Visioning CBER Research in 2025

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FDA Science Board

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Introduction

- CBER Overview
- McKinsey Consulting Company Review of CBER's ***Management*** of Regulatory Science Program
  - Purpose
  - Process
  - Recommendations
- Implementation of Recommendations:
  - Visioning CBER Research in 2025
- Next Steps

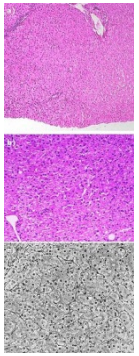
CBER Regulates Complex Products

Mission: To ensure the safety, purity, potency, and effectiveness of biological products including vaccines, blood and blood products, and cells, tissues, and gene therapies for the prevention, diagnosis, and treatment of human diseases, conditions, or injury

Cell & Gene Therapies

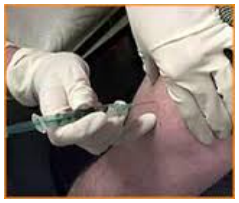
Blood, Blood Components and Derivatives

Xenotransplantation Products



Tissues

Vaccines: Preventive & Therapeutic

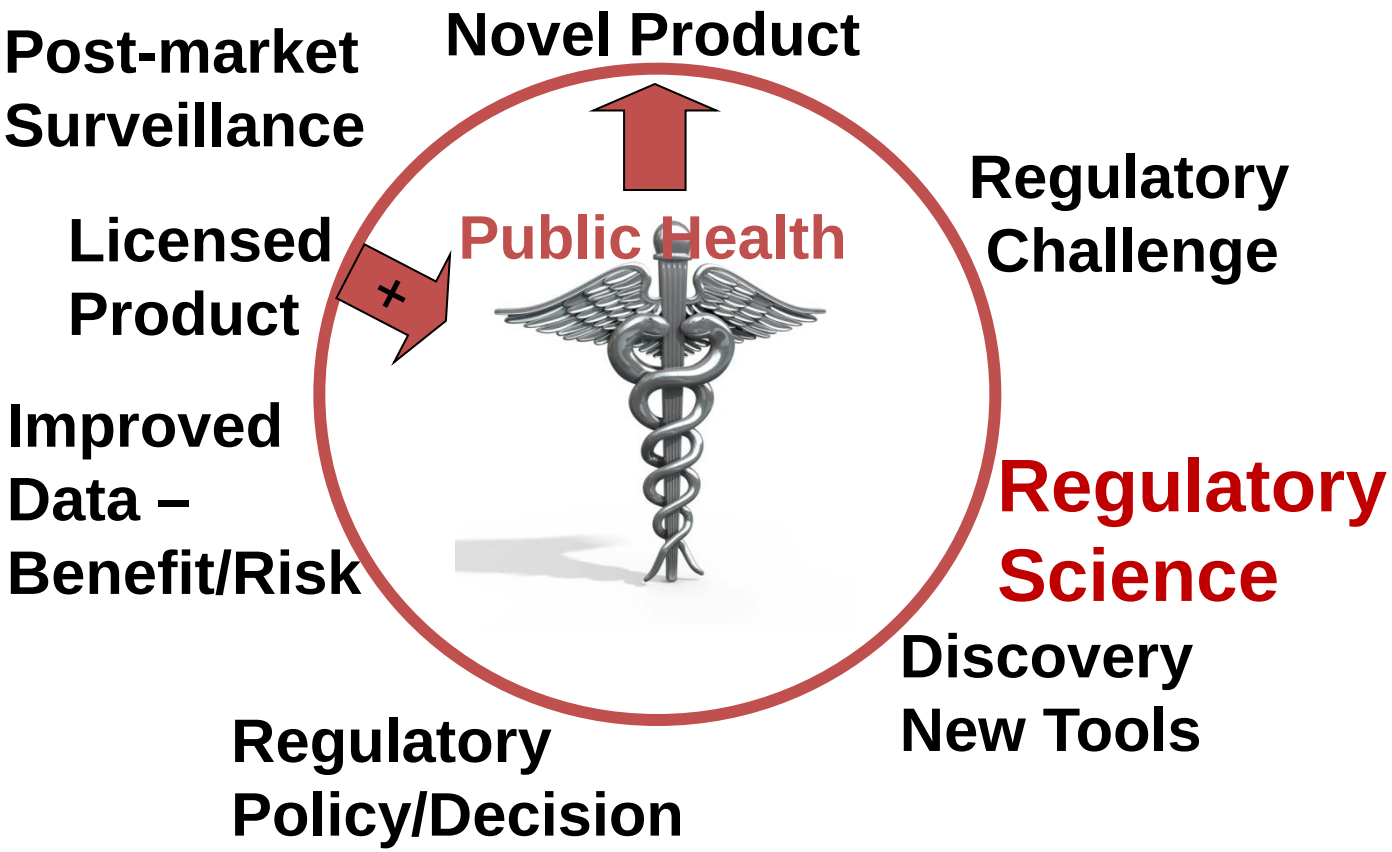


Related Devices

Live Biotherapeutics

Allergenic Products

Using Science and Regulation to Advance Product Development

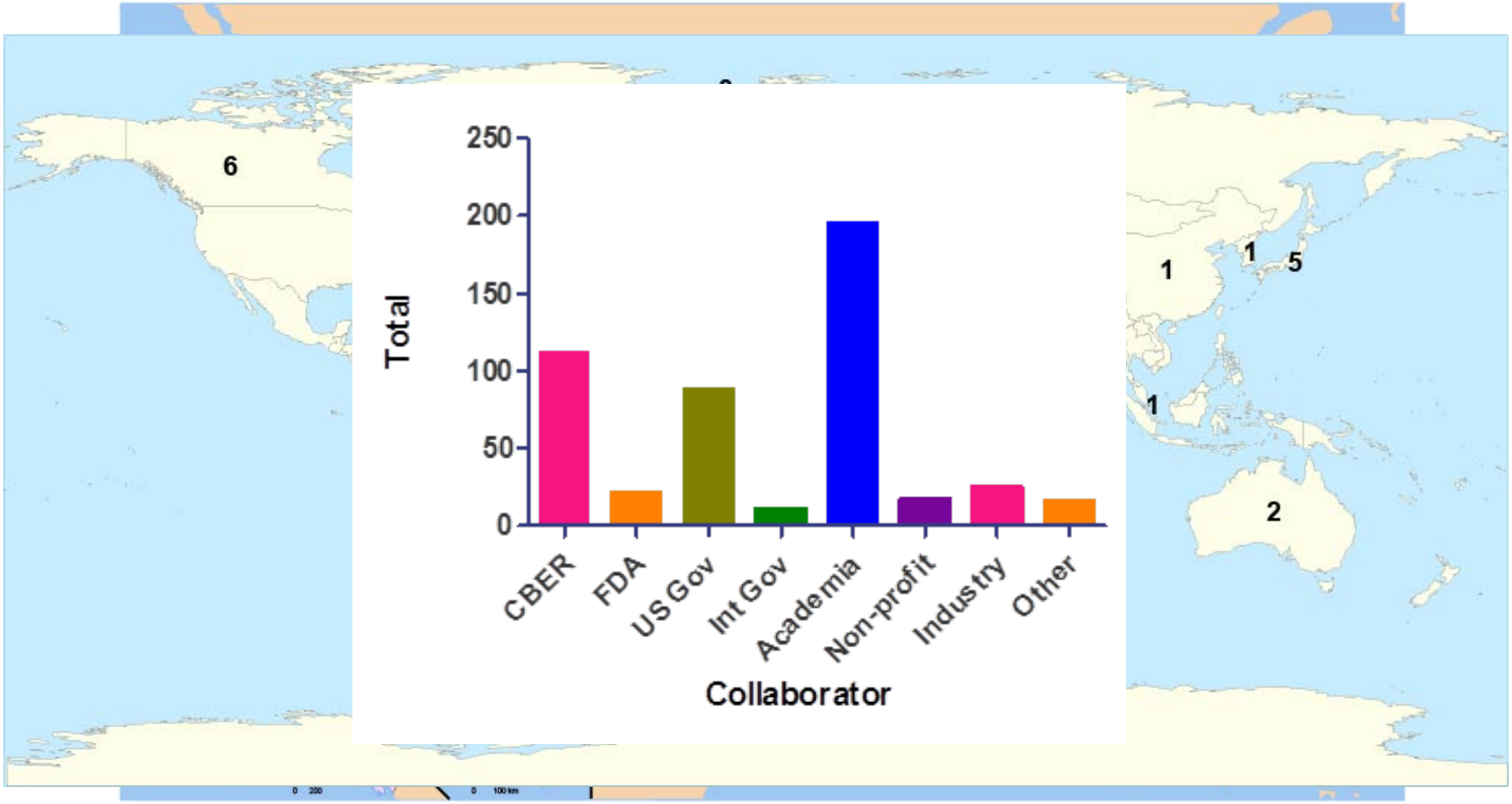


CBER researcher =
“Researcher-Reviewer”
~20% CBER Staff

Integration of research and review ensures

Relevance, Expertise, Timeliness, and Usability

CBER Advances Regulatory Science through External Collaborations



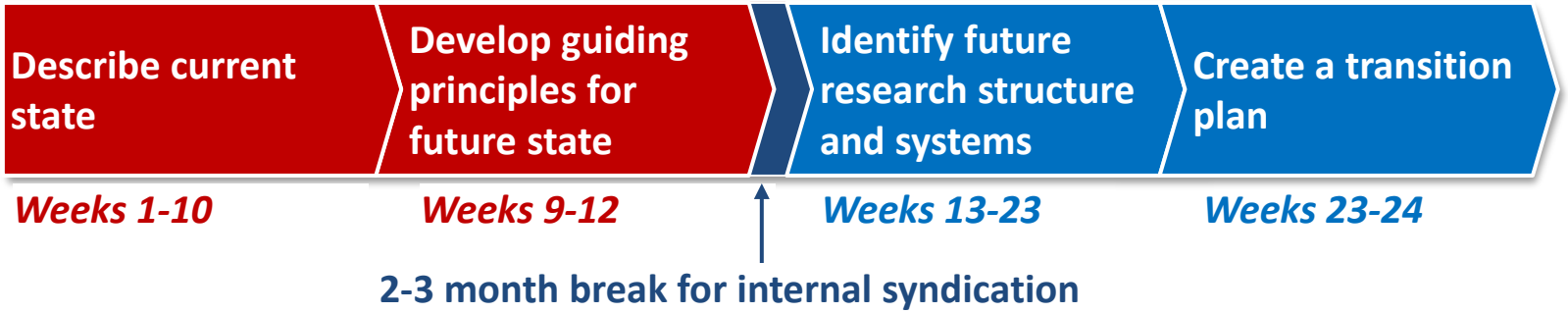
Data from FY16 CBER Research Reporting Database

Objective of McKinsey Review:

Enhance CBER's regulatory science and research to support its regulatory mission

- **Build upon a rich tradition of scientific research related to regulatory work in support of the public health**
- **Better understand the current state of research in the context of the Center, Agency, and public health**
- **Optimize processes, systems, and tools to maximize the impact of our investment in research activities**
- **Further foster an environment conducive to leadership in scientific research with regulatory relevance**

McKinsey Timeline



Input was obtained from a number of sources, including the following:

- 12+ biweekly Steering Committee meetings
- 50+ Working team / workstream meetings
- 40 1:1 interviews with key CBER stakeholders and 9 focus group sessions
- 10 benchmarking interviews with peer institutions
- Data extracts from several key systems and other sources

Initial Findings: CBER has a truly unique, world class research program that supports review activity and contributes to improving public health

Dating back to 1902, CBER has a rich history of regulatory- and research-related contributions to our nation's public health

CBER is currently a **world class organization** with a diverse research portfolio in support of its mission that:

- Allows CBER to **stay abreast of and contribute** to the rapidly growing technical and scientific knowledge base related to biologics
- **Improves CBER's ability to conduct** effective and efficient regulatory reviews
- Enables CBER to **nimbly and quickly investigate and respond** to urgent public health concerns

McKinsey's effort was designed to help CBER maintain and enhance its world class, mission-relevant research program

The first phase identified several strengths of CBER's current state research portfolio…

	Specific points cited by interviewees
Setting of research priorities	Noble, **mission-driven motivation** to improve public health At least in some Offices, **all PIs and staff are included** in setting of research priorities **High quality expertise** is helpful both day-to-day and in response to urgent public health needs
Allocation of funds	Office-level distribution of funding feels **in line with Office review and research mission** **Flexibility of funding** for emergencies can enable quick adjustments
Review and approval of projects	Current review processes – including Targeted Funds peer review – **have merit and add value** to overall CBER activities Multi-level review of research programs provides **ample opportunity for feedback**
Tracking and performance management	Data-tracking systems currently **capture a large amount of data** and allow for *ad hoc* analyses

The first phase identified several strengths of CBER's current state research portfolio…

	Specific points cited by interviewees
Overall organizational model and supporting systems	**Research-Reviewer model works well** and improves quality of both review and research Helpful and **collaborative culture** (e.g., willingness to share equipment) CBER research is supported by quality, **cutting edge technology and equipment**
Talent management and people development	Respect for top **tier scientific expertise** / talent of peer researchers and leadership Most fellows report **high level of satisfaction**
IT tools and systems	**IT systems perform their function**, and owners of IT systems and databases willingly work collaboratively **Annual Reports allows for thorough review** of research projects by Division and Office leadership

As well as four areas of opportunity for enhancement, turned into workstreams

Portfolio management: Prioritization, oversight, and review of research activity

Budget administration: Timing, allocation, and distribution of funds

Transparency: Data, tools, and processes that describe how research is unfolding and is managed

Organization: Structural and cultural enablers of research

As well as four areas of opportunity…

Portfolio management:

- Research priority setting process
- Governance and oversight
- Balance of different funds and role of peer review (internal / external)
- Common language/framework for discussing and evaluating impact
- Pathways for non-lab research

Budget administration:

- Management of different types of funding going to research
- Timing of funding
- Ability to track and anticipate funding / FTE burn and potential sources of extra money

Transparency:

Data, tools, and processes that describe how research is unfolding and is managed

- User-friendliness and accessibility of data
- Integration of different data sources
- Comprehensiveness of data
- Decision-making transparency

Organization:

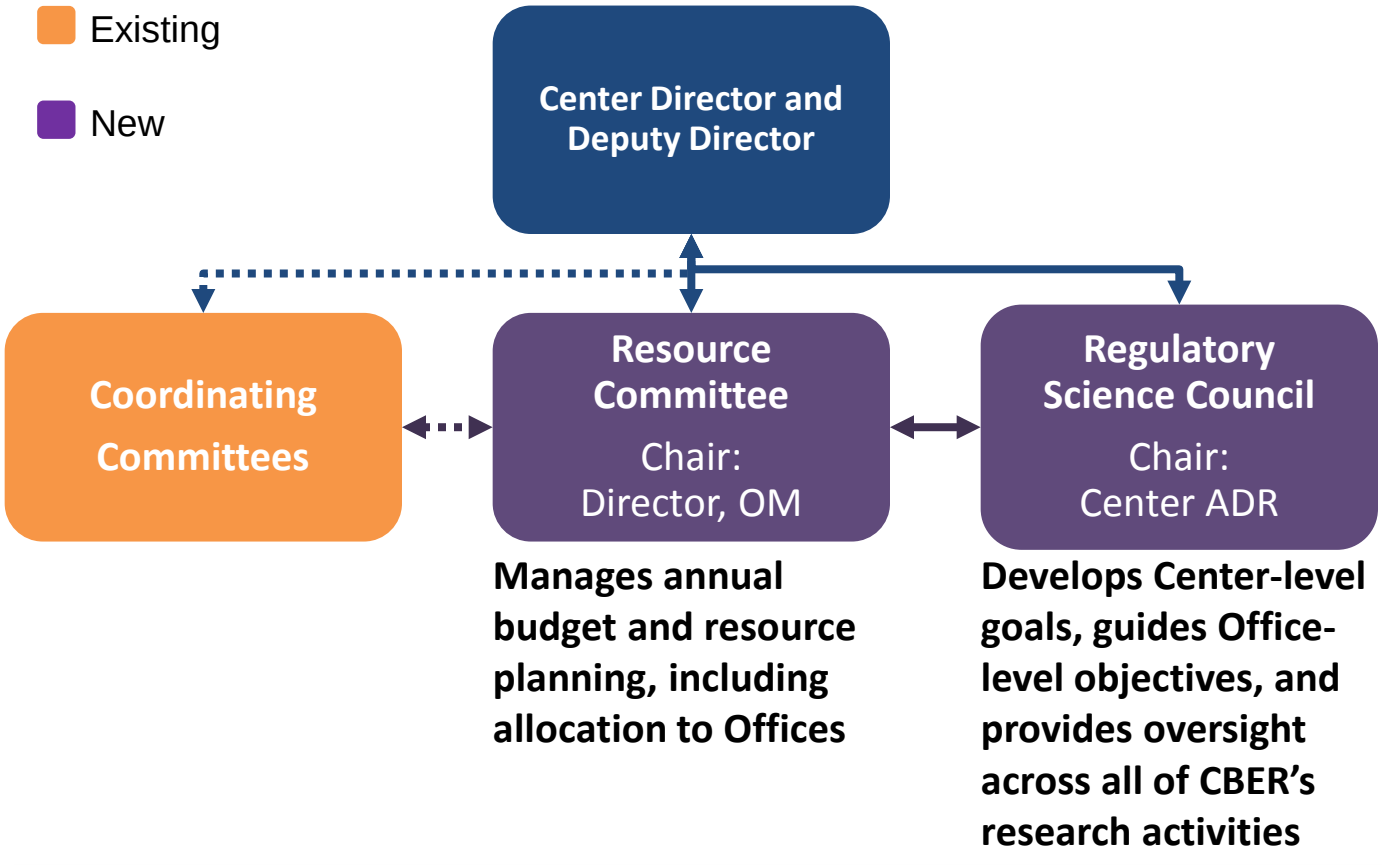
Structural and cultural enablers of research

- Leadership engagement
- Cross-Office dialogue
- Talent development and recruitment
- Culture of science
- Elevation of CBER's science to the outside (within FDA / external)

Overview of key solutions

- **Establishment of two governance bodies** to oversee research priorities and budget and resource planning
- Implementation of a **framework** for setting and regularly revisiting forward-looking, Center and **Office-level research goals and objectives**
- Changes in **fund allocation**
  - Increase in funds distributed to Offices as Operating funds
  - Prospective, Center-wide budgeting and resource allocation process and automation of budget planning
  - Revised advisory intramural peer review process
- Initiatives to **increase transparency** that include a **CBER research dashboard**, plan for an updated **time tracking system** and **updates to the annual report**
- Proposals to **elevate CBER's culture of science**, **leadership engagement** in research, and the **training environment** to CBER's junior staff

Establish two new governance bodies that oversee research priorities and budget and resource planning



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

Research Management Webpage

http://inside.fda.gov:9003/ProgramsInitiatives/Biologics/Research/ucm494810.htm

Research Central
CBER Research Management

CBER Regulatory Science and Research Vision

*To conduct scientific research of the **highest quality and relevance**, that is integral to the Center's regulatory mission and public health portfolio, **proactive and anticipates** regulatory and public health needs, and **in direct support of CBER's regulatory decision-making and policy development responsibilities.***

7 Pillars of CBER Research Management

- The Researcher-Reviewer model and investigator-initiated projects form the foundation of CBER research
- Forward-looking priority setting coupled with periodic, portfolio-level review ensures mission relevance, regulatory impact, and the breadth of expertise required to nimbly respond to public health needs
- Effective use of leadership engagement, unique internal knowledge, and external expert input supports a balanced approach to research review, oversight, and assurance of the highest scientific quality
- CBER uses a single impact framework to plan, evaluate, and communicate research
- Structure, timing, and communication of funding enables prospective planning of the content and direction of research
- Decision-making will be informed by transparency into research spend, status, and impact, and decision processes and criteria will be openly communicated to the entire Center
- CBER embraces a culture that values research and fosters recruitment and retention of the best available scientific talent

CBER's research management strategy provides the pillars upon which we fulfill our vision of conducting highly relevant, impactful regulatory science and research.

2016 Center Regulatory Science and Research Goals

Goal 1. 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.

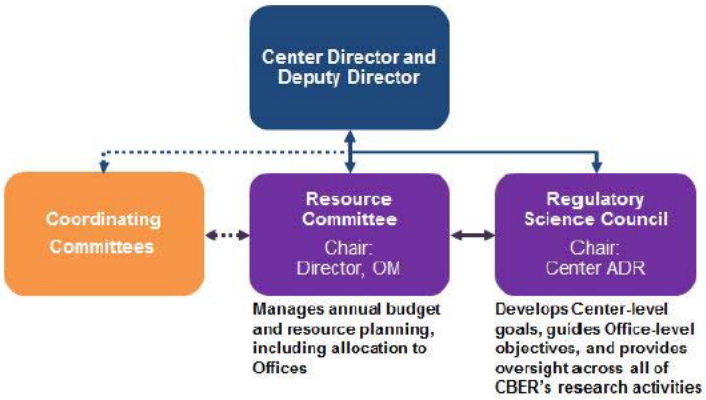
Goal 2. Develop and assess nonclinical models and methods with improved predictive value, and, as feasible, reduce, refine, or replace the use of animals, for evaluation of safety and effectiveness of CBER-regulated products.

Goal 3. 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.

Goal 4. Prepare for future regulatory and public health challenges through investments in emerging science and technology, and develop and sustain varied scientific expertise

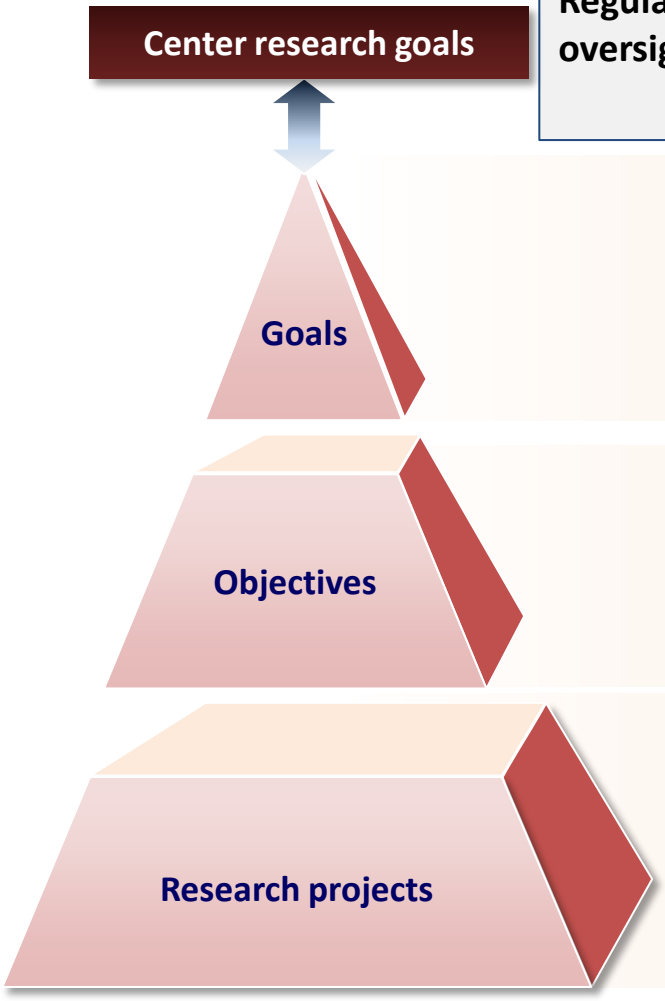
...*all 2016 Center and Office Goals and Objectives*

Management Structure



Link to charters

Implement a framework for setting and regularly revisiting forward-looking, Center and Office-level research goals and objectives



Regulatory Science Council develops broad, Center-level goals and provides oversight across CBER's research activities
(Refreshed every 5 years)

- Over-arching, multi-year efforts that are framed broadly
- Roll up and are aligned to Center goals
- Each Office should have 3-6
- Should be refreshed every 3-5 years

- Narrower, clearly achievable endeavors, aligned to goals
- Each Office should have 6-20
- Should be refreshed every year

- Investigator-initiated lines of scientific inquiry that will support and inform the regulation of that Office's products
- Should be reported on every year
- Include clear specific aims that can be tracked

2016 Center Goals
Regulatory Science and Research

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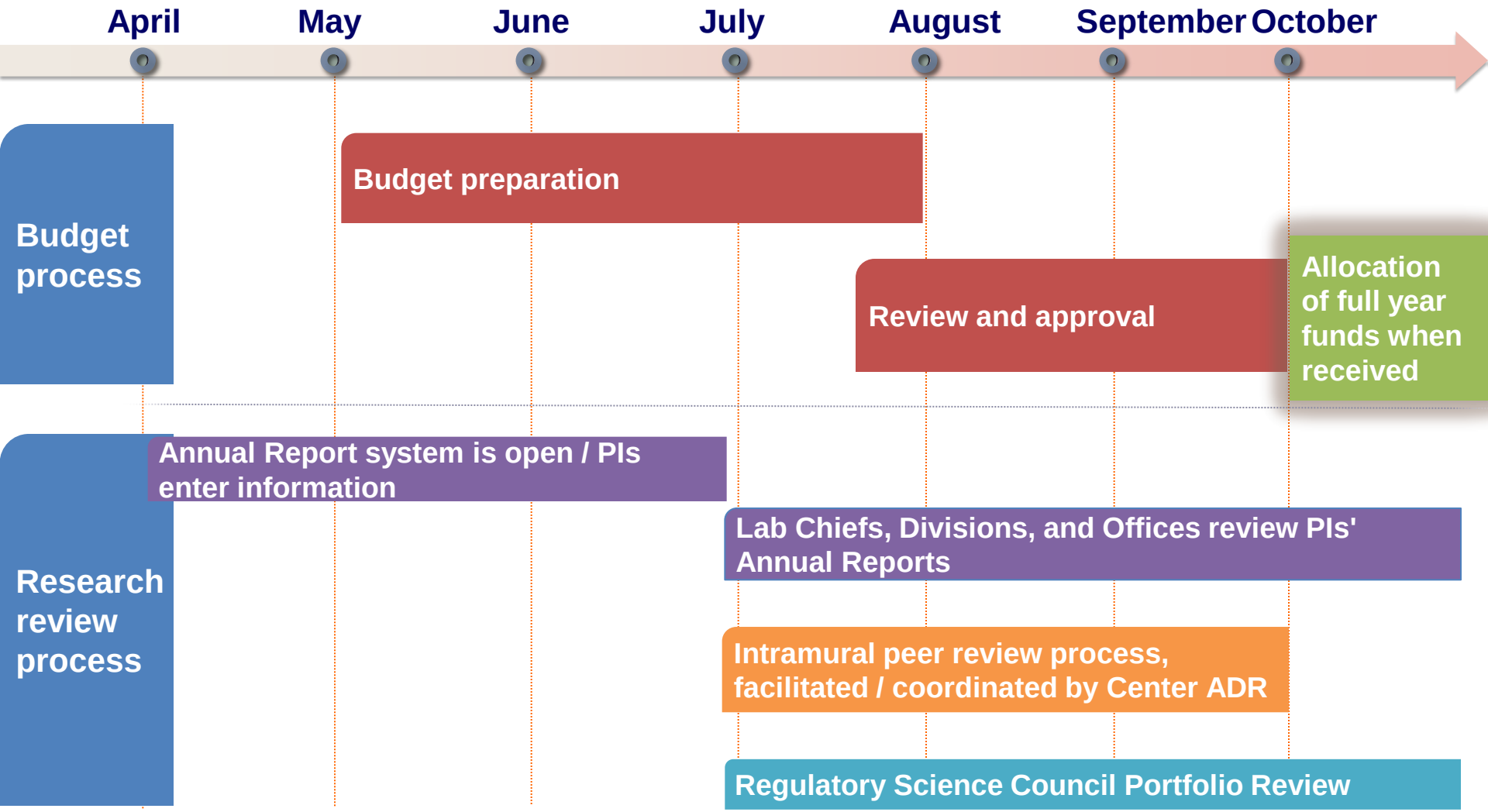
Goal 4. Prepare for future regulatory and public health challenges through investments in emerging science and technology, and develop and sustain varied scientific expertise.

Office Goals	Center Goal #1	Center Goal #2	Center Goal #3	Center Goal #4	Center Goal #5
OVRR 1	✓				✓
OVRR 2		✓		✓	✓
OVRR 3	✓				✓
OCTGT 1	✓				✓
OCTGT 2		✓		✓	✓
OCTGT 3	✓	✓		✓	✓
OCTGT 4	✓				✓
OBRR 1	✓	✓			✓
OBRR 2	✓	✓			✓
OBRR 3	✓	✓			✓
OBE 1			✓	✓	
OBE 2					✓
OBE 3			✓		✓
OBE 4			✓		

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**	

Allocate funds in the most effective manner



Review Processes

Intramural Peer Review

- Advisory role (indirectly informing funding allocation decisions)
- Review 25% of existing projects plus all new project each year
- Project information will come from updated annual reports
- Reviewers from CBER and other FDA Centers to increase expertise
- Provide written reviews
- Proposals qualitatively ranked: High-Med-Low

Portfolio Review

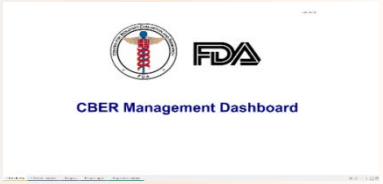
- FY17: All CBER Project to be reviewed
- Out-years: Individual offices to be reviewed
- Projects will be assigned to each office for review (offices will review programs from other offices)
- Office management will utilize the CBER Research Dashboard to review projects
- Offices will utilize the research impact framework to evaluate projects/portfolio.

CBER research dashboard for management tool

Dashboard

- **Insights into the research portfolio**
- **Enhances management decisions**
- **Detail at Office, program and project level** (e.g., goals, program titles, research staff)
- **Budget information**
- **Sources of funds**

1. **Introduction view**

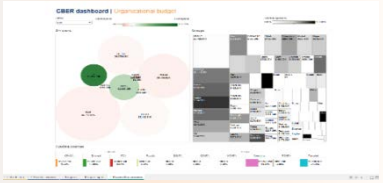


2. **Thematic overview**

3. **Programs view**

4. **Program report view**

5. **Organizational overview**



Revised Annual Reports

	Specific changes
Tighter alignment to Agency, Center & Office objectives	• Identify alignment of each program to one primary and up to two secondary FDA and CBER Goals • Identify alignment of each project to one primary and up to two secondary Office objectives • Include 1-3 clear specific aims for each project;
Clarifying expected impact	• Identify alignment of new scientific publications to – Primary and secondary FDA, Center, and Office research priorities – Specific projects • Describe projected final outcome and impact of projects
Clarity of funding and budget	• Specify funding streams applicable for each project • Report budget and staff information at the project level

Elevate CBER's culture of science, leadership engagement in research, and the training environment to CBER's junior staff

	Description
 **CBER Science Impact Series**	• Monthly presentations highlight the impact and relevance of CBER's research • Target audience: ENTIRE CENTER • 2 PIs present each month: • **Kick-off and 7 monthly seminars in 2016**
 **CBER Science Symposium**	• Every other year to share recent progress across all of CBER's research portfolio and support a culture that values science • **First symposium, May, 2016**

 **Enhanced support for fellows**	• Enhancing onboarding support
	  • **Updated Welcome Package**
	• Increasing transparency into developmental and career progression opportunities
	  • **FFA seminar series for career options**
	• Resume Repository
	  • **PILOT: Folder to denote FDA ORISE experience**
	• Satisfaction Survey
	  • **FFA – will be sent November, 2016**
	• Exit Surveys
	  • Through ORISE, to be implemented FY17

Other	• External facing PI Websites (pilot initiating)

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Draft External PI Website

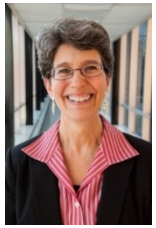
Vaccines, Blood & Biologics

Home › Vaccines, Blood & Biologics › Science & Research › Biologics Research Projects

- Science & Research (Biologics)
- Biologics Research Projects
- General Biologics Research
- Allergenics Research
- Blood Research
- Cellular & Gene Therapy Research
- Tissue Research
- Vaccines Research
- Xenotransplantation Research

Resources for You

- Science & Research (Biolgoics)
- About the Center for Biologic Evaluation and Research (CBER)



Viral Safety Studies in Xenotransplantation and Gene Therapy Products

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Office of Cellular, Tissue and Gene Therapies
Division of Cellular and Gene Therapies
Gene Transfer and Immunogenicity Branch

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General Biosketch Paragraph

General Overview

XXXX……..

Scientific Overview

Our laboratory is primarily interested in XXXXX ……

Publications

1. XXX
2. XXX
3. XXX
4. XXX
5. XXX
6. XXX
7. XXX
8. XXX
9. XXX
10. XXX

Links

- Research summaries (Innovation and Regulatory Science)
- Scientific Posters (Innovation and Regulatory Science)
- External FDA links of relevance (Guidance documents, etc.)

Taken together, these initiatives support a comprehensive **research management strategy** which provides the pillars upon which we fulfill our vision of conducting highly relevant, impactful regulatory science and research.

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Next Steps

- Review of FY16 Implementation of Recommendations
  - What works well
  - What improvements need to be made

- CBER Regulatory Science External Review

CBER Research Review Subcommittee to FDA Science Board

Given the existing breadth of CBER's current and anticipated future regulatory portfolio and responsibilities, are there changes CBER should make to its regulatory science research portfolio to best accomplish our regulatory and public health mission?

- Assess any gaps in regulatory science capabilities or expertise.
- Identify scientific areas where CBER should make programmatic and resource changes.
- Identify opportunities for collaboration to better leverage CBER's regulatory science programs.

With Thanks

Peter Marks, MD, PhD, Director, CBER

Emily Braunstein, PhD, Program Manager