Primary Immune Deficiency Treatment Consortium (PIDTC)  
Fourth Annual Scientific Workshop  

May 1 - 3, 2014, Seattle Children’s Hospital Research Center

DAY 1 Thurs, May 1:

12 Noon-1PM: REGISTRATION AND LUNCH (60’)

1:00–1:40PM: PIDTC STUDIES (40’)
  1. Introduction and Overview of the PIDTC project: Morton J. Cowan (20’)
  2. Comments from NIAID: Linda Griffith (10’)
  3. Data Management and Coordinating Center: Jeffrey Krischer (10’)

1:40–2:55PM: PROGRESS ON PIDTC PROTOCOLS (75’)
Moderator: Luigi Notarangelo
  1. Statistical aspects of PIDTC studies: Brent Logan (15’)
  2. 6901 update: Chris Dvorak and Rebecca Buckley (15’)
  3. 6902 update: Elie Haddad and Richard O'Reilly (15’)
  4. 6903 update: Elizabeth Kang and Harry Malech (15’)
  5. 6904 update: Lauri Burroughs and David Rawlings (15’)

2:55-3:35PM: INVITED SPEAKER (40’)
  1. Recognition of Trudy Small: Richard O'Reilly (10’)
  2. Reconstitution of B cell maturation and function post transplant
     Speaker: Jayanta Chaudhuri (MSKCC) (30’)

3:35-4:00PM: COFFEE/SNACK BREAK (25’)

4:00-4:40PM: NEWBORN SCREENING FOR SCID (40’)
Moderator: Jennifer Puck
  1. Newborn screening for SCID in the United States
     Speaker: Roshini Abraham (20’)
  2. Disposition of patients with low TREC on NBS who do not have a SCID genotype
     Speaker: Jennifer Puck (20’)

4:40-5:20PM: UPDATE ON PROSPECTIVE NB SCID TRIAL (40’)
Moderator: Mike Pulsipher
  1. Introduction and update: Sung-Yun Pai (10’)
  2. Core labs and research studies: Paul Szabolcs (15’)
  3. Recruitment and implementation: Chris Dvorak (15’)

5:20-6:30PM: RECEPTION AND END OF DAY 1
DAY 2 Fri, May 2:

7:00-8:00AM: **BREAKFAST (60')**

8:00-8:30AM: Patients transplanted for SCID in North America 2000-2009  
**Moderator:** Richard O'Reilly  
**Speaker:** Sung-Yun Pai (20'+10' discussion)

8:30-9:40AM: **GENE THERAPY UPDATE (70')**  
**Moderator:** Don Kohn  
1. **Update on XSCID and WAS trials in Europe**  
   **Speaker:** Alain Fischer (20')  
2. **Updates on gene therapy in the US**  
   **Speakers:** Luigi Notarangelo (XSCID), Harry Malech (XSCID), Sung-Yun Pai (WAS), Don Kohn (ADA-SCID and CGD) (10' each followed by 10' plenary discussion)

9:40-10:00AM: **COFFEE BREAK (20')**

10:00-10:45AM: **INVITED SPEAKER (45')**  
**Moderator:** Tom Fleisher  
**Introduce Pilot Project/T cell senescence and exhaustion post HCT for PID**  
**Speaker:** Hélène Decaluwe (30'+15' discussion)

10:45-11:05AM: Pilot Project update on B cell maturation post HCT for SCID  
**Speaker:** Sung-Yun Pai (20')

11:05AM -12:05PM: **4 BEST ABSTRACT PRESENTATIONS (60')**  
**Moderator:** Bill Shearer

12:05-1:30PM: **LUNCH (85')**  
(Breakout lunch for Trainees and Junior Faculty organized/lead by Lauri Burroughs, Sung-Yun Pai and Chris Dvorak)

1:30-2:50PM: **INTERNATIONAL COLLABORATIONS (80')**  
**Moderator:** Mort Cowan  
1. **Possible collaborations – update of IEWP:** Andy Gennery (20')  
2. **The Latin American Society for Immunodeficiencies:** Ricardo Sorensen (20')  
3. **PID in South America:** Carmem Bonfim (20')  
4. **PID in Japan:** Kohsuke Imai (20')

2:50-3:45PM: **PATIENT ADVOCACY GROUP DISCUSSION (55')**  
**Moderator:** Christopher Scalchunes  
1. **Transitioning patients to adult care:** Danielle Friedman (MSKCC) (20')  
2. **QOL in WAS families ± HCT:** Sumathi Iyengar/Rob Sokolic (15')  
3. **IDF: Newborn Screening:** Emily Hovermale (10')  
4. **Jeffrey Modell Foundation:** ?
3:45-4:45PM: COFFEE BREAK AND POSTER WALK (60’)

4:45-5:30PM: INVITED SPEAKER (45’)
   Emerging immunodeficiencies
   Speaker: Alain Fischer (30’ +15’ discussion)

5:30PM: END OF DAY 2

7:00PM: GROUP DINNER
   FareStart Restaurant
   7th and Virginia
   Ph. 206 267-7601

DAY 3 Sat, May 3:

7:00-8:00AM: BREAKFAST (60’)

8:00-8:30AM: REPORTS FROM PIDTC FELLOWSHIP AWARDEES (30’)
   Moderator: Linda Griffith
   1. Carolyn Kuo (15’)
   2. Katja Weinacht (15’)

8:30-9:30AM: DEBATE: Splenectomy for WAS: should you or shouldn’t you? (60’)
   Moderator: Luigi Notarangelo
   Speakers: Adrian Thrasher (Pro) vs Hans Ochs (Against)

9:30-10:15AM: INVITED SPEAKER (45’)
   Update of XLT
   Speaker: Michael Albert (30’ +15’ discussion)

10:15-10:45AM: COFFEE BREAK (30’)

10:45-11:30AM: INVITED SPEAKER (45’)
   Common lymphoid progenitors in mice and humans
   Speaker: Irv Weisman (30’ +15’ discussion)

11:30AM-12:15PM: B Cell Mediated Autoimmunity in Mice and Humans with WAS
   Speaker: David Rawlings (30’ +15’ discussion)

12:15PM: ADJOURN