

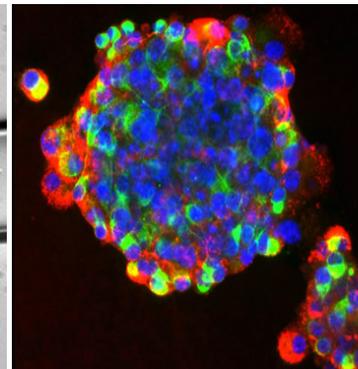
Center for Biologics Evaluation and Research FDA

Overview

Regulatory Science Program and Priorities

Carolyn A. Wilson, Ph.D.

Associate Director for Research



My comments are an informal communication and represent my own best judgment.

These comments do not bind or obligate FDA.

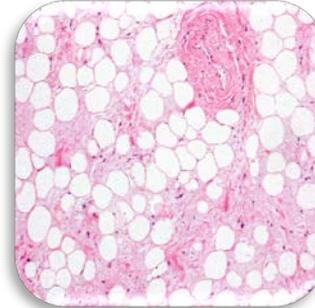
CBER Regulates Complex Products



Cell & Gene
Therapies



Blood, Blood
Components
and Derivatives



Tissues



Vaccines:
Preventive &
Therapeutic



Xenotransplantation
Products



Therapeutic
Probiotics

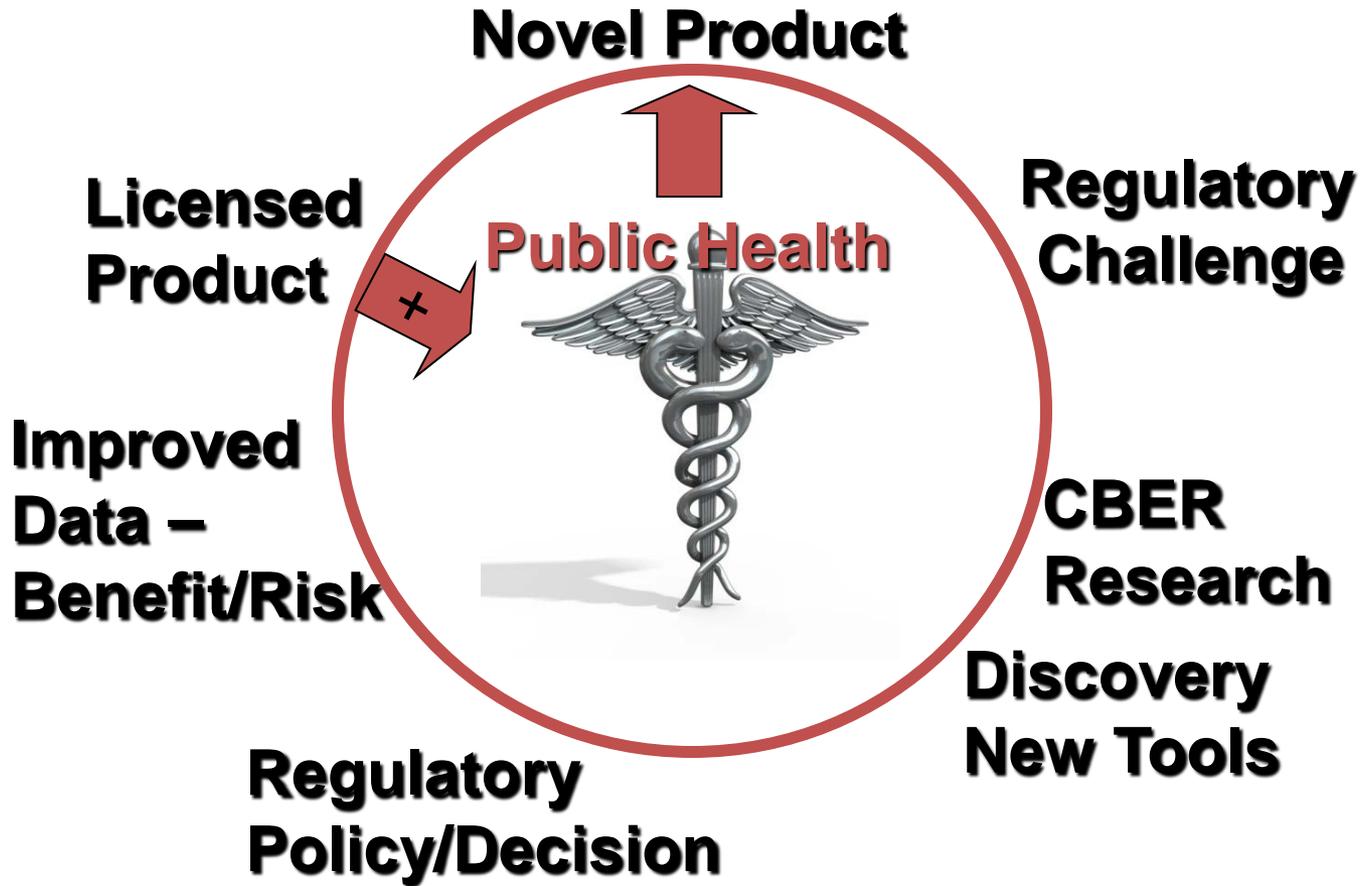


Allergenic
Products

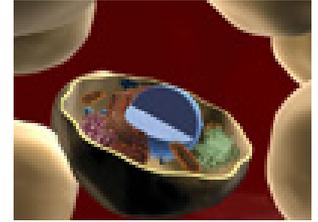


Related Devices

CBER Mission: Role of Research



CBER Strategic Plan for Regulatory Science and Research*



- **Increase national preparedness** to address threats from bioterrorism, pandemic and EIDs
- **Improve global public health** through international collaboration
- **Enhance ability of science and technology** to facilitate development of safe and effective biological products
- **Ensure safety** of biological products
- **Advance regulatory science and research**
- **Manage for organizational excellence**

*<http://www.fda.gov/BiologicsBloodVaccines/ScienceResearch/default.htm>



Scientific Expertise

- Novel technologies: NMR, mass spec, flow cytometry, high throughput sequencing
- Microbiology: parasitology, bacteriology, virology
- Immunology
- Biochemistry and molecular biology
- Cell and developmental biology
- Epidemiology, meta-analyses of large healthcare databases
- Biostatistics

CBER Laboratory Program

Now at FDA's White Oak Campus, SE Quad

Expanded Core Technologies:

flow cytometry

confocal microscopy

high throughput sequencing and bioinformatic support

10 BSL-3 suites

Designed to support work of at least 12 infectious agents and work of 36 PI's

Many suites with capacity for animal holding rooms

1 suite to support sterile sorts and live cell confocal microscopy on BSL-3 agents

Insectariums (BSL-2 and BSL-3)

Suites designed to support Microarray and PCR

Expanded NMR facility

Imaging facility with MRI, digital X-ray, IVIS, ultrasound

Transgenic derivation facility

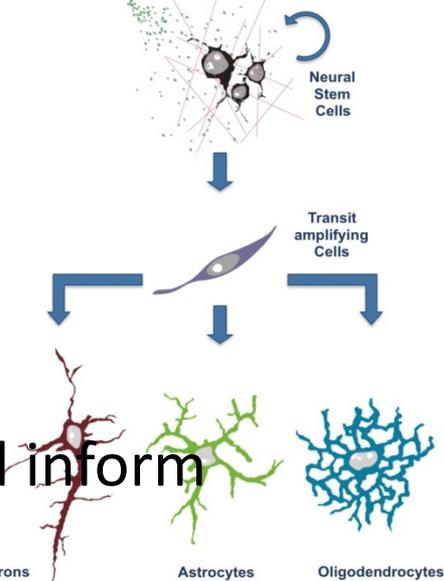
NHP holding, surgical suite

05/31/2013

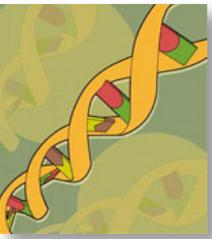
CBER Priorities for NCTR-CBER Collaborative Research

- **Imaging:** Trafficking and functional analysis of neural stem cells
- **Bioinformatics:** Validation of data standards for RNASeq using Next Generation Sequencing
- **Microbiology:** Improved assays to characterize fecal microbiota transplants

Imaging: Trafficking and functional analysis of neural stem cells



- **Need:** Improved in vivo imaging assays that could inform questions underlying regulatory decision-making:
 - Do cell therapies localize to the intended therapeutic site or are they found in ectopic/diffuse sites?
 - Are there imaging modalities that can assess cell function in vivo?
 - Are there imaging modalities that can identify markers that may represent appropriate or inappropriate cellular differentiation in vivo (or, if relevant, retention of stem cell phenotype)?
- Serguei Liachenko, NCTR
- Brent McCright and Alex Bailey, CBER
- Use immunodeficient mice and possibly relevant disease models to assess safety, function, and trafficking of neural stem cells

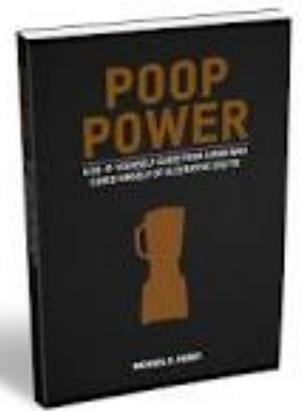


Bioinformatics: Validation of data standards for RNASeq using Next Generation Sequencing

- **Need:** Development of medical products that use NGS as a platform for the product itself or use NGS data to support medical product evaluation is expanding. NGS data and meta-data elements to capture need to be standardized to facilitate FDA review of these data/products, and advance the use of these technologies.
- Weida Tong, NCTR and Vahan Simonyan, CBER
- Validation of RNA seq pipelines within standardization framework proposed
 - Continuation of SeqQC consortium activities

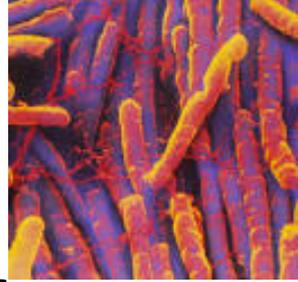


Microbiology: Improved assays to characterize fecal microbiota transplants



- ***Need 1***: Potency assays for fecal microbiota transplantation in order to evaluate product quality and effectiveness in a more standardized manner.
- Bruce Erickson, NCTR and Scott Stibitz, CBER
- Continuous culture of intestinal microbial consortia and determination of “colonization resistance” to intestinal pathogens.

Microbiology: Improved assays to characterize fecal microbiota transplants



- ***Need 2:*** Improved understanding of mechanisms of action of FMT for *C. difficile* treatment.
- Robert Wagner, NCTR and Scott Stibitz, CBER
- Challenge of two and three dimensional cultures of human intestinal epithelial and dendritic cell cultures with *C. difficile* bacteria with a readout of inflammatory biomarkers.

CBER's Regulatory Science Program

- ***Fills a unique niche to facilitate product development and meet our regulatory mission:***
 - Cadre of scientific experts who also understand the regulatory process
 - Allows proactive research to address regulatory science gaps
 - Allows facile responses to public health/regulatory emergencies