



Public Meeting on Patient-Focused Drug Development for Patients Who Have Received an Organ Transplant

September 27, 2016

- 8:00 – 9:00 am **Registration**
- 9:00 – 9:05 am **Welcome**
Meghana Chalasani
Office of Strategic Programs (OSP), Center for Drug Evaluation and Research (CDER), FDA
- 9:05 – 9:10 am **Opening Remarks**
Edward M. Cox, MD, MPH
Director, Office of Antimicrobial Products (OAP), CDER, FDA
- 9:10 – 9:20 am **Overview of FDA’s Patient-Focused Drug Development (PFDD) Initiative**
Theresa Mullin, PhD
Director, OSP, CDER, FDA
- 9:20 – 9:30 am **An Overview of Organ Transplants and Available Post-Transplant Treatment Options**
Marc Cavaillé-Coll, MD, PhD
Medical Officer, Division of Transplant and Ophthalmology Products (DTOP), OAP, OND, CDER, FDA
- 9:30 – 9:35 am **The Road from PFDD Meetings to Clinical Trial Endpoints**
Michelle Campbell, PhD
Clinical Outcomes Assessment (COA) Staff, Office of New Drugs, CDER, FDA
- 9:35 – 9:45 am **Overview of Discussion Format**
Sara Eggers, PhD
OSP, CDER, FDA
- 9:45 – 10:15 am **Panel #1 Discussion on Topic 1**
A panel of patients will provide comments to set up the context on life after receiving an organ transplant
- 10:15 – 10:45 am **Large-Group Facilitated Discussion on Topic 1**
Patients and patient representatives in the audience are invited to add to the dialogue
- 10:45 – 11:00 am **Break**
- 11:00 – 11:30 **Panel #2 Discussion on Topic 2**
A panel of patients will provide perspectives on managing their condition.
- 11:30 – 12:30 pm **Large-Group Facilitated Discussion on Topic 2**
Patients and patient representatives in the audience are invited to add to the dialogue.
- 12:30 – 1:15 pm **Lunch**

Afternoon Scientific Session: Medication Adherence and Experience with Intervention

- 1:15-1:20 pm **Afternoon Opening Remarks**
Ozlem Belen, MD, MPH
Deputy Director for Safety, DTOP, OAP, OND, CDER, FDA

Scientific Discussion #1: Causes of Late Allograft Loss and The Impact of Nonadherence, Definitions, Terms, and Background

- 1:20-1:35 pm **Overview of Late Allograft Outcomes: Etiology, Risk Factors and Natural History**
Peter Nickerson, MD, FRCPC, FCAHS
Vice-Dean (Research) and Distinguished Professor, Flynn Family Chair in Renal Transplantation, Rady Faculty of Health Sciences, University of Manitoba
- 1:35 – 1:50 pm **Exploring Non-Adherence in Solid Organ Transplant Recipients**
Rita Alloway, PharmD, FCCP
Research Professor of Medicine & Director, Transplant Clinical Research, University of Cincinnati, College of Medicine
- 1:50 – 2:05 pm **Prevalence of Nonadherence after Organ Transplantation**
Mary Amanda Dew, PhD
Professor of Psychiatry, Psychology, Epidemiology, Biostatistics, and Clinical and Translational Science, Director, Clinical Epidemiology Program, Western Psychiatric Institute and Clinic University of Pittsburgh School of Medicine
- 2:05 – 2:15 pm **Adherence to Immunosuppressive Medications in Pediatric and Adolescent Transplant Recipients: A Pediatric Nephrologist's View**
Robert Ettenger, MD
Distinguished Research Professor, Emeritus, Department of Pediatrics, Division of Nephrology Mattel Children's Hospital, David Geffen School of Medicine at UCLA
- 2:15 – 2:50 pm **Panel Discussion on Session #1**
- 2:50 – 3:05 pm **Break**

Scientific Discussion Session 2: Interventions to Mitigate Non-Adherence

- 3:05– 3:20 pm **Pharmaceutical Dosage Forms to Improve Adherence: What Can be Done? What are the Limitations?**
William E. Fitzsimmons PharmD, MS
Executive Vice President, Regulatory Affairs, Astellas Pharma Global
- 3:20-3:35 pm **Interventions to Maximize Adherence after Heart, Lung, or Liver Transplantation in Adults**
Mary Amanda Dew, PhD
Professor of Psychiatry, Psychology, Epidemiology, Biostatistics, and Clinical and Translational Science, Director, Clinical Epidemiology Program, Western Psychiatric Institute and Clinic University of Pittsburgh School of Medicine
- 3:35 – 3:50 pm **Interventions to Improve Adherence Among Adult Renal Transplant Recipients**
Marie Chisholm-Burns, PharmD, MPH, MBA, FCCP, FASHP, FAST
Dean and Professor, College of Pharmacy, Professor of Surgery, College of Medicine, University of Tennessee
- 3:50 - 4:00 pm **Interventions to Improve Medication Adherence and Outcomes in Adolescent Transplant Recipients**
Robert Ettenger, MD
Distinguished Research Professor, Emeritus, Department of Pediatrics, Division of Nephrology Mattel Children's Hospital, David Geffen School of Medicine at UCLA
- 4:00 – 4:35 pm **Panel Discussion on Session #2**
- 4:35 – 4:50 pm **Open Public Comment**
- 4:50 – 5:00 pm **Closing Remarks**
Renata Albrecht, MD
Director, DTOP, OAP, OND, CDER, FDA

Discussion Questions (Morning Session):

Topic 1: Disease symptoms and daily impacts that matter most to patients

- 1) What have been the most significant changes in your overall health since you received your transplanted organ?
 - a) How long has it been since you received your transplant?
- 2) Focusing on symptoms related to your organ transplant and post-transplant effects, which 1-3 symptoms have the most significant impact on your life? (Examples may include pain, infection, anxiety, etc.)
- 3) Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your transplant? (Examples of activities may include sleeping through the night, driving, walking/running, exercising, etc.)
 - a) How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days? (Examples may include limitations on the ability to undertake physically strenuous activities, restrictions on the ability to travel, lack of appetite, fatigue, etc.)
- 4) How has your experience with your transplanted organ changed over time? Do particular symptoms come and go as your duration of time with a transplanted organ has increased? If so, do you know of anything that makes your symptoms better? Worse?
- 5) What worries you most about your health post-transplant?

Topic 2: Patients' perspectives on transplant and treatment impacts

- 1) What are you currently doing to maintain your transplanted organ or treat related health concerns following transplantation? (Examples may include immunosuppressants, antibiotics, antivirals, over-the-counter products, and other therapies including non-drug therapies)
 - a) How has your post-transplant treatment regimen changed over time, and why?
- 2) How well does your current treatment regimen manage the most significant symptoms you experience post-transplantation?
 - a) How well do these treatments improve your ability to do specific activities that are important to you in your daily life?
 - b) How well have these treatments worked for you as your experiences post-transplant have changed over time?
- 3) What are the most significant downsides to your current treatments, and how do they affect your daily life? (Examples of downsides may include bothersome side effects, need for multiple medications, risk of infection, need for hospitalization, etc.)
 - a) What are the biggest challenges you face in maintaining your post-transplant treatment regimen (Examples of challenges may be bothersome side effects, need for multiple medications, etc.)
- 4) What specific things would you look for in an ideal treatment for managing your transplanted organ?

Scientific Discussion Questions (Afternoon Session):

Panel 1 Questions:

1. How well do we understand the extent of non-adherence in patients post-transplantation? What type of non-adherence is affecting patient outcomes the most?
2. Are healthcare providers appropriately involved when it comes to promoting adherence or are they not paying enough attention? What improvements would you suggest?
3. How critical is it to collect adherence data in clinical trials of new drugs or new regimens? What are the consequences of not doing so?

Panel 2. Questions:

1. How can we incentivize (or promote) adherence?
 - a. Does one strategy work for all patients or is there a personalized way to incentivize adherence?
 - b. Would electronic monitoring help? Would keeping track of e.g., tacrolimus or cyclosporine trough concentrations help?
2. What are some barriers to increasing transplant programmatic resources allocated to promoting adherence efforts?
3. How can transplant programs help patients to support each other in their efforts to adhere to their medical regimen after transplant?
4. What medication reminder systems are most acceptable and helpful to patients?
 - a. What are the challenges to using them? How can we track the usefulness or success of these systems?
 - b. How can we harness power of “gamification” (use of game design) and health apps to support patients’ ability to track their medication taking and other medical regimen requirements?
5. What is preventing the development of more “forgiving” drugs so it would be less critical if patients miss a dose?