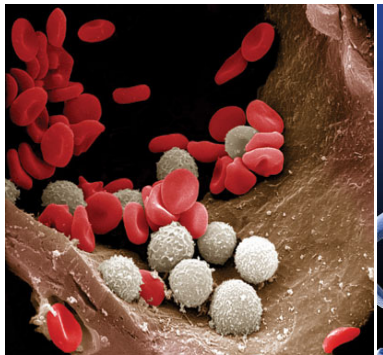
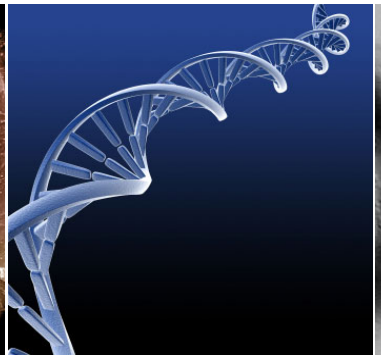
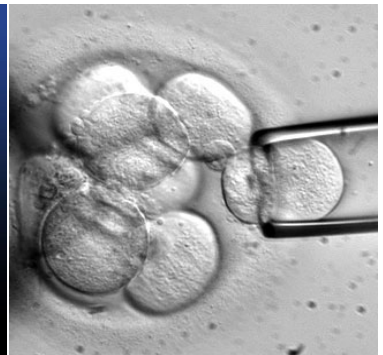
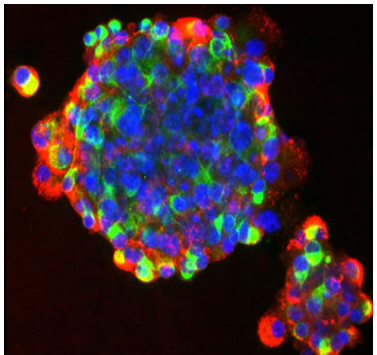
U.S. FOOD & DRUG ADMINISTRATION

Center for Biologics Evaluation and Research FDA

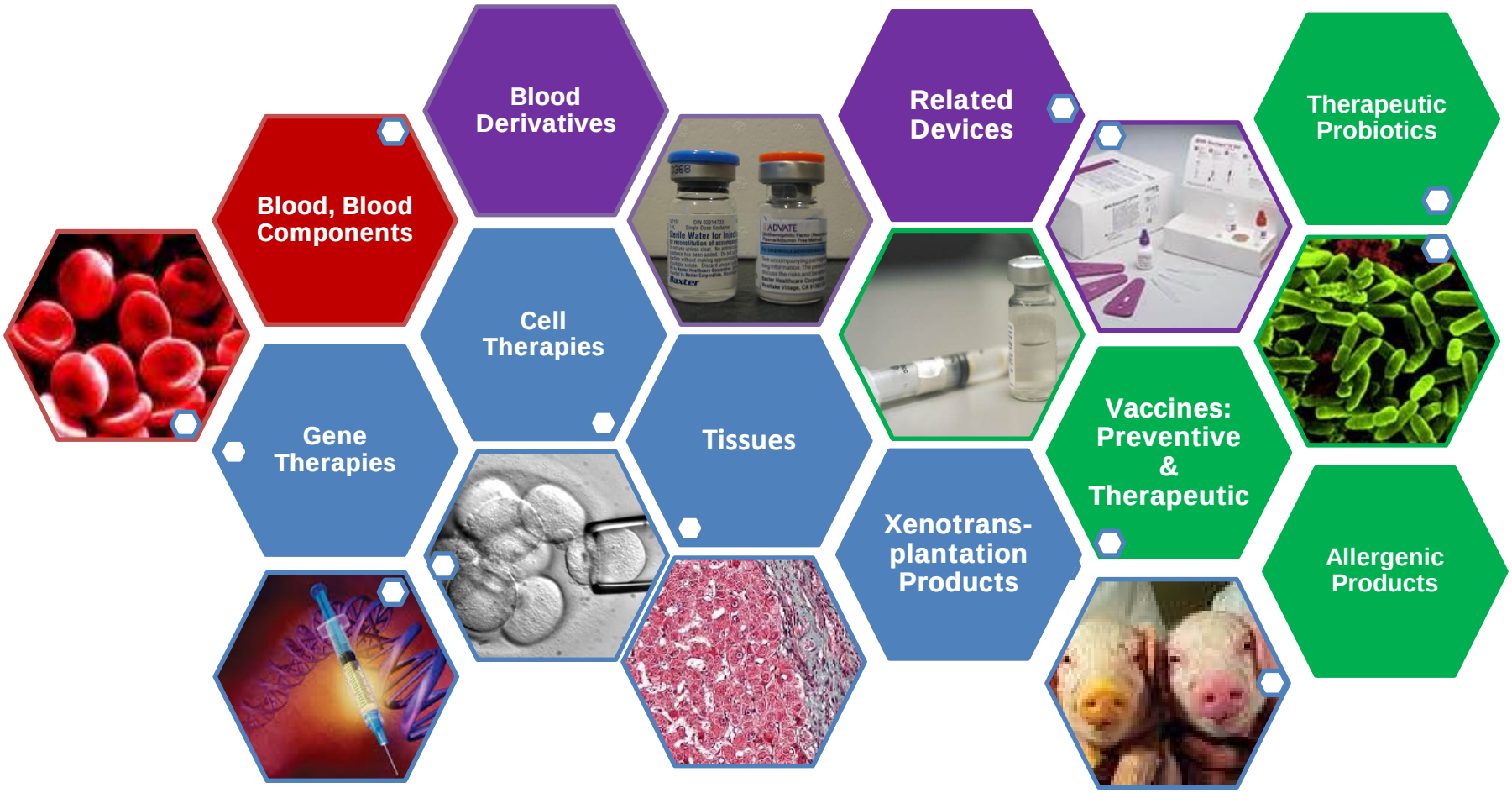
Overview

Carolyn A. Wilson, Ph.D.

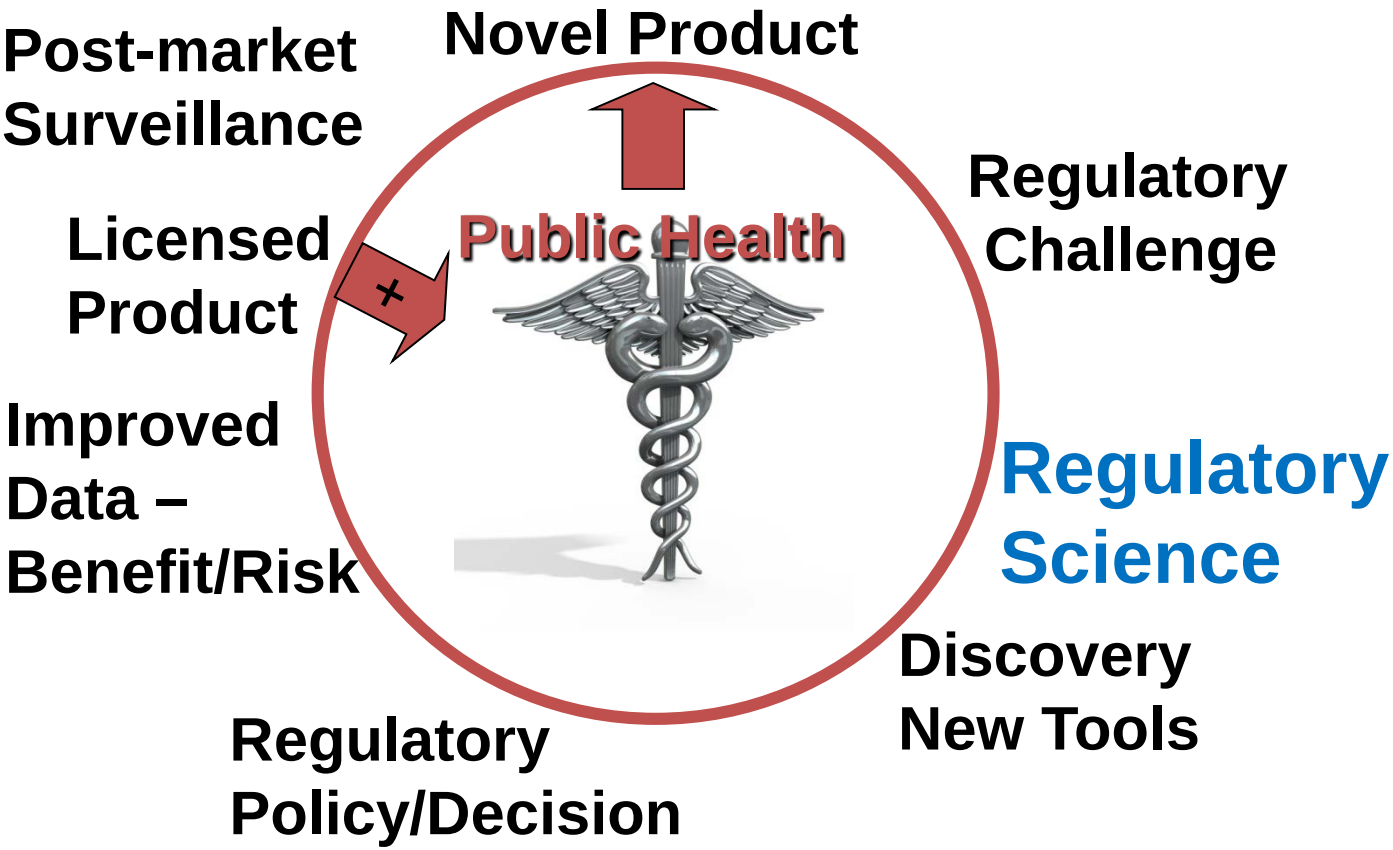
Associate Director for Research

CBER Regulates Complex Products



Using Science and Regulation to Advance Product Development



Collaborative Approach to the Regulation of Biologics

- Review of Data Submitted to FDA
- Active Research
- Surveillance
- Internal CBER Discussion
- External Experts

CBER researcher =“Researcher-Reviewer”

The integration of research and review

- **ensures relevance, expertise, timeliness, and usability**
- **fosters rational policy and decisions based on sound science, law and public health impact**

Benefits of CBER Research Program

- Prepare for future innovative products and public health challenges
- Develop tools and data that are available to all stakeholders and support development of product classes
- Recruit and maintain highly trained scientists with necessary expertise to review regulatory submissions
- Fills gaps to inform policy development and regulatory decision-making

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, tissue engineering/microphysiologic systems
- Epidemiology, meta-analyses of large healthcare databases
- Biostatistics
- Bioinformatics

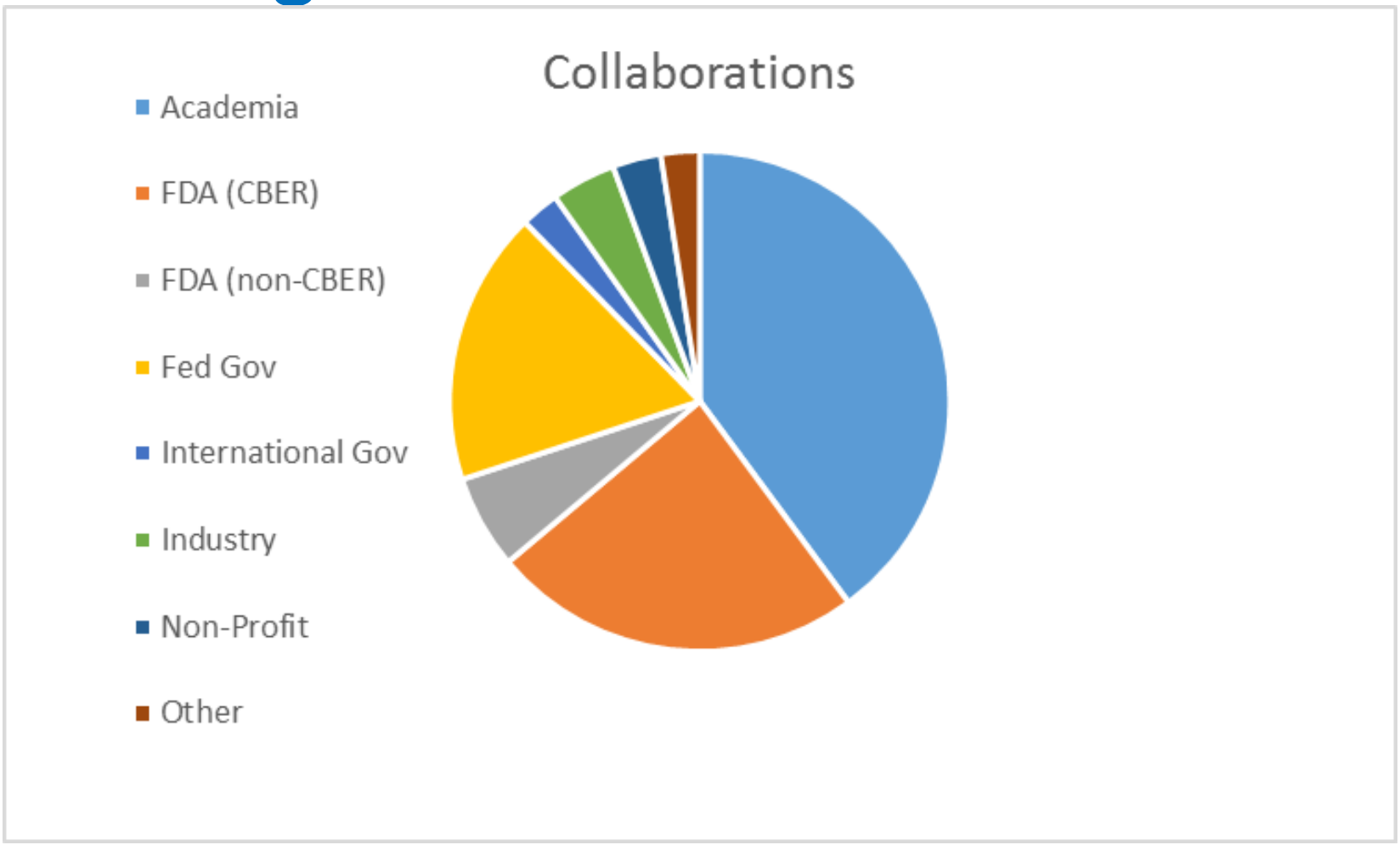
White Oak Lab Facility

- Core Facilities:
  - Flow cytometry
  - Confocal and electron microscopy
  - Biotechnology
    - ✓ Illumina HiSeq and MiSeq
    - ✓ Oligonucleotide, siRNA, PNA, and peptide synthesis
    - ✓ Peptide and DNA sequencing (ABI, capillary)
    - ✓ Taqman probe synthesis
    - ✓ HPLC; Capillary electrophoresis
    - ✓ Mass Spectrometry/Proteomics
    - ✓ Amino acid analysis
  - Bioinformatics support for NGS data analysis and storage
- State-of-the-Art Vivarium
  - Imaging facility with MRI, digital X-ray, IVIS, ultrasound, CT
  - ABSL-2 and -3; procedure rooms
  - Transgenic derivation facility

CBER Peer Mentoring Group

- CBER Peer Mentoring Group
  - Monthly meeting, open to all PI's
  - Discuss general issues, how to manage different responsibilities, eg, recruitment, budget, personnel issues, etc
  - Informally mentored by senior PI volunteers

CBER Advances Regulatory Science through External Collaborations



Data from FY17 CBER Research Reporting Database

Research Management Processes

- CBER Regulatory Science/Research Goals
- Research impact framework
- Evaluation of research program
  - Regulatory Science Council
  - Internal and External Peer Review

CBER Research Goals

Advancing the scientific basis for regulation of biologics, human tissues and blood by:

Goal 1 Developing and evaluating technology, reagents, and standards to inform and improve chemistry, manufacturing, and controls (CMC).

Goal 2 Developing and assessing nonclinical models and methods predictive of clinical performance with respect to toxicity and effectiveness.

Goal 3 Improving clinical evaluation pre- and post-licensure through use of big data, innovative designs and statistical, analytical and modeling approaches.

Goal 4 Preparing for future regulatory and public health challenges.

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	

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Internal Review Research Started FY17

- 25% of research programs and all new project proposals
  - internal peer review committee
- All programs:
  - Annual by Supervisory, Division, Office review of Annual Research Report
- Portfolio review:
  - Regulatory Science Council

All use Research Impact Framework

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

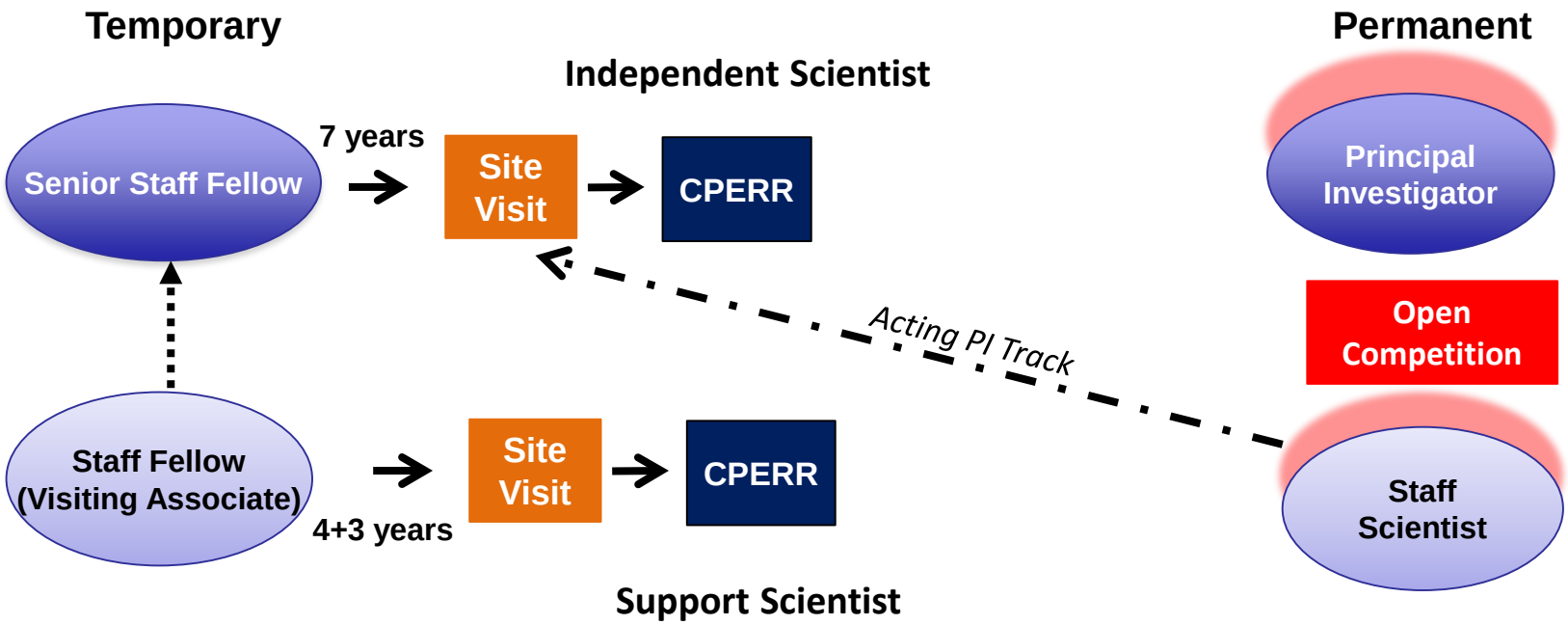
- Each PROJECT:
  - Relevant Office Goal/Objective
  - Executive Summary and Background
  - Review Capability supported by research
  - Expected outcome and impact
  - 1-3 Specific Aims:
    - Experimental Approach, Progress, Plans, Anticipated Results,
  - Admin:
    - Personnel, budget request, relevant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

Cyclic Peer Review of Every PI Every 4 Years

External – Site Visits
peer review by scientific experts

Internal – Committee for Promotion and Evaluation of Researcher-Reviewers (CPERR)

Career Pathways for Research Scientists



CPERR:
Committee for Promotion and Evaluation of Researcher-Reviewers

Site Visit Report

- Draft report is distributed to full Advisory Committee
- Outcomes of Advisory Committee Meeting
  - Accept report
  - Amend report
  - Reject report and send back to Site Visit Team
- Once 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)

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!