrollment goals for these studies.

In addition to opening 6903 and 6904, we have nearly completed the activation of 9 new sites to the PIDTC which will be participating in our 6901, 6903, and 6904 studies. I would like to thank the research coordinators and principal investigators at each of the following sites for your hard work during the taxing activation process:

- A.I. duPont Hospital for Children, Nemours
- Children's Hospital of Pittsburgh
- Columbia University
- Mayo Clinic
- Nationwide Children's Hospital
- New York Medical College
- Phoenix Children's Hospital
- University of Rochester
- Rainbow Babies

Welcome! In continuing with exciting news, Dr. Sung-Yun Pai and Dr. Mike Pulsipher presented our newest protocol, 6905, Balsufan dose finding study, to NIAID and submitted the grant application Dec 4th. I would like to extend a large thank you to Drs. Pai and Pulsipher for all of their hard work as well as to over 40 PIDTC and PBMTC centers that have agreed to participate in this groundbreaking study!

I look forward to celebrating all of these accomplishments with you at this coming year's PIDTC Scientific Workshop in Los Angeles on May 19th to the 21st! I thank every site for their commitment to the research of the PIDTC! As we go forth in year seven I wish to conclude with a reminder of this year's goals for 6902, 6903, and 6904. I have full confidence that with our continued level of excellence we will succeed in achieving these goals!

<table>
<thead>
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<th>66% of goal</th>
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<td>Additional 6902 Cross-sectional visits to reach our goal of 300 total by year 8</td>
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<th>50% of goal</th>
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<td>Additional 6903 CGD retrospective patients to enroll and complete of 288 total patients</td>
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<table>
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<th>50% of goal</th>
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<tr>
<td>Additional 6904 WAS retrospective patients to enroll and complete of 294 total patients</td>
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Thank you all, Mort
PAG Update

We are pleased to announce that Dr. Suhag Parikh from Duke will be joining Dr. Jennifer Puck as the new junior liaison between the PIDTC and the Patient Advocacy Groups! Thank you, Suhag; we are very pleased to have you in this role.

Currently, the PIDTC is exploring the possibility of adding a developmental testing component to the cross-sectional visits, at least for selected patients who are more than 8 years post-transplant. We are hoping to be able to use testing tools that can be administered by the centers without the need of a neuropsychologist, which would be quite expensive to implement.

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 submitted to ASBMT in early October. The abstract has been chosen for oral presentation at the Pediatric Plenary Session. Congratulations to Chris Dvorak, Jen Heimall, Brent Logan and the members of the 6901 Protocol Working Team for all their efforts to get this done. Currently, the manuscript is underway and a good draft should be released soon!

6902 Retrospective and Cross-sectional SCID Update

As of this past year, the Straturn A retrospective cohort is closed, with Straturn B and C soon to follow. However, we would like to remind sites that we are still actively recruiting patients for the cross-sectional cohorts. Our goal is to bring in an additional 200 patients for a cross-sectional visit over the next 2 years, and completing half that by August 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. Please be on the lookout for 6902 Cross-sectional updates and deadlines from our new project manager, Tara Bani, over the next year! Please email her with any inquiries at Tara.Bani@ucsf.edu.

In other 6902 news, the data clean-up of the 6902 dataset is currently underway, and we would like to acknowledge Dr. Elie Haddad, Dr. Monica Thakar, Dr. Richard O’Reilly, Dr. Sung-Yun Pai, Dr. Mark Vander Lugt, Dr. Luigi Notarangelo, Dr. Mort Cowan, Dr. Brent Logan, Dr. Linda Griffith, and Dr. Jennifer Puck for all their efforts on this!

6903 CGD and 6904 WAS Update

Our 6903 and 6904 studies are now up and running at a majority of our sites! This year we aim to enroll at least half of our projected WAS and CGD patients in the retrospective cohorts. In order to meet this goal we will be setting 3 deadlines this year, each requiring 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 notices to each site with the first deadline: Feb 28, 2016. Sites will receive a reimbursement of $800 per patient upon the completion of all CRFs for that patient. All the electronic CRFs for the retrospective 6904 cohort are available on the DMCC website. The 6903 retrospective cohort eCRFs will be available on December 18th, 2015. Paper CRFs are provided for your use until the electronic CRFs are available.

For questions concerning the 6903 and 6904 studies and the retrospective deadline, please contact our project manager, Megan Murnane at megan.murnane@ucsf.edu.

Primary Immune Deficiency Treatment Consortium

DMC Update

BY ROSALIE HOLLAND

As you are all well aware, over the past quarter we were able to implement full use of the eRegulatory binder across all sites. The eReg binder 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!

This past quarter we also were able to announce the completion of the new public website for the PIDTC. Please follow this link if you have not already checked it out! 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 transplanter and an immunologist for your site, please do so ASAP.

DMCC Update

BY DR. SUNG-YUN PAI

Dr. Pulsipher and I presented the 6905, Bulsufan Dose Finding study, to NIAID in October, and the grant application was submitted December 4th.

We are overwhelmed by all of the support we have received on this exciting study with over 40 PIDTC and PBMTC centers that have agreed to participate! We would like to thank everyone for their letters of support!

For any DMCC inquiries contact Rosalie Holland at Rosalie.Helfrich@epi.usf.edu

CURRENT ACCRUALS:

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Announcements and Deadlines

**PIDTC Research Award**

**The application deadline for the PIDTC Research Award has been extended to **Tuesday, December 15, 2016.**

The purpose of the award is to encourage research in transplantation, gene therapy or outcomes of primary immune deficiency therapies; however, applications may be on any aspect of primary immune deficiencies. It is open to trainees or Junior Faculty with 2 years or less as a faculty member. It will be awarded for the academic year beginning July 1, 2016 and will provide $25,000 salary/consumables support through June 30, 2017.

Please send a CV, letter from your program director, letter from your department chair documenting sufficient resources and time commitment to complete the project, letter from your mentor, and a three page NIH-style grant application to Dawn Westerfer: WESTERFER@email.chop.edu. Please

**2016 PIDTC Scientific Workshop and Education Day**

Scientific Workshop: May 19th - May 21st 2016
Education Day: May 18th 2016
Los Angeles, CA

**Other Deadlines**

- First 6903/6904 Retrospective CRF Completion Deadline: February 28, 2016
- The Pilot Project Application deadline will be announced shortly!