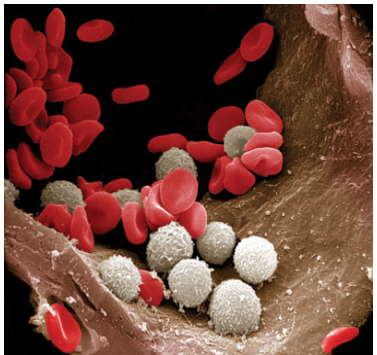
U.S. FOOD & DRUG ADMINISTRATION

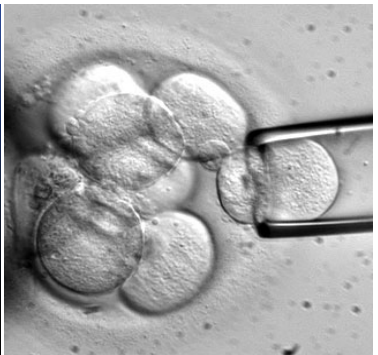
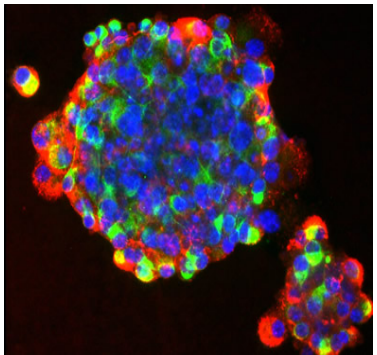
Center for Biologics Evaluation and Research FDA

Overview

Carolyn A. Wilson, Ph.D.

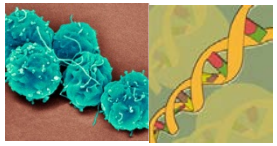
Associate Director for Research

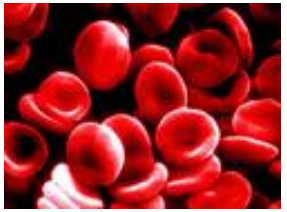



CBER Regulates Complex Products

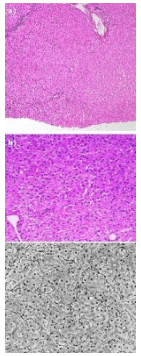
Cell & Gene Therapies



Blood, Blood Components and Derivatives

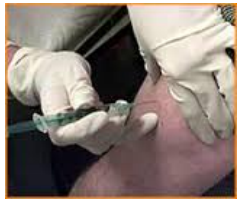


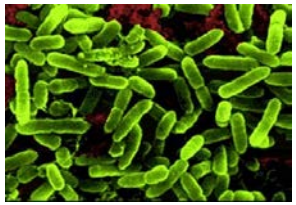
Xenotransplantation Products



Tissues

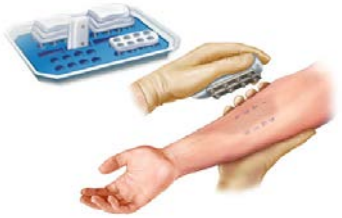
Vaccines: Preventive & Therapeutic





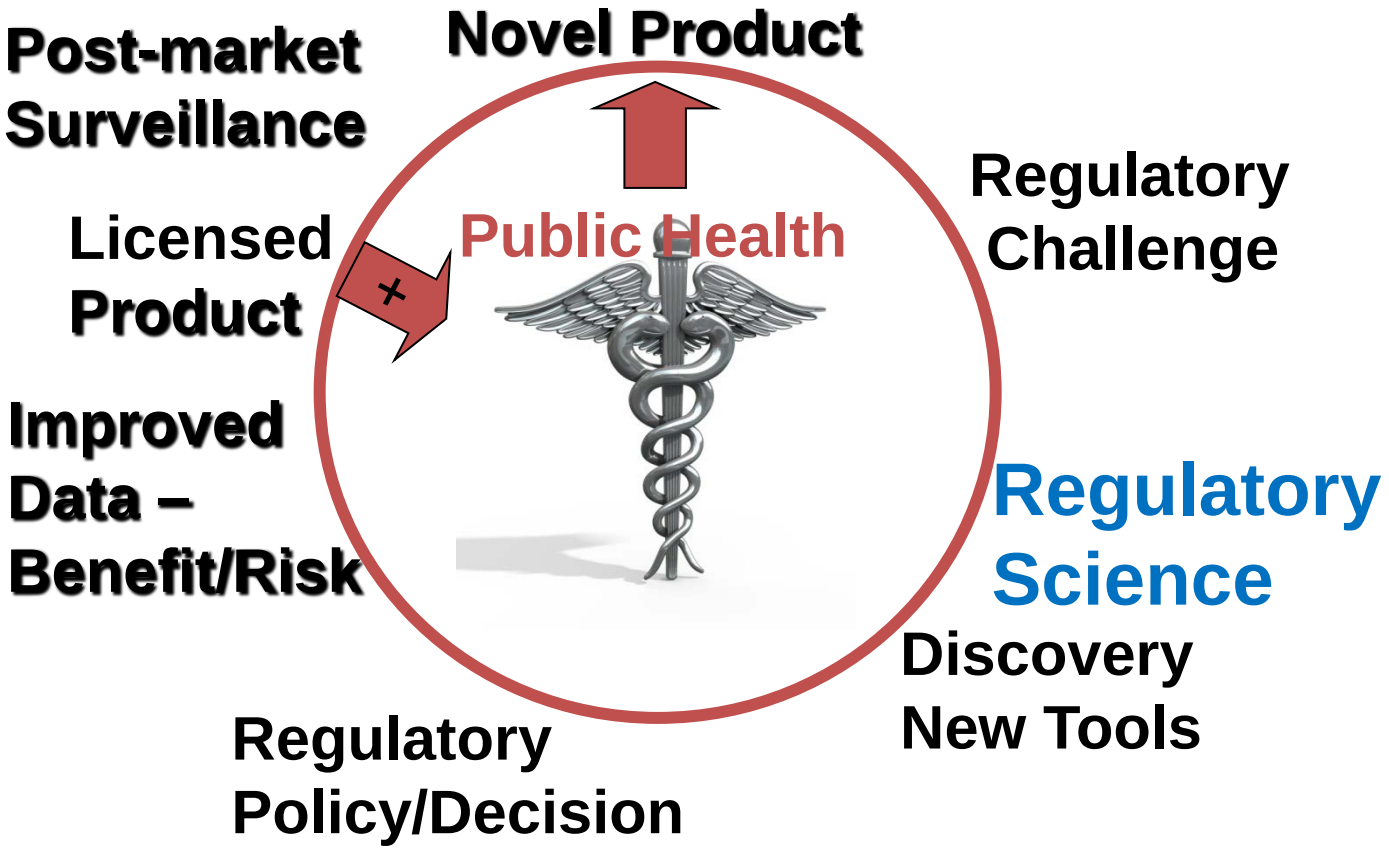
Live Biotherapeutics

Related Devices



Allergenic Products

Using Science and Regulation to Advance Product Development



CBER researcher =“Researcher-Reviewer”

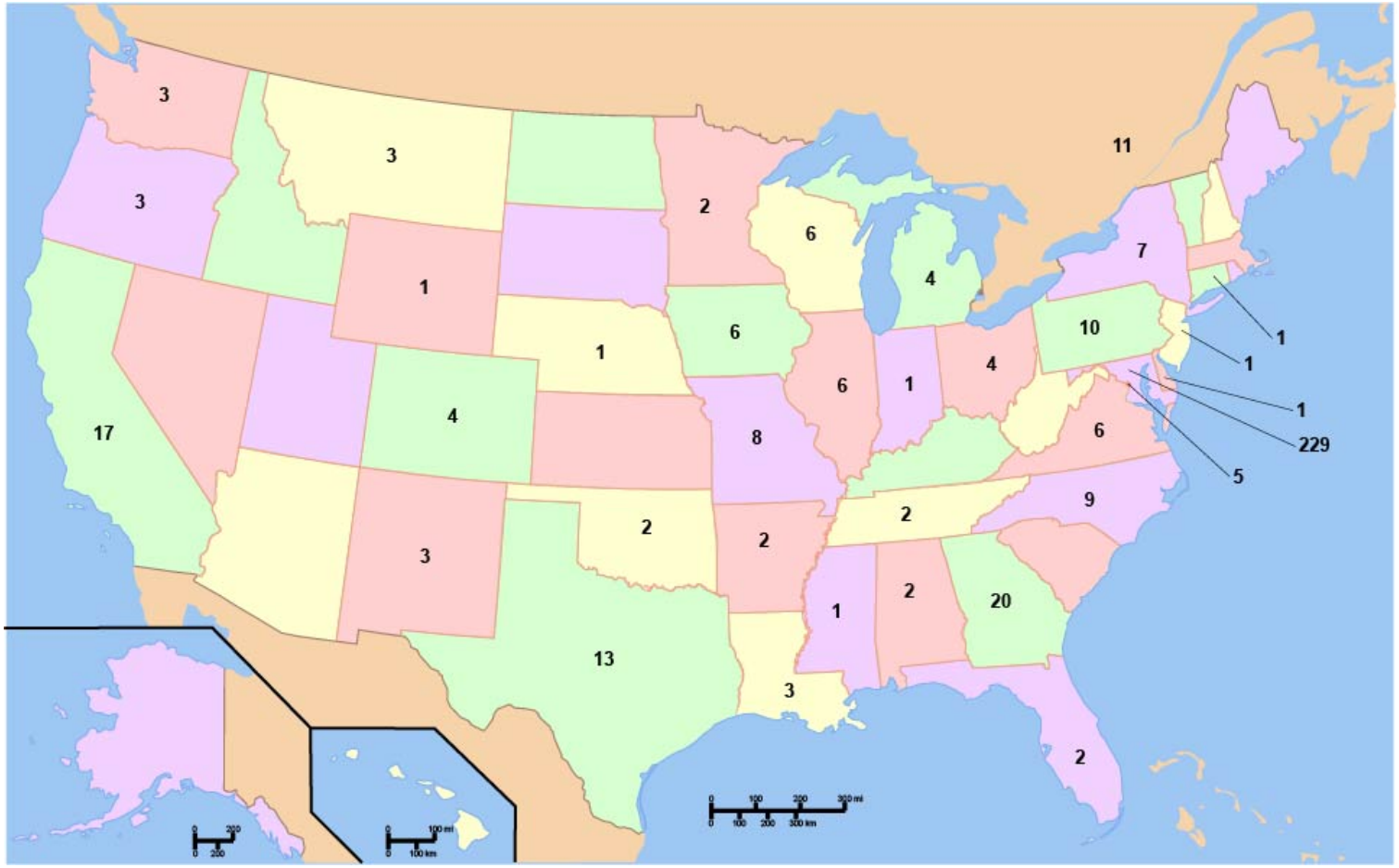
INTEGRATION OF RESEARCH AND REVIEW ENSURES

RELEVANCE, EXPERTISE, TIMELINESS, AND USABILITY

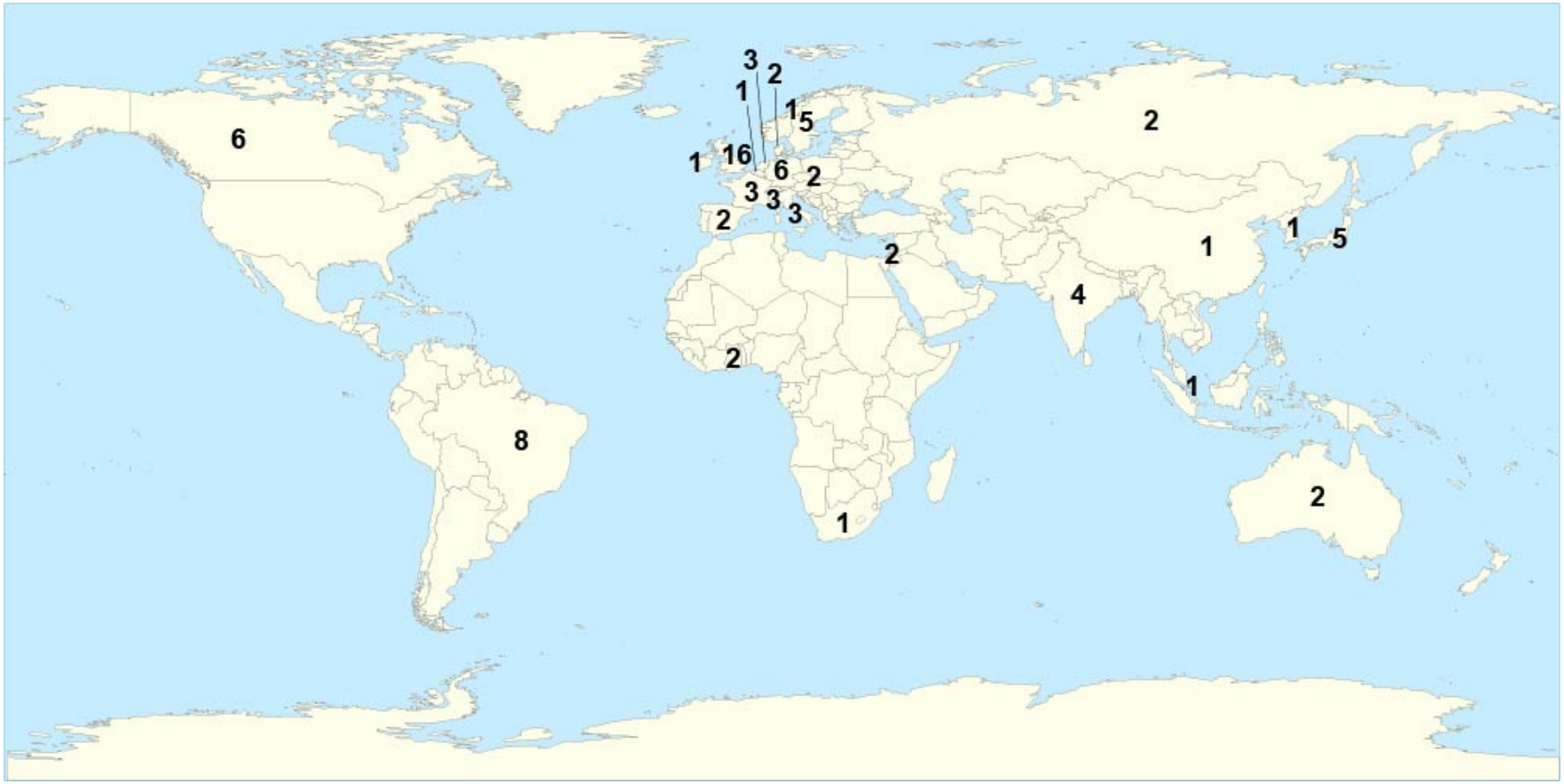
Scientific Expertise

- 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

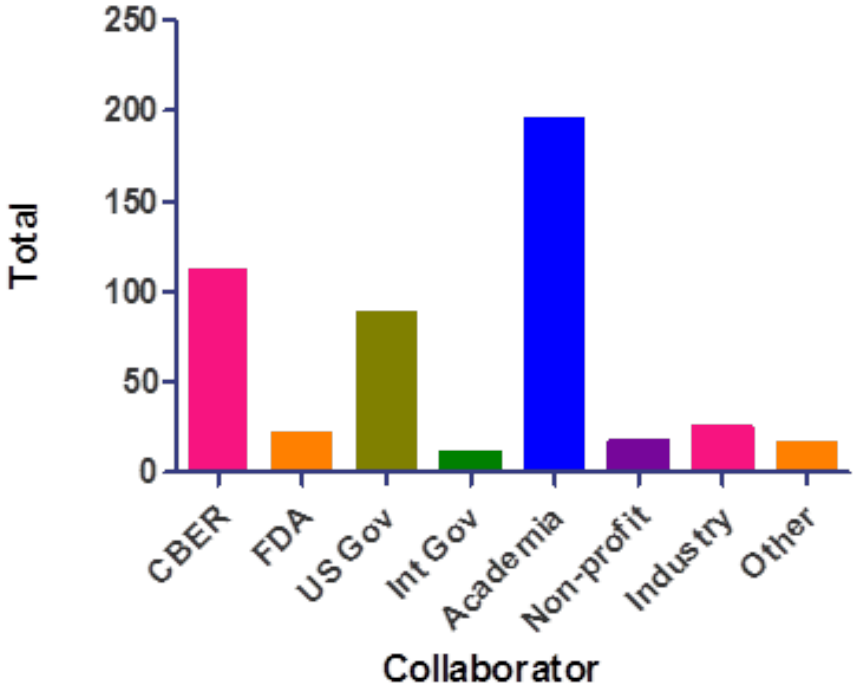


CBER Advances Regulatory Science through External Collaborations



Data from FY16 CBER Research Reporting Database

CBER Advances Regulatory Science through External Collaborations

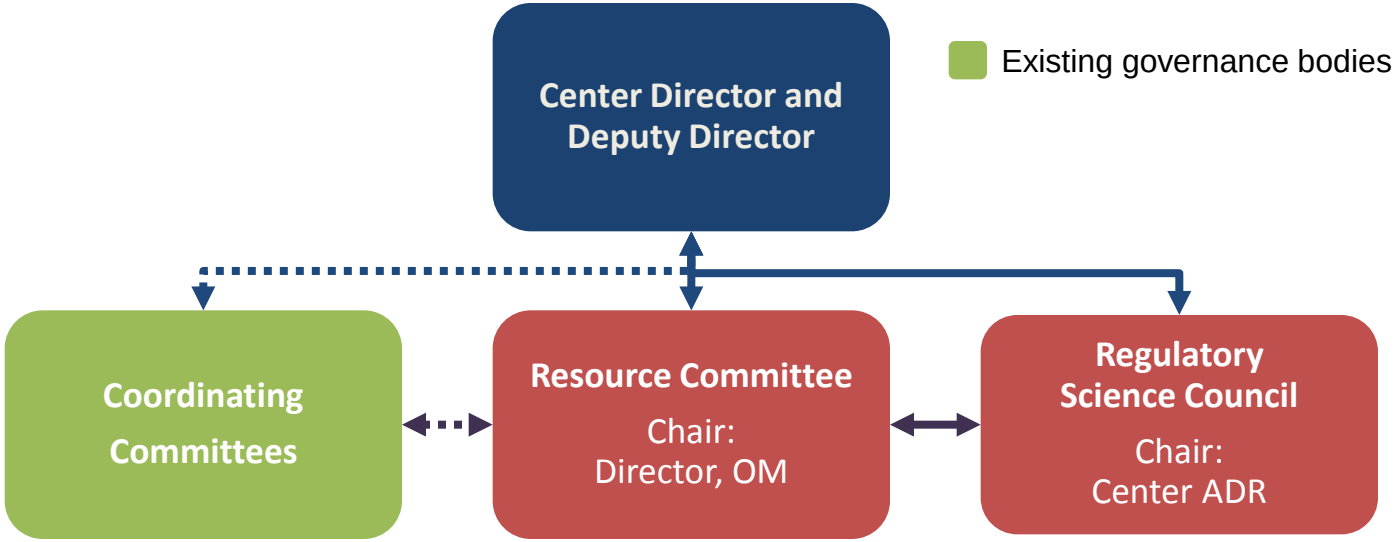


Data from FY16 CBER Research Reporting Database

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



- Manages annual budget and resource planning, including allocation to Offices

- Develops Center-level goals, guides Office-level objectives, and provides oversight across all of CBER's research activities

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

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

2016 CBER Regulatory Science and Research Goals

- 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	Applies to …	Primary use
Mission relevance and potential for impact	Alignment with **major Center- or Office-wide strategic initiatives** and priorities	**Portfolio** and individual projects	Consistent approach for **portfolio management** and communicating about CBER research to external stakeholders
	Building a world class review capability for current or anticipated pipeline	**Portfolio**	
	Maintenance of an **agile set of internal capabilities** for addressing unexpected, urgent public health needs	**Portfolio**	
Position to make a unique contribution	Using CBER's **unique perspective to address scientific gaps and questions** to enhance our ability to fulfill our regulatory mission	Individual **projects**	Annual Reporting and oversight of CBER research **projects**
	Scientific merit	Individual **projects**	
	PI's historical **productivity**	Individual **projects**	

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!