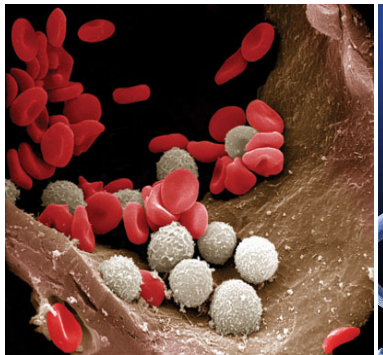
U.S. FOOD & DRUG ADMINISTRATION

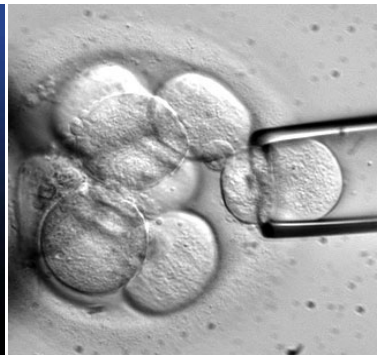
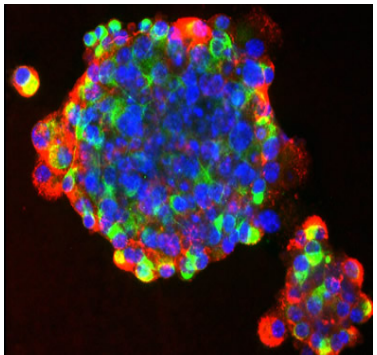
Center for Biologics
Evaluation and Research
FDA

Overview

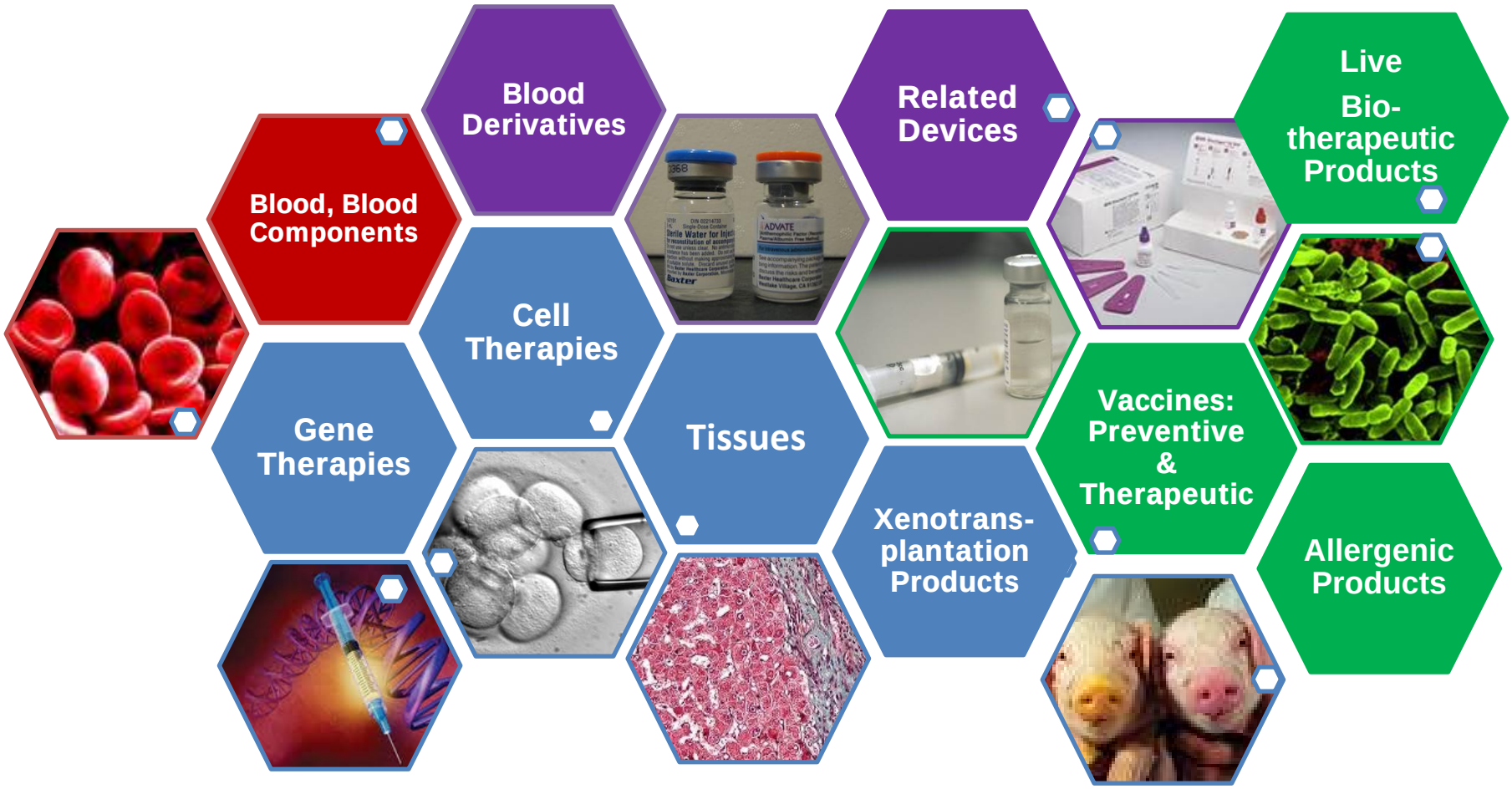
Carolyn A. Wilson, Ph.D.

Associate Director for Research

CBER Regulates Complex Products



Using Science and Regulation to Advance Product Development

Novel Product

Post-market Surveillance

Licensed Product

Improved Data – Benefit/Risk

Regulatory Policy/Decision

Regulatory Challenge

Regulatory Science

Discovery New Tools

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.

New Scientific Initiatives: Extramural and Expanded Intramural Capacity

- **Advanced Manufacturing – intramural new program**
  - Influenza vaccines
  - Hematopoietic stem cells
- **Pathogen Reduction Technologies**
  - Expand PRT to whole blood

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

White Oak Lab Facility

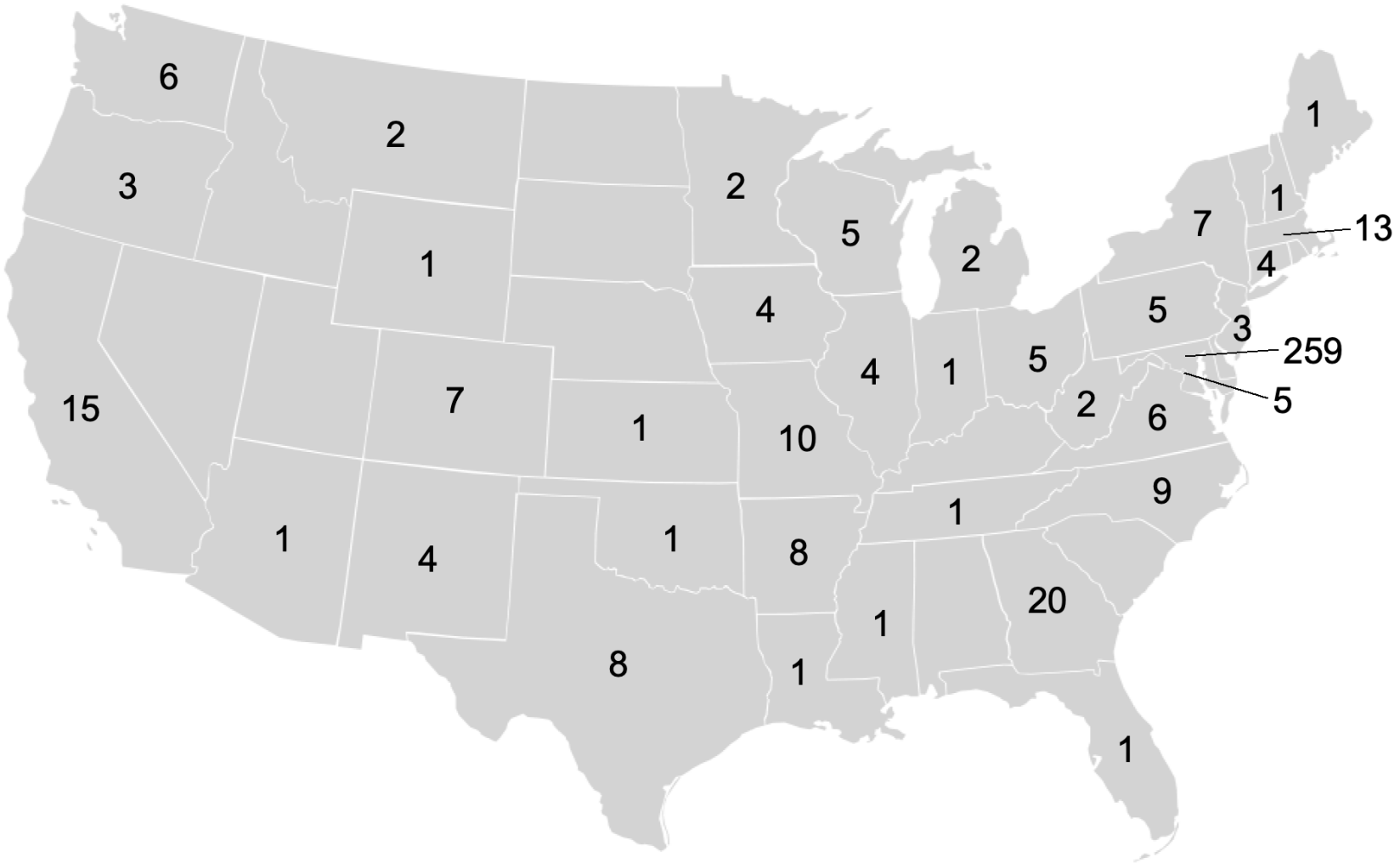
- Core Facilities:
  - Flow cytometry
  - Confocal and electron microscopy
  - Biotechnology
    - ✓ Illumina
    - ✓ Oligonucleotide, siRNA, PNA, and peptide synthesis
    - ✓ Peptide and DNA sequencing (ABI, capillary)
    - ✓ Taqman probe synthesis
    - ✓ HPLC; Capillary electrophoresis
    - ✓ Mass Spectrometry/Proteomics
    - ✓ Amino acid analysis
    - ✓ Biofermentation
  - High performance computing and 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 PI Networking and Information Group

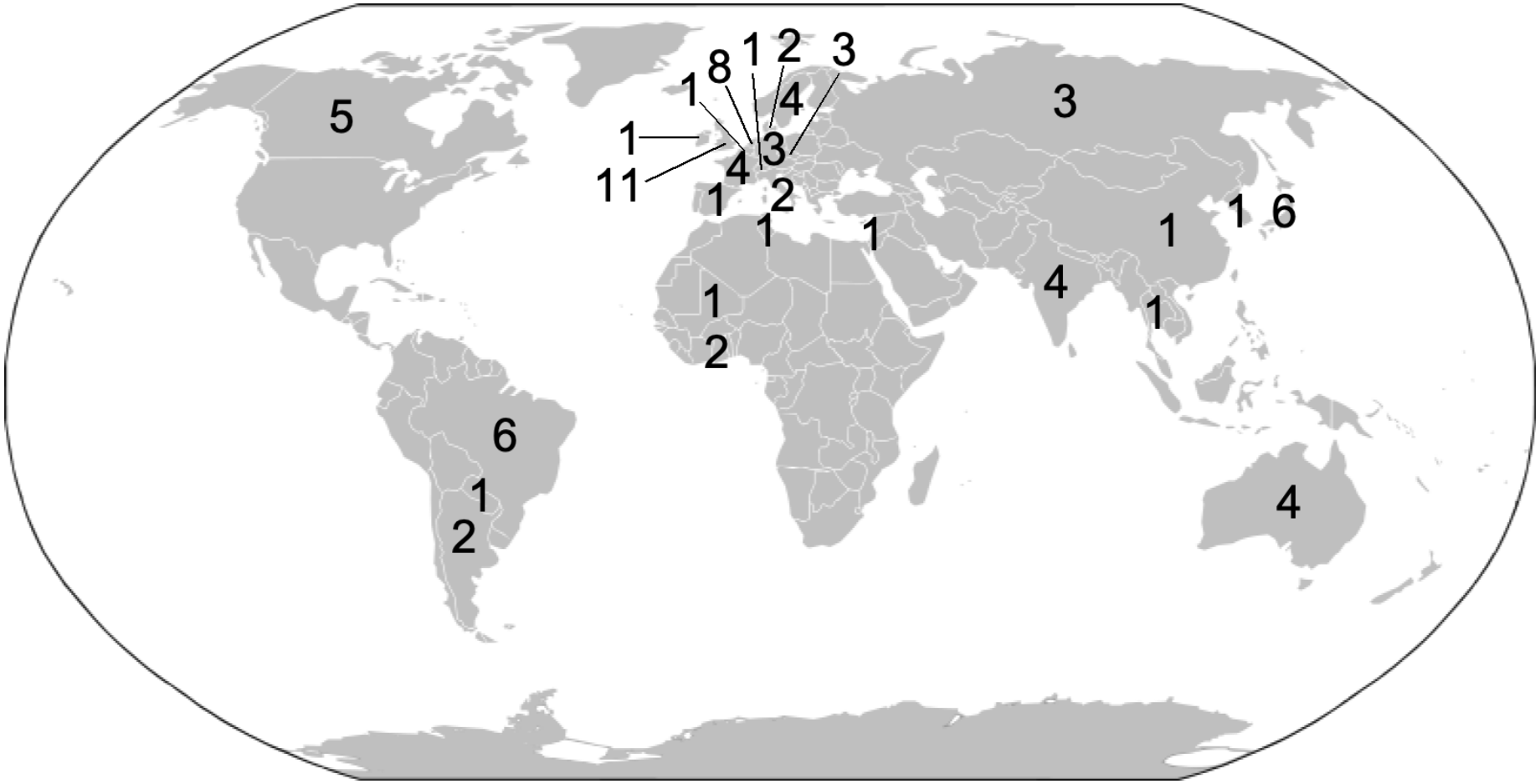
- Provides Peer Mentoring and Information Sharing
  - Monthly meeting, open to all PI's
  - Discuss general issues, how to manage different responsibilities, eg, recruitment, budget, personnel issues, etc
  - Informally mentored by senior PI volunteers

CBER Advances Regulatory Science through External Collaborations



Data from FY19 CBER Research Reporting Database

CBER Advances Regulatory Science through External Collaborations



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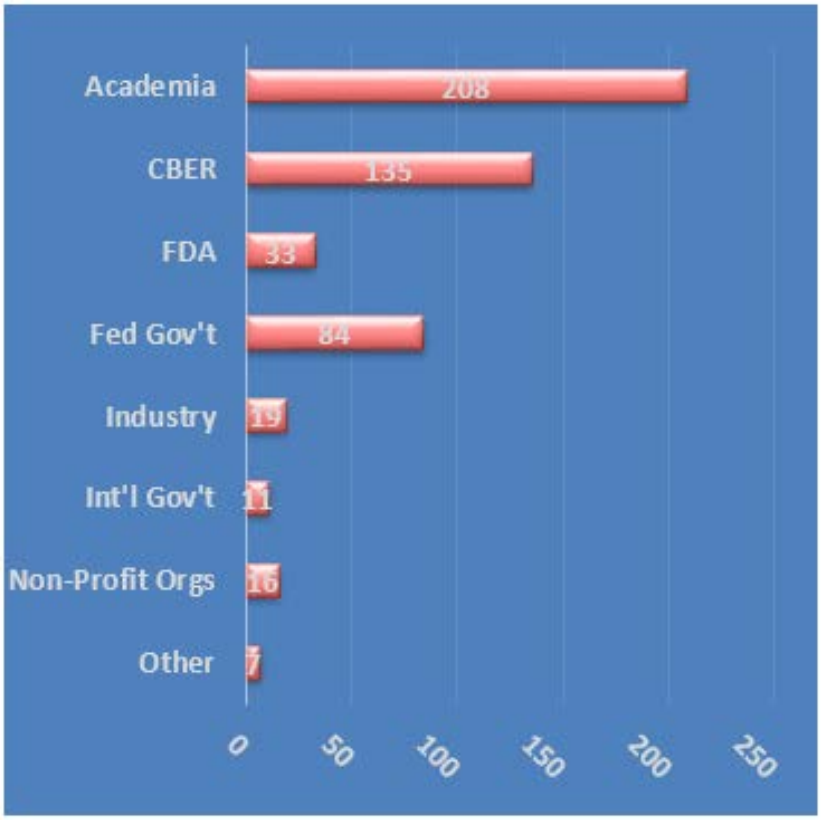
CBER Advances Regulatory Science through External Collaborations

- External collaborations



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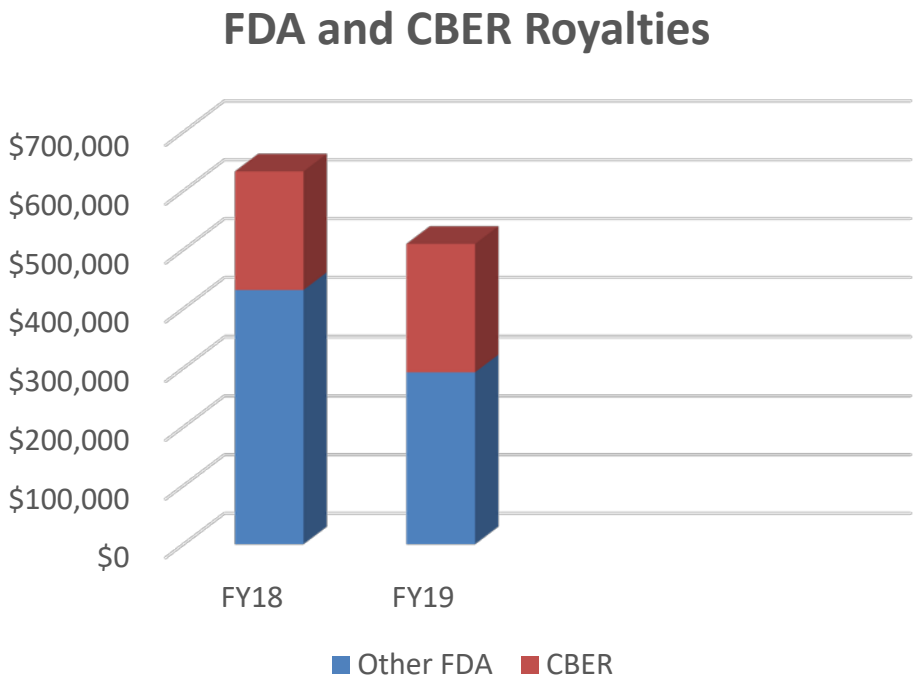
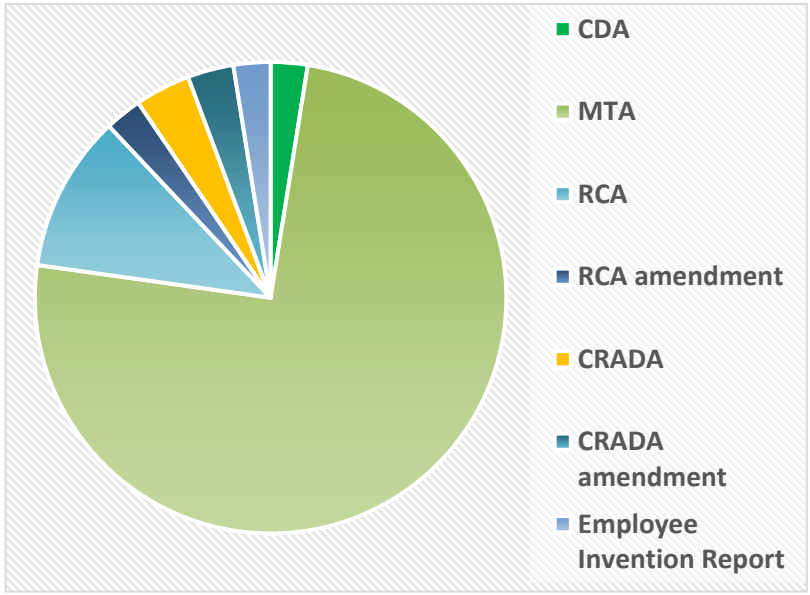
CBER Advances Regulatory Science through External Collaborations

- Leveraging formal external mechanisms: Contracts, Grants, Tech Transfer (158 agreements)





Data from FY19 CBER Research Reporting Database

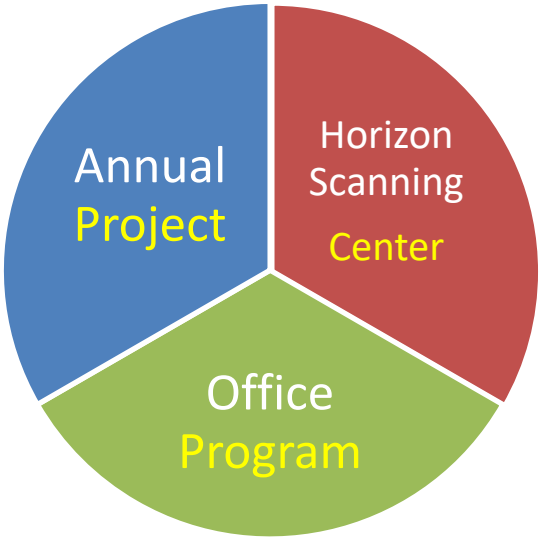
Research Management Processes

- CBER Regulatory Science Council develops
  - Research Goals and Objectives
  - Research evaluation framework and criteria to measure scientific and regulatory impact
  - Portfolio review of research program

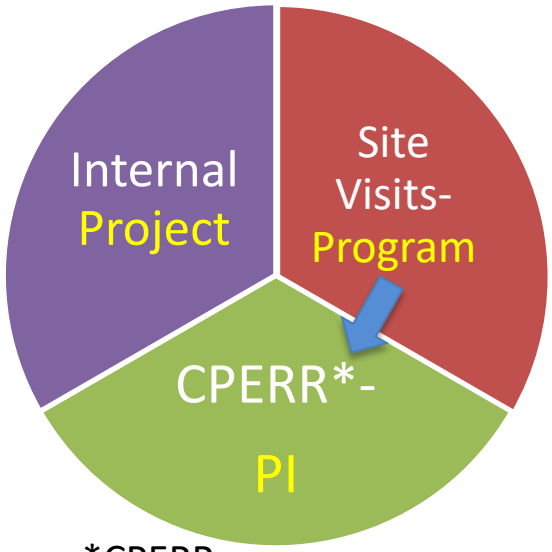
- Evaluation of research program
  - Management (Office and Division level)
  - Internal and External Peer Review

Overview of CBER Research Evaluation: Management and Peer Review

Management Review



Peer Review



*CPERR:
Committee for
Promotion and Evaluation
of Researcher-Reviewers

Evaluation	Frequency	By Whom
Internal Project Review	New Projects, every 4 years	FDA Scientists
Site Visits-External Program Review	Every 4 years	External SME
CPERR-PI Review (GS-14,15)	Initial and every 4-5 years	CBER scientists

CBER Evaluation Framework

Mission Relevance:
Alignment with Goals and Objectives

Scientific and review capability

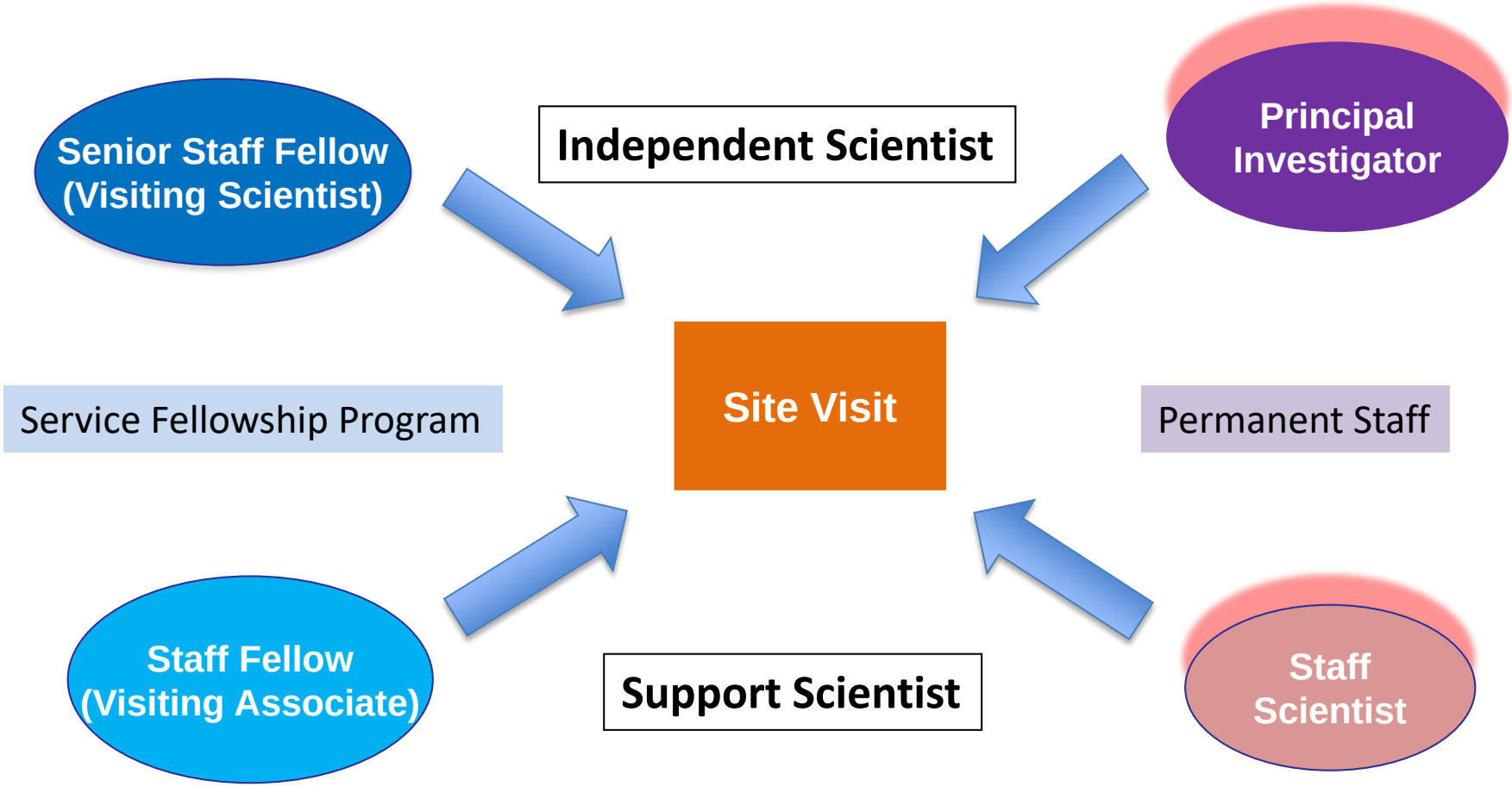
Dissemination:

Presentations publications

Scientific Impact:
Uptake by scientific community and regulated stakeholders

Unique Contribution Regulatory Practice:
Scientific outcomes enhance our regulatory mission

Researcher-Reviewers Who Undergo External Peer Review



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 Committee for Promotion and Evaluation of Researcher-Reviewers (CPERR) for personnel actions
  - By PIs for improving research program
  - By management, resource allocation decisions may be impacted by report (pending resource availability)

Benefits of CBER Research Program

- The integration of research and review:
  - Ensures relevance, expertise, timeliness, usability
  - Fosters rational policy and decisions based on sound science, law, public health impact
- 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

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!