UCSF-Stanford CERSI

Innovations in Regulatory Science Summit

January 12, 2020

Kathy Giacomini, UCSF & Russ Altman, Stanford
Co-PIs UCSF-Stanford Center for Regulatory Science & Innovation
Research in Regulatory Science is a Critical Bottleneck

Regulatory Science: Research that helps regulators make better decisions

Kathy
Goals of Research in Regulatory Science

FDA Scientists

Evaluate and Monitor Medical Products

Research Mission

Collaboration with Academia
Centers of Excellence In Regulatory Science and Innovation, CERSIs

UCSF-Stanford

Yale-Mayo Clinic

University of Maryland

Johns Hopkins

Kathy
UCSF-Stanford CERSI Research

Collaborative Research Projects: UCSF or Stanford Faculty and FDA

• 42 collaborative research projects
• 28 active & 14 completed
• All medical product FDA centers represented

Poster Session at 4 pm
Impact of CERSI Research

Publications: Scientific Literature

- E.g., “A Research Roadmap for Next Generation Sequencing Informatics”
  Science Translational Medicine, 2016

FDA Guidelines and Practices

Excipients Database

http://excipients.ucsf.bkslab.org
Examples of CERSI Research

• Patient Preference and Patient Reported Outcomes

• Precision Medicine and Diverse Populations

• Clinical Trials

• Real World Data and Pharmacovigilance

• Biologics, Genome Editing and Cell-Based Therapies

• Devices and Digital Health

• Preclinical Discovery and Clinical Pharmacology

Research Projects Listed in Your Packets
FDA Goal: Include patient preference in regulatory decisions when weighing risks and benefits of new devices

How can FDA incorporate patient preference and experience information into their evaluation of upper limb prosthetic devices?

Leslie Wilson, Ph.D.
Patient preferences in limb prosthetic devices
FDA Goal: Improve drug labels for pediatric patients?

How can FDA ensure that drug label information is understandable for pediatric drugs?

Lee Sanders, M.D.
Bonnie Halpern-Felsher, Ph.D.

Safer Labeling of Pediatric Medications

[Image of a mother and child with a medication label diagram]
FDA Goal: Improve clinical trials-statistical framework and efficiency

- How can FDA improve the efficiency of data collection in clinical trials?
- How can FDA develop and use novel statistical methods that will allow accurate assessment of benefits/risks?

Laura Esserman, M.D., MBA
Steve Goodman, M.D., Ph.D.

OneSource Phase 2
Operational Framework for Defining Strength of Evidence in Clinical Trials
FDA Goal: Use real world data to ensure medical product safety and efficacy

- How can the FDA use UCSF/Stanford EHR data to monitor safety and efficacy of medical products?
- Can we develop tools and methods to improve the efficiency and rigor of pharmacovigilance at the FDA?

Atul Butte, M.D., Ph.D.
Russ Altman, M.D., Ph.D.

Using the University of California Data Warehouse To Monitor Biologics, CAR-T cells, Vaccines

Improving the Efficiency and Rigor of Pharmacovigilance at FDA
FDA Goal: Develop methods and standards to evaluate digital health and other new devices for safety and efficacy

- How can we develop methods to integrate digital health tools into clinical practice?
- Can we develop tools and standards for monitoring medical devices?

Andrew Auerbach, M.D.
Jeff Lotz, Ph.D.
Shuvo Roy, Ph.D.

Developing Tools for Integrating Digital Health Tools Into Clinical Practice
Advancing Computational Modeling to Evaluate Spinal Fusion Cages
Developing Tools and Standards for Thrombogenicity Testing of Dialysis and Other Medical Devices
**FDA Goal:** Improve generic drug approval processes

- Can excipients interfere with drug absorption causing bioinequivalence?

**Potential Effects on Blood Levels**

- Preservative
- Dye
- Surfactant
- Flavorant
Other CERSI Activities: Beyond Research

Educational Programs

www.ucsfstanfordcersi.org/education

FDA Scientist Visiting Scientist Program
125 FDA Visiting Scientists Since 2015

www.ucsfstanfordcersi.org/vsp
How can you participate in CERSI activities?

- Enroll in our courses or volunteer to teach
- Watch UCSF-Stanford CERSI Youtube Channel
- Attend CERSI seminars and events
- Attend future conferences
- Participate in CERSI research

Sign up for our email list at: ucsfstanfordcersi.org
Today: Call for industry participation in CERSI projects

- Patient Preference and Patient Reported Outcomes
- Precision Medicine and Diverse Populations
- Clinical Trial Design
- Real World Data and Pharmacovigilance
- Biologics, Genome Editing and Cell Based Therapies
- Devices and Digital Health
- Preclinical Discovery and Clinical Pharmacology

Complete the interest form in your packet or email info@ucsfstanfordcersi.org
Keynote Speakers and Lightning Talks

- Janet Woodcock, MD - Director, Center for Drug Evaluation and Research

- Peter Marks, MD, PhD - Director of Center for Biologics Evaluation and Research

CERSI Lightning Talks

Steve Goodman, MD, MHS, PhD
Lee Sanders, MD, MPH
Leslie Wilson, PhD
Brian Shoichet, PhD
Panel Discussions

• Accelerating Clinical Trials in the Development and Approval of Medical Products
  - Laura Esserman
  - Janet Woodcock

• Academia, Government and Industry in Regulatory Science: Cross-sector Collaborations
  - Howard Bauchner
  - Rob Califf

• Real World Evidence, Artificial Intelligence and Novel Medical Devices
  - Adam Gazzaley
  - Anne Wojcicki

• Advancing Discovery to First-In-Human Clinical Trials for New Medical Products
  - Joe Wu
  - Jay Bradner

Kathy
The UCSF-Stanford CERSI Team and Organizers

Kathy Giacomini, PhD
Co-Director

Russ Altman, MD, PhD
Co-Director

Terry Blaschke, MD
Educational Program Advisor

Kuldev Singh, MD, MPH
Organizer

Mark Dresser, PhD
Educational Director

Maria Friciello
Program Director

Lawrence Lin, PhD
Director, External Affairs & Outreach

George Scangos, PhD
Organizer

Next Speakers:
Kathy
Marc Tessier-Lavigne, PhD | President, Stanford University
Sam Hawgood, MD | Chancellor, University of California San Francisco