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

Kathy

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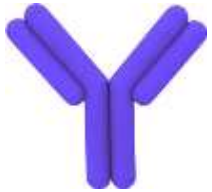
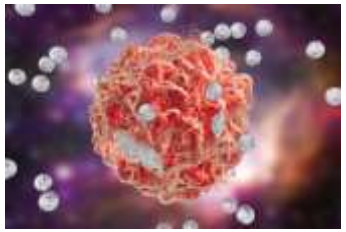
FDA Guidances and Policies Are Science-Based



Research in Regulatory Science is a Critical Bottleneck

Regulatory Science: Research that helps regulators make better decisions

Kathy

Unmet Needs: Research Helps Regulators Do Their Jobs, Altman RB et al., Science Translational Medicine, 315:22, 2015

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Goals of Research in Regulatory Science

FDA Scientists

Evaluate and Monitor Medical Products

Research Mission

Collaboration with Academia

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Centers of Excellence In Regulatory Science and Innovation, CERSIs

Yale-Mayo Clinic

UCSF-Stanford

University of Maryland

Johns Hopkins

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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

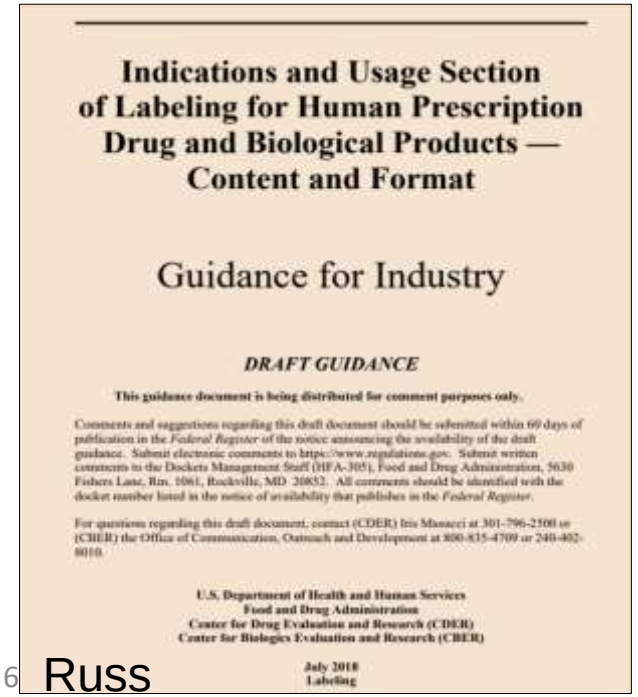
Poster Session at 4 pm

Impact of CERSI Research

Publications: Scientific Literature

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

FDA Guidances and Practices



Indications and Usage Section of Labeling for Human Prescription Drug and Biological Products — Content and Format

Guidance for Industry

DRAFT GUIDANCE

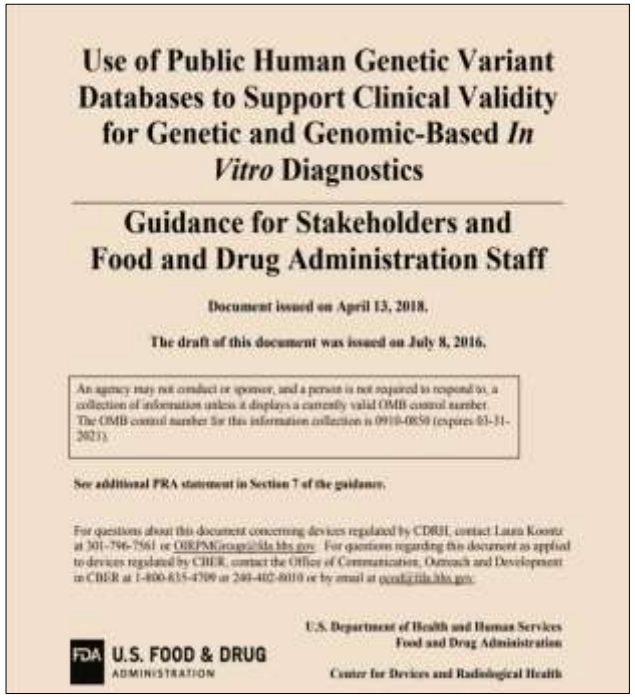
This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Iris Masucci at 301-796-2500 or (CBER) the Office of Communication, Outreach and Development at 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

July 2018
Labeling



Use of Public Human Genetic Variant Databases to Support Clinical Validity for Genetic and Genomic-Based *In Vitro* Diagnostics

Guidance for Stakeholders and Food and Drug Administration Staff

Document issued on April 13, 2018.

The draft of this document was issued on July 8, 2016.

An agency may not conduct or sponsor, and a person is not required to respond to, a collection of information unless it displays a currently valid OMB control number. The OMB control number for this information collection is 0910-0850 (expires 03-31-2021).

See additional PRA statement in Section 7 of the guidance.

For questions about this document concerning devices regulated by CDRH, contact Laura Koontz at 301-796-7561 or OIRPMGroup@fda.hhs.gov. For questions regarding this document as applied to devices regulated by CBER, contact the Office of Communication, Outreach and Development in CBER at 1-800-835-4709 or 240-402-8010 or by email at ocod@fda.hhs.gov.

U.S. FOOD & DRUG ADMINISTRATION

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health

Excipients Database

http://excipients.ucsf.bkslab.org

Russ

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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

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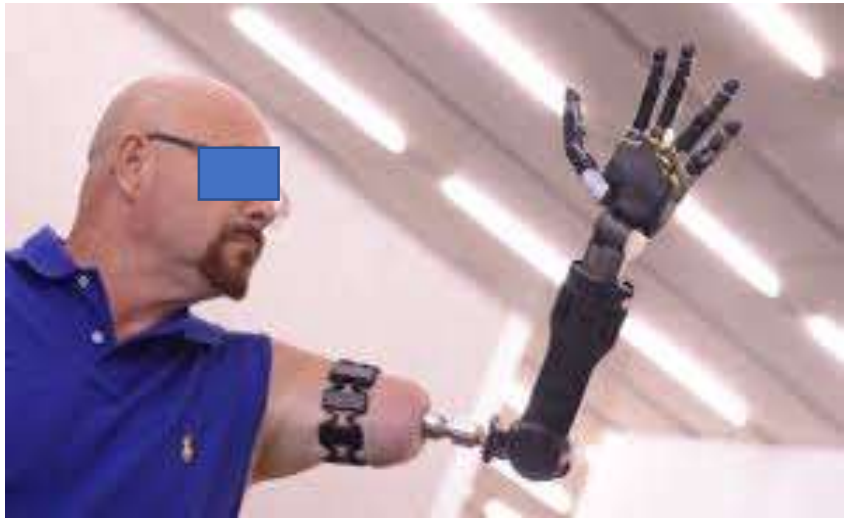
FDA Goal: Include patient preference in regulatory decisions when weighing risks and benefits of new devices

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

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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

Russ

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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

Russ

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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

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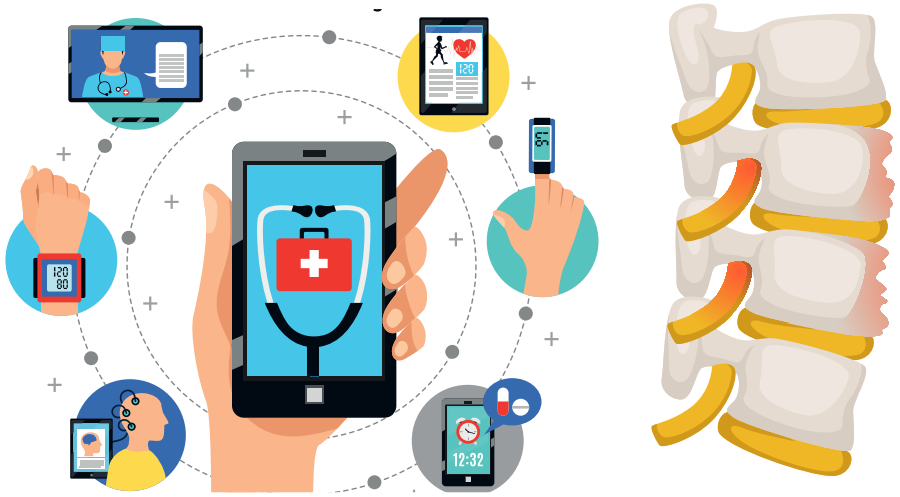
FDA Goal: Develop methods and standards to evaluate digital health and other new devices for safety and efficacy

- 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

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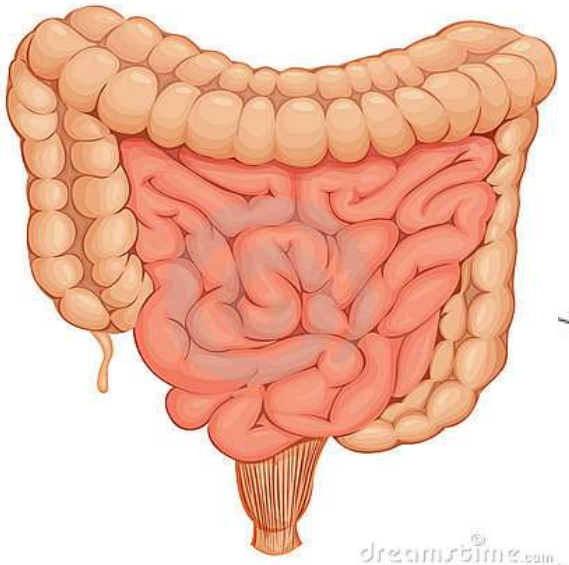
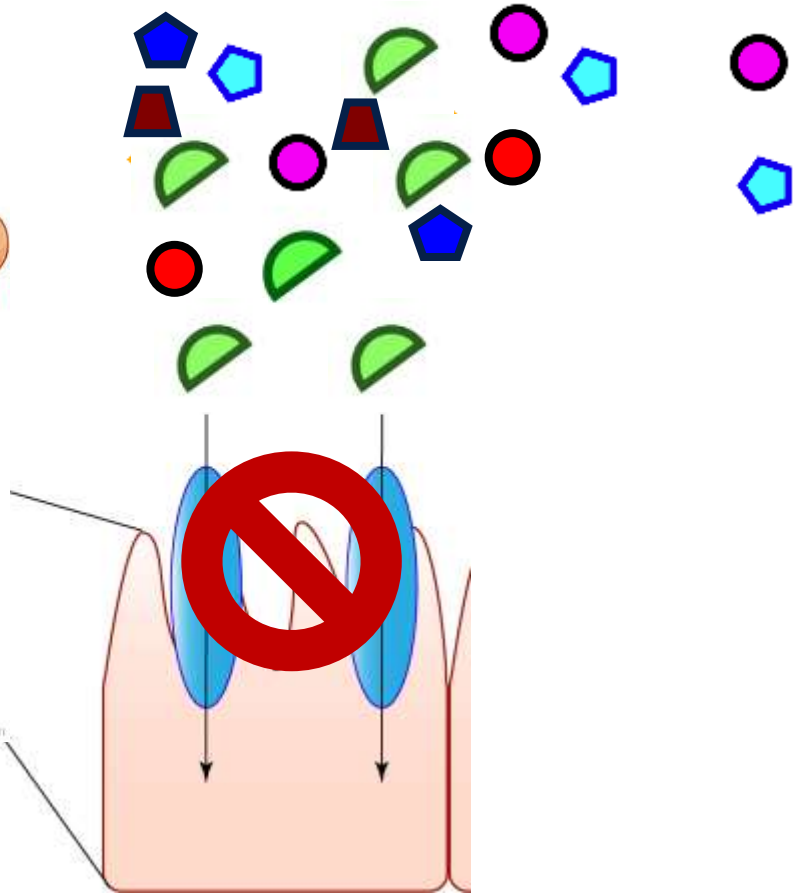
FDA Goal: Improve generic drug approval processes

- Can excipients interfere with drug absorption causing bio-inequivalence?

Active drug





Preservative

Dye

Surfactant

Flavorant

Potential Effects on Blood Levels

Systemic Circulation

Other CERSI Activities: Beyond Research

Educational Programs

Kathy Cheung, PharmD,

Jennifer Wilson, PhD,

Obi Okafor, PharmD

Yelena Ionova, PharmD,

www.ucsfstanfordcersi.org/education

FDA Scientist Visiting Scientist Program

125 FDA Visiting Scientists Since 2015

www.ucsfstanfordcersi.org/vsp

Kathy

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

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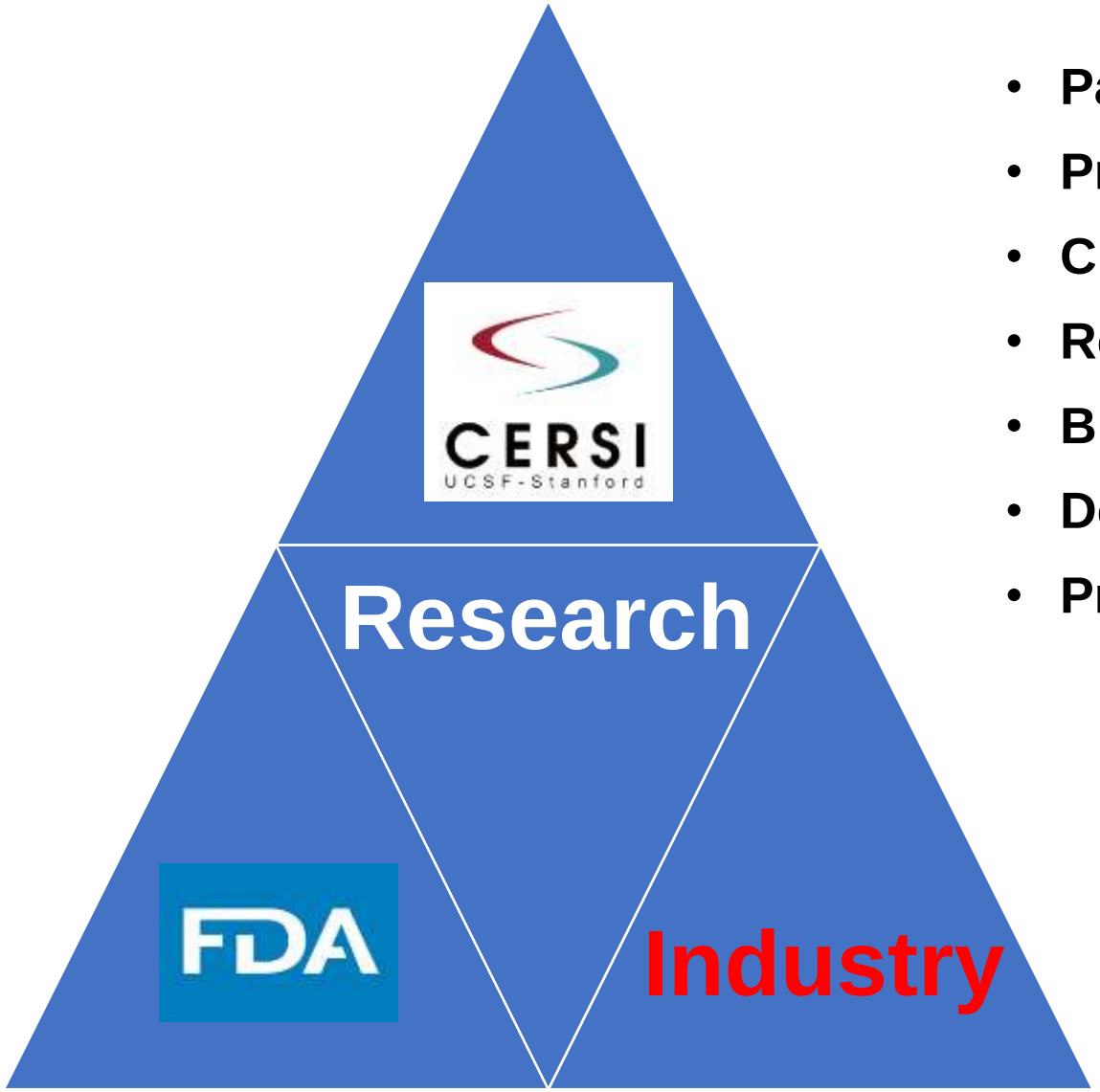
Today: Call for industry participation in CERSI projects



- 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

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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

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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

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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:

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

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