

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

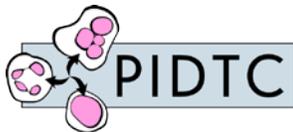
1. **Newborn screening for SCID in the United States**
Speaker: Roshini Abraham (20')
2. **Disposition of patients with low TRECs on NBS who do not have a SCID genotype**
Speaker: Jennifer Puck (20')

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: H  l  ne 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')

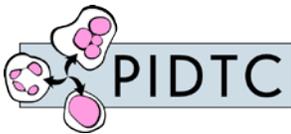
Moderator: Mort Cowan

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