Center for Biologics
Evaluation and Research
FDA

Overview

Carolyn A. Wilson, Ph.D.
Associate Director for Research
CBER Regulates Complex Products

- Cell & Gene Therapies
- Blood, Blood Components and Derivatives
- Xenotransplantation Products
- Vaccines: Preventive & Therapeutic
- Tissues
- Live Biotherapeutics
- Related Devices
- Allergenic Products
Using Science and Regulation to Advance Product Development

- Post-market Surveillance
- Licensed Product
- Improved Data – Benefit/Risk
- Public Health
- Novel Product
- Regulatory Challenge
- Regulatory Science
- Discovery
- New Tools
- Regulatory Policy/Decision

www.fda.gov
CBER researcher = “Researcher-Reviewer”

INTEGRATION OF RESEARCH AND REVIEW ENSURES

RELEVANCE, EXPERTISE, TIMELINESS, AND USABILITY
Scientific Expertise

- Applied technologies: NMR, mass spec, flow cytometry, microarray, high throughput sequencing and related bioinformatics/IT
- Microbiology: parasitology, bacteriology, virology, microbiome
- Immunology
- Biochemistry and molecular biology
- Cell, developmental biology and tissue engineering
- Epidemiology, meta-analyses of large healthcare databases
- Biostatistics
- Bioinformatics
CBER Advances Regulatory Science through External Collaborations

Data from FY16 CBER Research Reporting Database
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What’s New?

• CBER Peer Mentoring Group
• Move to White Oak Campus
• New research management processes
  • New governance bodies
  • CBER Regulatory Science and Research Goals
• Research impact framework
Established two new governance bodies that oversee research priorities and budget and resource planning

- **New governance bodies** will manage overall budget and research across all of CBER, and are additive to / do not replace any existing structures already in place within the Offices.

**Changes will benefit CBER by:**
- Increasing overall transparency into decision-making
- Ensuring proper direction and prioritization of research needs
- Allowing earlier and more holistic budget planning

**Existing governance bodies**

- **Center Director and Deputy Director**
- **Resource Committee**
  - Chair: Director, OM
  - Manages annual budget and resource planning, including allocation to Offices
- **Regulatory Science Council**
  - Chair: Center ADR
  - Develops Center-level goals, guides Office-level objectives, and provides oversight across all of CBER’s research activities

**Coordinating Committees**
• Advance the scientific basis for regulation of biologics, human tissues and blood to enhance safety, effectiveness, quality and consistency through development and evaluation of new concepts, methods, models, and reagents.

• Develop and assess nonclinical models and methods with improved predictive value, and, as feasible, reduce, refine, or replace the use of animals, for evaluation of safety and effectiveness of CBER-regulated products.

• Improve clinical evaluation related to CBER-regulated products through the use of new biomarkers, large scientific and healthcare datasets, and innovative design and analysis of clinical studies by applying new statistical, epidemiological, and mathematical modeling approaches, and considering patient input to inform benefit-risk assessment of general and special populations.

• Prepare for future regulatory and public health challenges through investments in emerging science and technology, and develop and sustain varied scientific expertise.
Annual Review of PI Research, starting FY17

• Peer review of 25% of research programs and any new project proposals
• Supervisory, Division, Office review of Annual Research Report (details next)
• Portfolio review by Regulatory Science Council (Entire portfolio for FY17 with one Office/year subsequent)
• All use Research Impact Framework
## Research Impact Framework

### Portfolio and Project Level Review

#### Key elements

<table>
<thead>
<tr>
<th>Mission relevance and potential for impact</th>
<th>Applies to …</th>
<th>Primary use</th>
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<tbody>
<tr>
<td>Alignment with major Center- or Office-wide strategic initiatives and priorities</td>
<td><strong>Portfolio</strong> and individual projects</td>
<td>Consistent approach for portfolio management and communicating about CBER research to external stakeholders</td>
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<tr>
<td>Building a world class review capability for current or anticipated pipeline</td>
<td><strong>Portfolio</strong></td>
<td>Annual Reporting and oversight of CBER research projects</td>
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<tr>
<td>Maintenance of an agile set of internal capabilities for addressing unexpected, urgent public health needs</td>
<td><strong>Portfolio</strong></td>
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<tr>
<td>Using CBER’s unique perspective to address scientific gaps and questions to enhance our ability to fulfill our regulatory mission</td>
<td>Individual <strong>projects</strong></td>
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<tr>
<td>Scientific merit</td>
<td>Individual <strong>projects</strong></td>
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<tr>
<td>PI’s historical productivity</td>
<td>Individual <strong>projects</strong></td>
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PI Submits to Research Reporting Database: Program-level Information

• Overview
• Expertise
• Relevance to CBER Goals and FDA Priorities
• Staffing and Collaborators
• Lab Space Assigned
• Major Equipment
• Cold Storage Units (important for inventory management of hazardous biological agents and toxins)
• Publications, presentations, other output
PI Submits to Research Reporting Database: Project-level Information

• Each PROJECT:
  • Relevant Office Goal/Objective
  • Executive Summary and Background
  • Review Capability supported by research
  • Expected outcome and impact
  • 1-3 Specific Aims: Experimental Approach, Progress, Plans, Anticipated Results,
  • Admin: personnel, budget request, relevant IBC, RHISC, ACUC, Data Management Plan
Cyclic Peer Review of Every PI Every 4 Years

External – Site Visits
peer review by scientific experts

Internal – Promotion, Conversion, Evaluation Committee
Site Visit Report

- Draft report is distributed to full Advisory Committee
- Final report is approved by full Advisory Committee
- Final report used in many ways:
  - Internal peer review of research/PI by Promotion, Conversion, Evaluation Committee (PCE) for personnel actions
  - By PIs for improving research program
  - By management, resource allocation decisions may be impacted by report (pending resource availability)

- Outcomes of Advisory Committee Meeting
  - Accept report
  - Amend report
  - Reject report and send back to Site Visit Team
Thank you!

To the Site Visit reviewers and Advisory Committee

Your input improves CBER’s research programs

External review is critical to fulfilling our regulatory mission!