Updates in Regulatory Science

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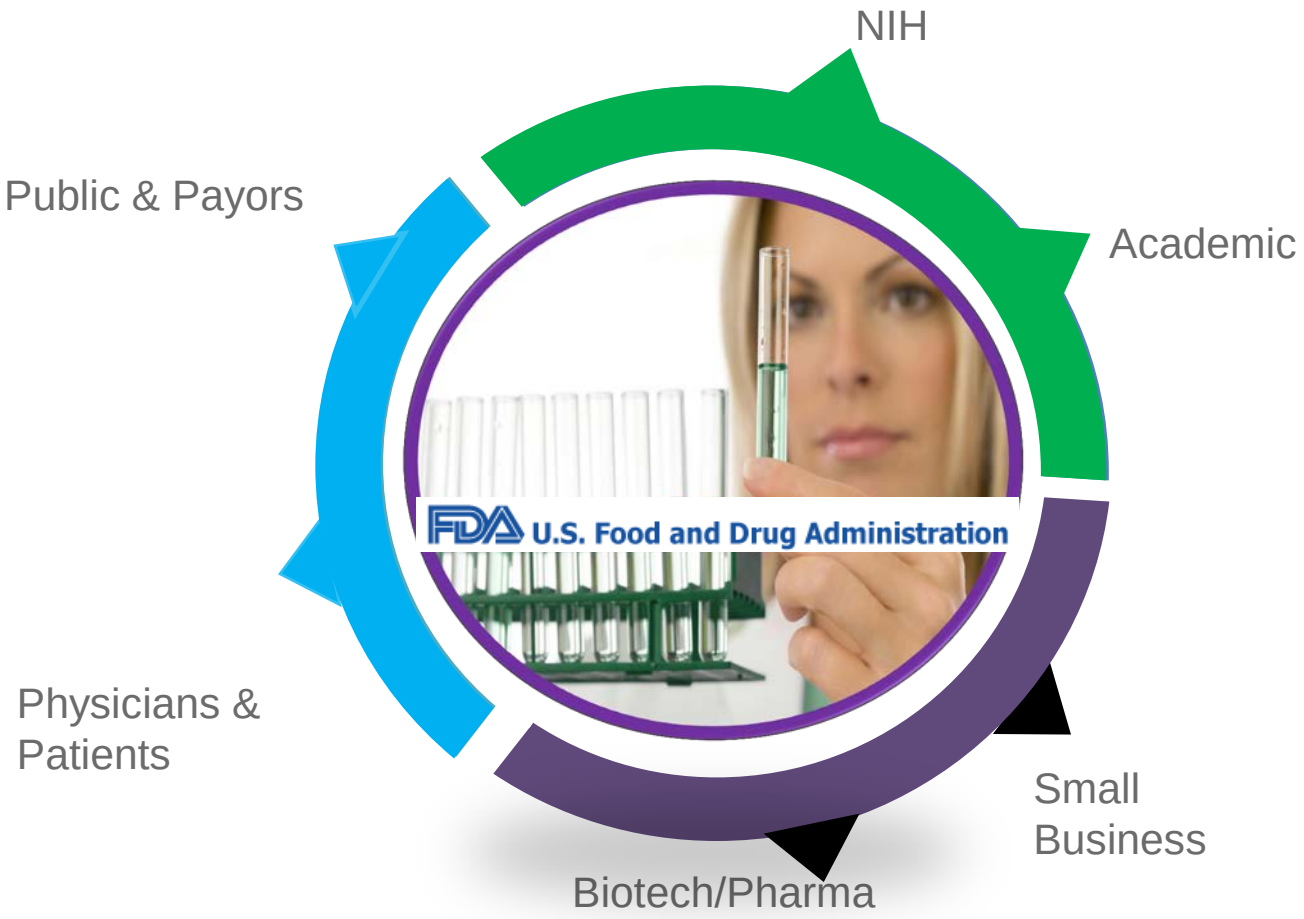
Disclaimer

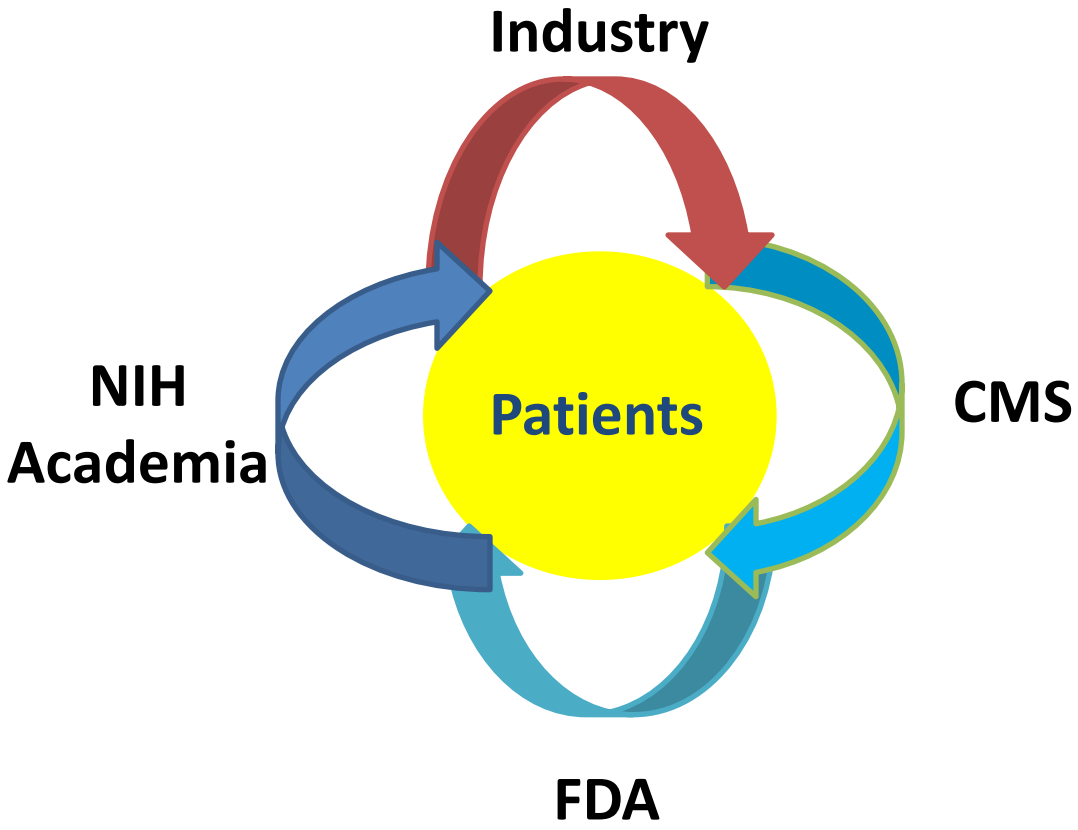
This presentation reflects the views of the authors and should not be construed to represent FDA's views or policies.

Innovation is Linked to Ecosystem and Partnerships

FDA-regulated products account for <u>25 cents</u> of every consumer dollar spent in the U.S.



New Paradigm for Product Development



Partnerships and Collaboration

- Today's challenges are too complex for any one party or sector to solve
- Urgent public health situations have *required* robust public-private partnering, formal or informal, for timely success
- Such challenges provide models for innovative partnering, *and for culture change, both inside and outside government*
- FDA is actively engaged and welcomes more ideas/models

Opportunity is in Complexity

- Successful drug (product) regulation requires that FDA performs at a high level in
  - Science
  - Law
  - Medicine
  - Policy
  - Management and execution
  - Political and stakeholder engagement

Power of PPP's:
Examples of FDA Engagement

- Biomarkers Consortium – engages FDA, NIH, industry, patients
  - I-SPY 2 trial incorporating multiple companies and academics in evaluation of 5 new agents in a novel study design for breast cancer
- ADNI: Alzheimer imaging & biomarkers
  - With NIH, 13 companies, 1300+ patients
  - Standardizing imaging methods, identifying predictive host genetic and CSF biomarkers
- Coalition Against Major Diseases
  - With patient groups, ~ 15 companies
  - Focusing on clinical data standards and early identification of neurodegenerative diseases
- OMOP: with academia, pharmaceuticals, health data systems
- CERSIs: academic research and training partnerships

Critical Path and Regulatory Science Initiatives

- More than 100 Regulatory Science-Critical Path Initiative projects (with CBER, CDER, NCTR, CVM, CDRH, CFSAN)
- FDA collaborates with more than 30 organizations on RS-CPI projects
- Research programs and science support areas
  - FDA intramural research grants
  - FDA wide science and research core infrastructure support
  - FDA wide SME scientific working groups
  - CERSI Centers of Excellence in Regulatory Science and Innovation
  - Technology Transfer and Strategic Partnerships
  - BAA Broad Agency Announcement to advance regulatory science
  - Communication, information and training hub (collaboration with OSPD, including the FDA Clinical Investigator Training course)

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OFFICE OF REGULATORY SCIENCE AND INNOVATION

U.S. FOOD & DRUG ADMINISTRATION

FDA established the Office of Regulatory Science and Innovation to help foster the creation and use of innovative tools and technologies necessary for supporting the scientific basis for regulating new medical products based on emerging technologies.

MISSION

The Office of Regulatory Science and Innovation (ORSI) supports FDA regulatory science research and innovation by:

- Developing collaborations with stakeholders and public-private partnerships
- Funding and facilitating cross-center intramural research that addresses the challenges of new and emerging products
- Funding extramural research to solve regulatory science challenges where FDA lacks the in-house resources
- Funding and facilitating education and training with academic and private sector partners.
- Managing the FDA Senior Science Council, through which intra-agency working groups focus on cross-cutting scientific and technological topics
- Facilitating formal collaborative and cooperative agreements, and patenting and licensing of FDA inventions, through its Office of Technology Transfer

Extramural Programs Advancing Regulatory Science

Centers of Excellence in Regulatory Science and Innovation (CERSI)

Advances regulatory science through collaboration with academic centers conducting focused research, education and training exchange.

- *UMD CERSI*
- *Georgetown University*
- *UCSF/Stanford*
- *Johns Hopkins*
- *Yale/Mayo Clinic*

Broad Agency Announcement (BAA)

A contract mechanism for all of FDA to gain access to new solutions to regulatory science problems. Proposals address the nine topic areas of the Advancing Regulatory Science Strategic Plan. Examples include:

- *OpenFDA*
- *Social Media Data Mining*
- *Musculoskeletal Atlas Project*
- *Organs on Chips*

Intramural Programs

- *Chief Scientist's Challenge Grants*
- *Nanotechnology Grants CORES*
- *Facilitates the Office of Counterterrorism and Emerging Threats (OCET) Medical Countermeasures initiative (MCMi) Challenge Grants*
- *Facilitates the Office of Minority Health (OMH) Challenge Grants*
- *Facilitates the Office of Women's Health (OWH) Intramural Scientific Research Funding*

Science Resource Coordination

Supports cross-center, interagency and national/international collaboration, coordination, training, and research.

- *FDA Senior Science Council*
- *FDA Scientific Working Groups*
- *Interagency Networking*
- *Core Scientific Infrastructure*

Technology Transfer

Assists FDA in development and transfer of FDA inventions to the commercial marketplace through collaborations and partnerships. Implements federal technology transfer mandates for FDA, which include:

- *Cooperative Research and Development Agreements (CRADA's)*
- *Research Collaboration Agreements*
- *Material Transfer Agreements*
- *Inventions and Patents*
- *Confidential Information*
- *Intellectual Property Guidance*

Office of Regulatory Science and Innovation
Carol D. Linden, PhD, Director

CERSI Mission & Goals

To facilitate cooperative relationships and build strategic alliances among FDA and leading academic institutions to provide the Agency ready access to research capabilities, training and education and a platform for communication and dialogue with stakeholders in support of FDA's regulatory science needs, scientific workforce development, and its regulatory mission that includes speeding innovations to advance public health.

CERSI Framework

Research	• Research Collaborations with FDA • Pilot Projects
Education/Training	• Certificate & Masters Programs • Mini-Courses • Fellowships
Collaborative Interactions	• Workshops & Lectures • Visiting Scientists Program
Administration	• Core Facilities and Program Supports

CERSIs

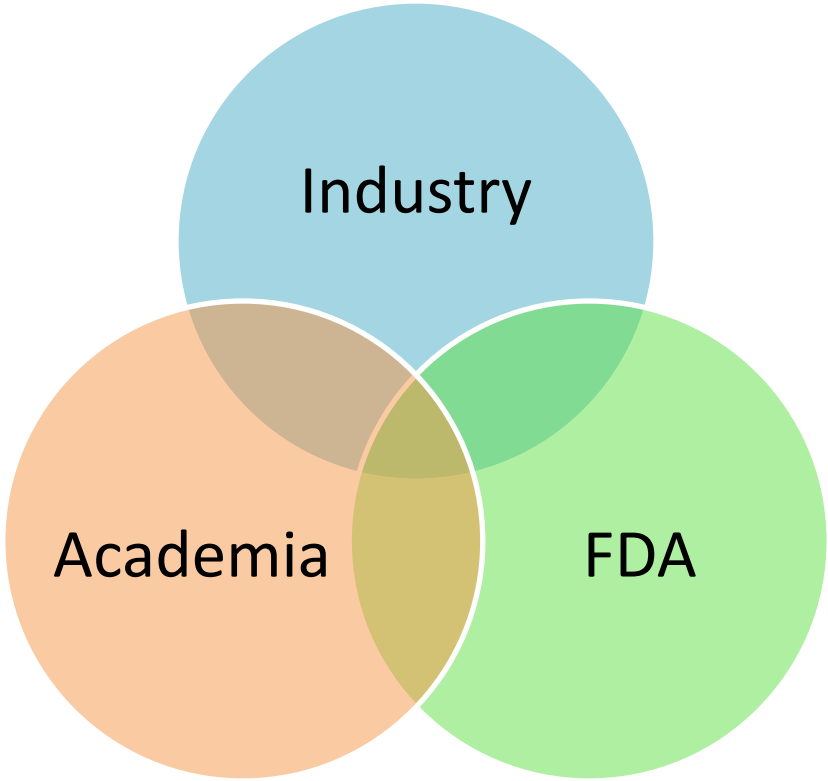
- **Georgetown University – 2011 - 2017**
- **University of Maryland – 2011 - 2018**
- **UCSF-Stanford University – 2013 - 2021**
- **Johns Hopkins University – 2013 - 2018**
- **Yale University - Mayo Clinic – 2016 - 2018**

For more information, visit CERSI web site:
https://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/ucm301667.htm

BROAD AGENCY ANNOUNCEMENT (BAA)

FDA "Rolling" Broad Agency Announcement to Advance Regulatory Science and Innovation

Encourage participation of science and technology based firms and educational institutions in meeting FDA goals for regulatory science research in nine priority areas.



Regulatory Science Strategic Plan:
http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/ucm267719.htm#

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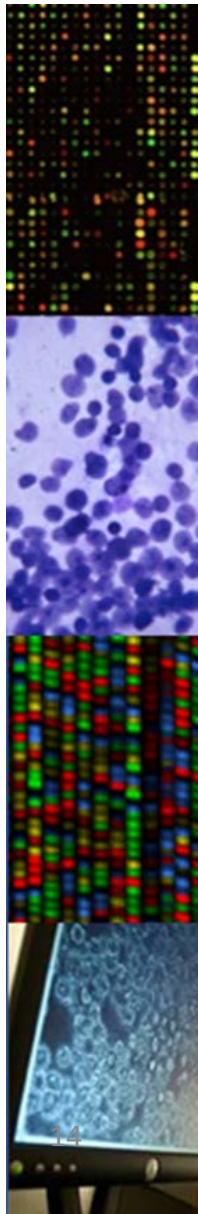
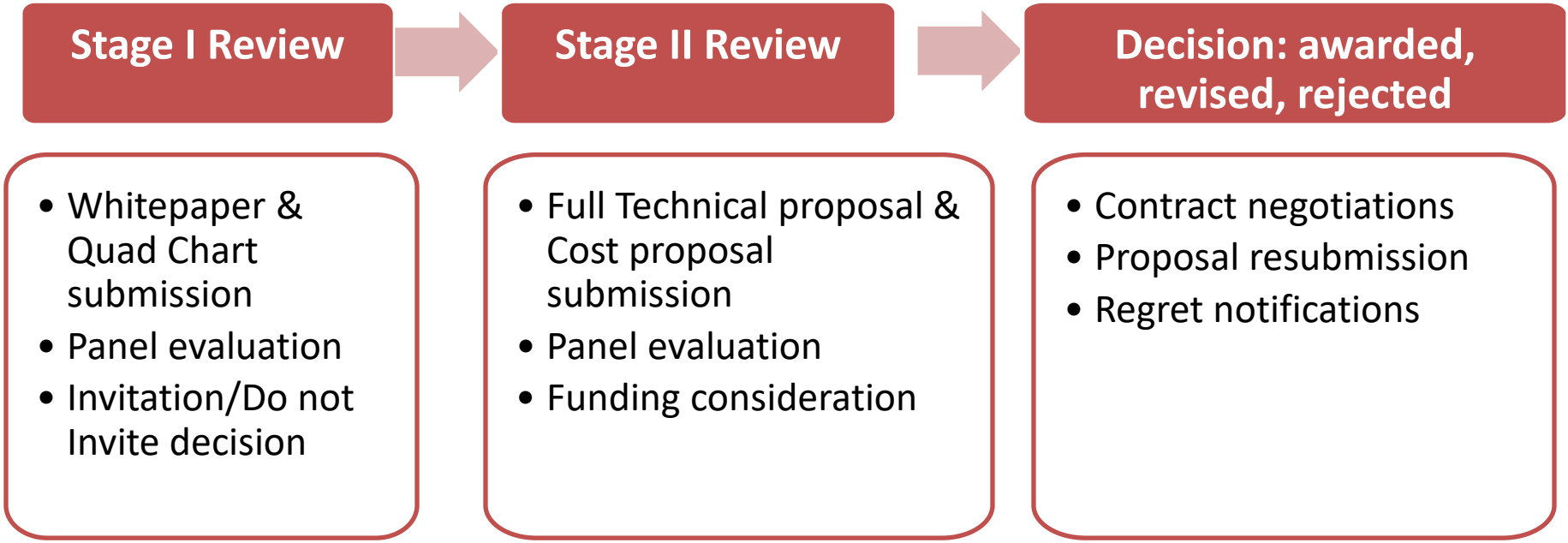
FDA Regulatory Science Research Priorities

1. Modernize Toxicology to Enhance Product Safety
2. Stimulate Innovation in Clinical Evaluation & Personalized Medicine to Improve Product Development and Patient Outcomes
3. Support New Approaches to Improve Product Manufacturing and Quality
4. Ensure FDA Readiness to Evaluate Emerging Technologies
5. Harness Diverse Data through Information Sciences to Improve Health Outcomes
6. Implement a New Prevention-Focused Food Safety System to Protect Public Health
7. Facilitate Development of Medical Countermeasures to Protect Against Threats to U.S. and Global Health and Security
8. Strengthening Social and Behavioral Science at FDA by Enhancing Audience Understanding
9. Strengthening the Global Product Safety Net



BAA Review Process

- **Current Announcement on FedBizOpps.gov**
  - FDABAA-17-00123N
  - Updated every year; open until further notice



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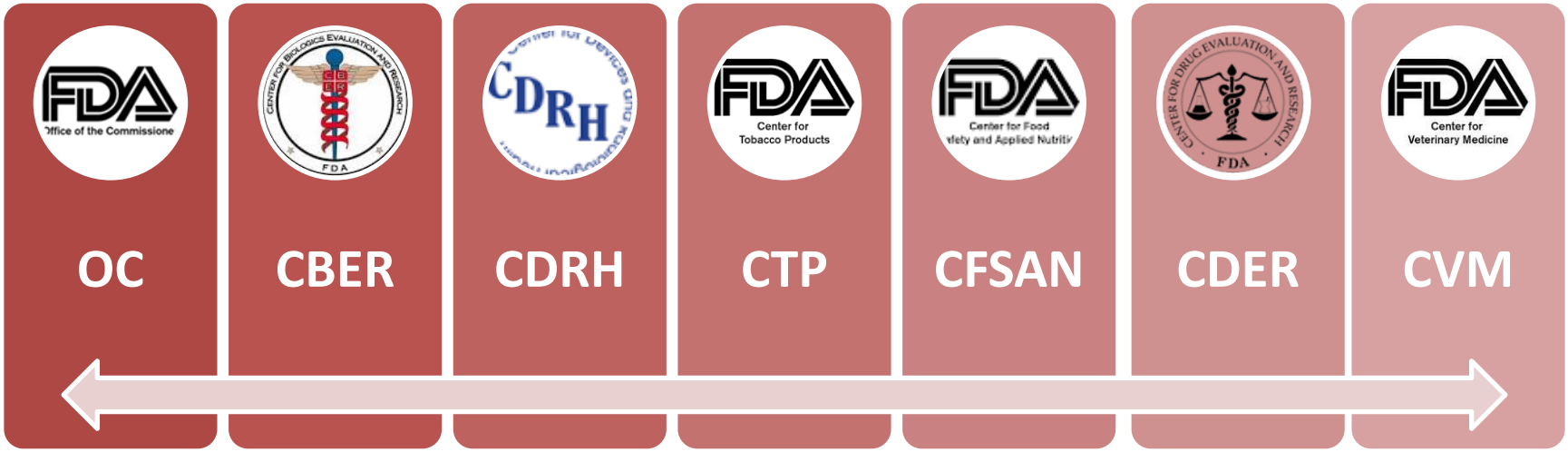
BAA Review Panel



- Technical SMEs/reviewers are recruited across the FDA
- Based on the center priority and mission
- 2- 3 technical reviewers per proposal
- Access to 100 + pool of expert SMEs

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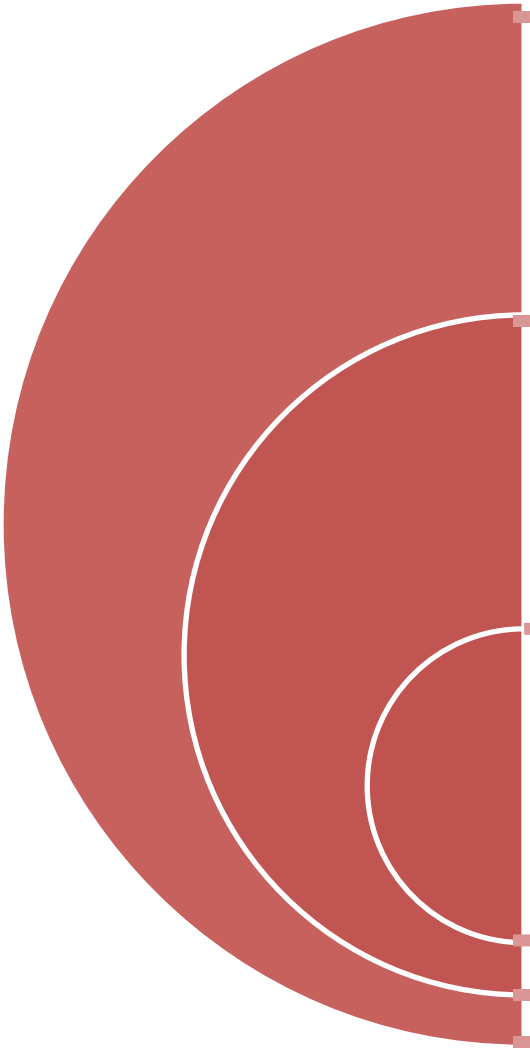
Evaluation Criteria



Scientific and Technical Merit

- Degree of innovation
- Soundness, feasibility and validity of the proposed plans
- Understanding of the scope and technical effort

Importance to Agency programs

- Inform regulatory decision-making
- Addresses an important need in the areas of regulatory science

Capabilities & Experience

- Qualification of the team
- Research management
- Partnership

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Senior Science Council (SSC)

An Agency forum comprising FDA scientific leadership from Centers and Office of the Commissioner. SSC provides advice and guidance to Agency and center leadership on cross-cutting regulatory science issues, including planning, reporting, programs, policies, and communication.

Responsibilities

- Enhance communication and coordination on cross-cutting regulatory science activities at FDA.
- Provide input on regulatory science strategic planning and reporting.
- Provide input on the development of intramural and extramural regulatory science grants funded through the OCS, and facilitate review of resulting proposals.
- Draft or provide input on, Agency-wide regulatory science policies for consideration by FDA leadership.
- Provide input on cross-cutting regulatory science activities managed within the Office of the Chief Scientist, such as professional development, training, and scientific integrity.

Working Groups under the SSC

OCS and the Office of Regulatory Science and Innovation (ORSI) provide financial and managerial support to help working groups implement their activities (e.g., workshops, lecture series, and specific training for FDA staff).

- The **Office of the Chief Scientist** (OCS) supports scientific working groups at FDA with the goals of:
- communicating and disseminating information among product centers,
- coordinating scientific projects, and
- collaborating and exchanging resources and expertise.
- These working groups serve FDA senior management as a scientific information resource, for example, to identify experts at FDA who can help with regulatory decision-making.
- A well-trained, well-connected, and well-supported scientific workforce is essential to meet FDA's challenges of integrating emerging sciences and technologies into the Agency's research and review processes.

The current working groups that are part of this initiative are:

Current FDA Scientific Working Groups *

Scientific Working Group	Chair	Co-Chair
Additive Manufacturing Working Group	James Coburn (CDRH)	Matthew Diprima (CDRH)
Animal Welfare Subcommittee	John Dennis (CBER)	
Biomarker Working Group	Christopher Leptak (CDER)	Raj Puri (CBER)
Emerging Sciences Working Group	Donna Mendrick(NCTR)	
Genetics and Genomics Team	Aaron Schetter (CDRH)	Rachel Goehe(CDRH)
Genomic Working Group	Eric Donaldson (CDER)	Khaled Bouri (OC/OCS/ORSI)
Microbiome Working Group	Jennifer Patro (CDER)	Paul Carlson (CBER)
Modeling and Simulation Working Group	Tina Morrison(CDRH)	
Nanotechnology Task Force (NTF)	Anil Patri (NCTR)	
Social and Behavioral Sciences Working Group	Lee Zwanziger (OC)	
Standards Committee	Hany Demian (OC)	
Statistical Association	Yun Wang (CDER)	
Toxicology Working Group	Tracy Chen(OCS)	

*** Please see attached document for list of workshops and lecture series organized by the working group in 2017**

CERSI Workshops*

Date	Title	Hosts
June 15, 2017	Use of Natural Language Processing to Extract Information from Clinical Text	FDA, UCSF-Stanford CERSI & San Francisco State University
November 18, 2016	Substitutability of Generic Drugs: Perceptions and Reality	FDA & Johns Hopkins CERSI
November 7-9, 2016	Clinical Investigator Training Course	FDA & University Maryland CERSI
September 23, 2016	Pediatric Master Protocols	FDA & University of Maryland CERSI

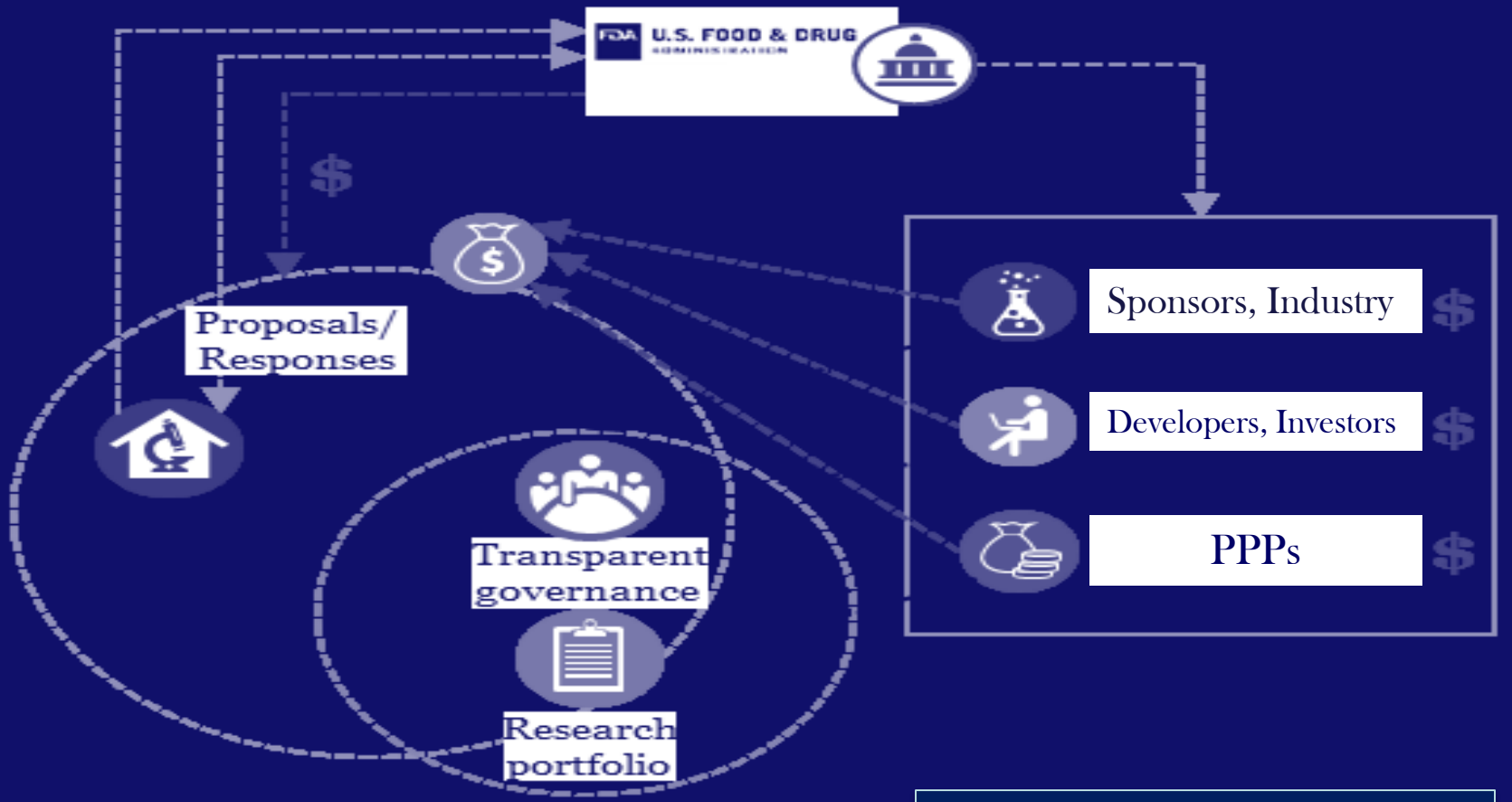
* See www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/ucm493022.htm for more information

CERSI Lectures*

Date	Title	Host
February 9, 2017	Neuroprosthetics and Brain Machine Interface: Innovation to Implementation	Johns Hopkins CERSI
March 14, 2017	Ensuring Accuracy in Clinical Trial Publications	University of Maryland CERSI
April 13, 2017	Zika Virus Vaccines & Therapeutics	University of Maryland CERSI
May 11, 2017	Reproducibility in Medicine and Science	UCSF-Stanford CERSI
June 6, 2017	Sex Differences& Vascular Disease: Presentation, Diagnostics & Treatment	Georgetown CERSI

* See www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/ucm539576.htm for more information

Emerging Model: Collaborative platform-based funding and research governance



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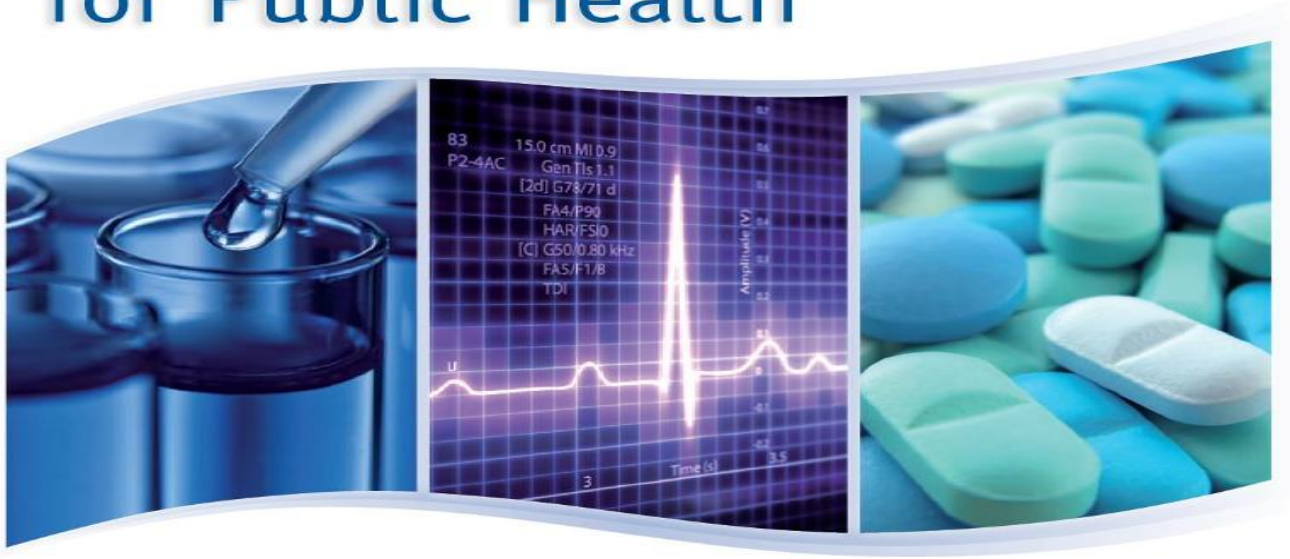
U.S. FOOD & DRUG ADMINISTRATION

Advancing Regulatory Science for Public Health



THANK YOU