CBER's Perspective on Working Together with Our Rare Disease Partners across FDA

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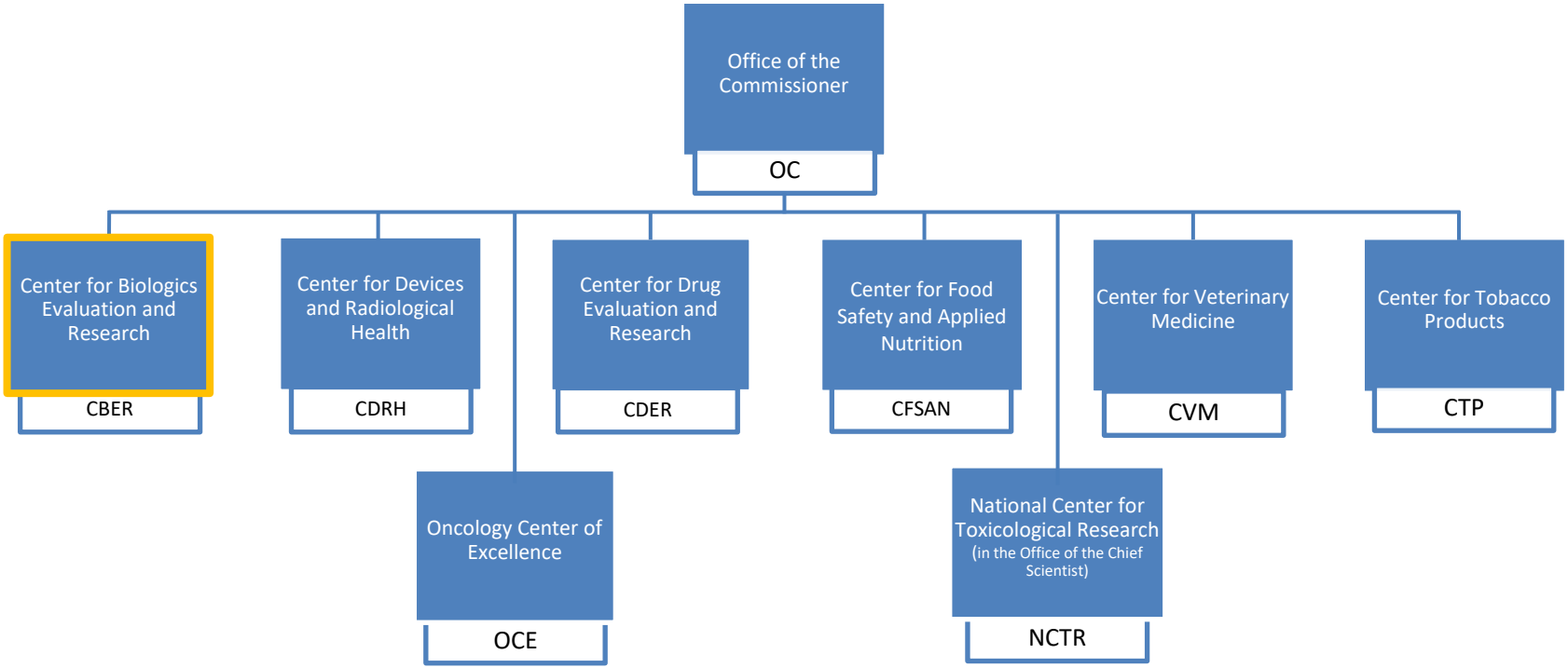
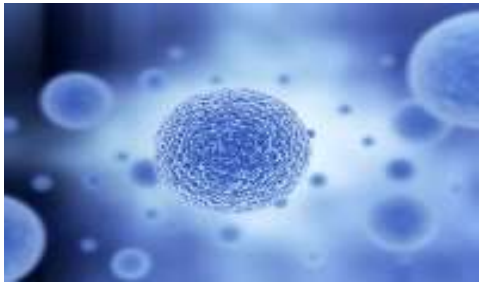
Learning Objectives

- Discuss CBER's recent rare disease and orphan product approval trends
- Describe CBER's Rare Disease Program
- Discuss CBER's rare disease-related PDUFA VI commitments
- Provide examples of how CBER collaborates with rare disease partners at FDA

The Center for Biologics Evaluation and Research (CBER)





Products Regulated by CBER

- Allergenics
- Blood Products
- Human Tissues and Cellular Products
- Gene Therapies (including genome editing)
- Vaccines (preventative and therapeutic)
- Xenotransplantation Products
- Devices Related to Biologics
- Miscellaneous (e.g., phage therapy, FMT)

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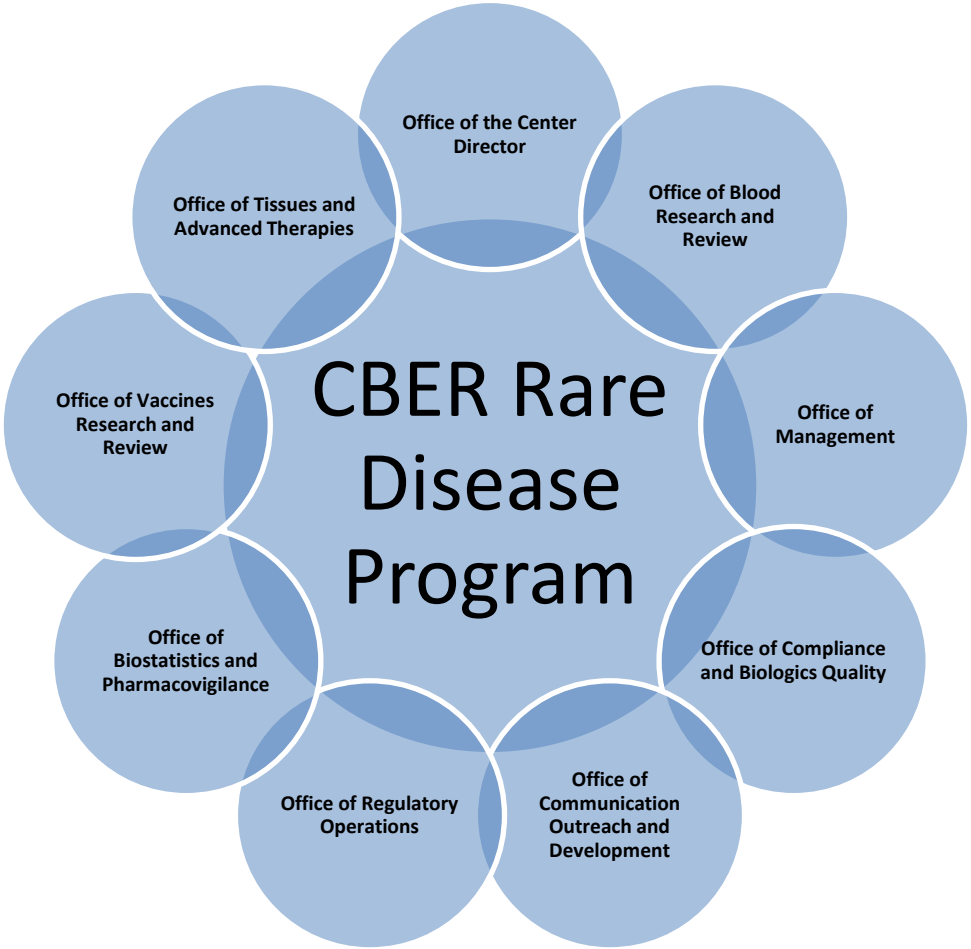
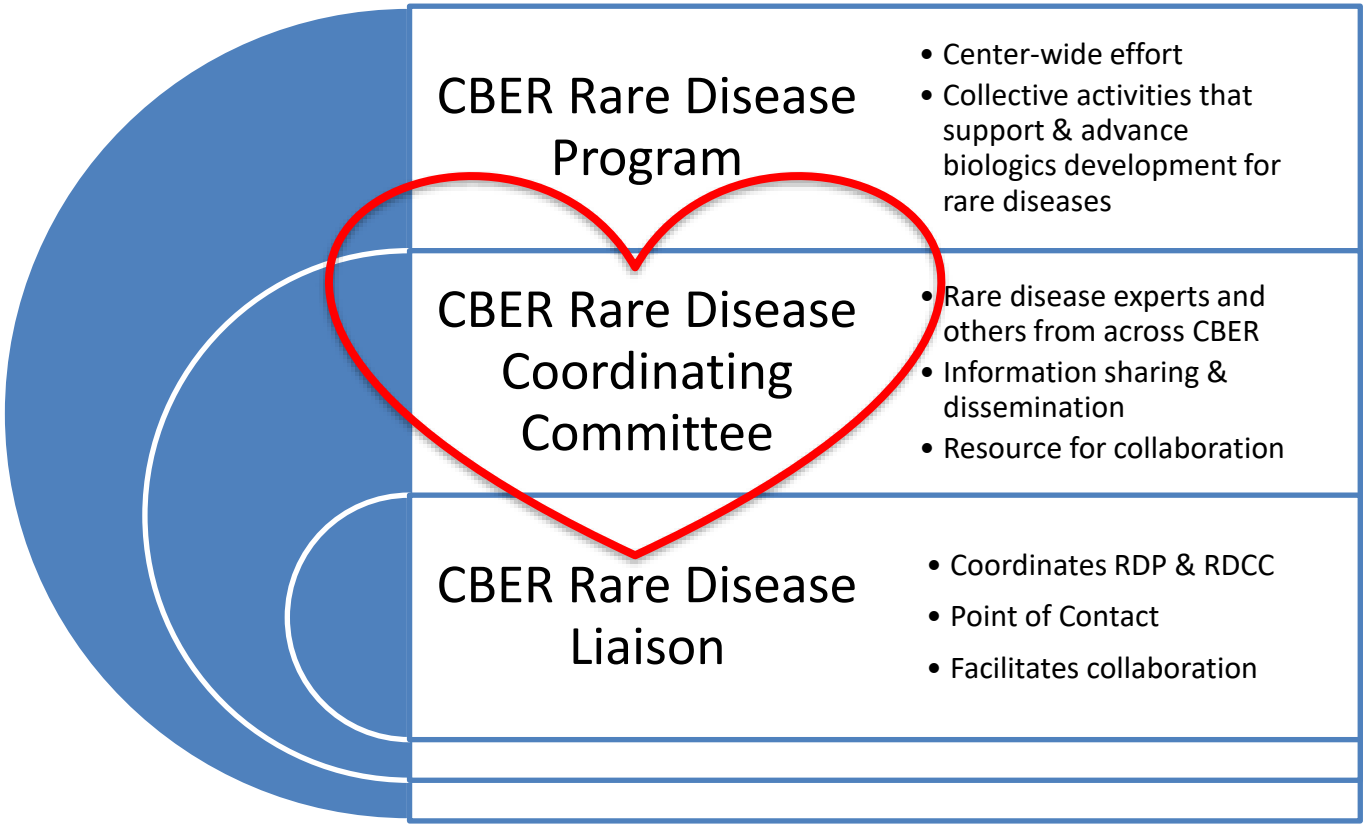
CBER approved 59 novel biologics* from 2015 through 2021

Of these,

- 38 (64%) were approved for use in rare diseases
- 18 (30%) were orphan designated

*Excludes in vitro diagnostic products, reagents and intermediate biological products approved for further manufacture, such as source plasma


CBER Rare Disease Program
Office of the Center Director
Office of Blood Research and Review
Office of Management
Office of Compliance and Biologics Quality
Office of Communication Outreach and Development
Office of Regulatory Operations
Office of Biostatistics and Pharmacovigilance
Office of Vaccines Research and Review
Office of Tissues and Advanced Therapies


CBER Rare Disease Program
• Center-wide effort
• Collective activities that support & advance biologics development for rare diseases
CBER Rare Disease Coordinating Committee
• Rare disease experts and others from across CBER
• Information sharing & dissemination
• Resource for collaboration
CBER Rare Disease Liaison
• Coordinates RDP & RDCC
• Point of Contact
• Facilitates collaboration

CBER Rare Disease Program Mission Statement

To facilitate and advance the development and timely approval of safe and effective biologics to improve the lives of children and adults with rare diseases.

CBER Rare Disease Commitments

(PDUFA VI)

- Ensure review offices consider flexible & feasible approaches in review
- Provide rare disease training to review staff
- Engage in stakeholder outreach
- Include updates on activities & successes in annual performance report

CBER Collaborates with Rare Disease Partners at FDA

- Patient engagement events
- Training for reviewers
- Stakeholder outreach (e.g., FDA Rare Disease Day)
- Submission Review
- PDUFA commitments
- Cross-cutting issues or requests
- Guidance development
- Regulatory initiatives (e.g., CDER's new ARC program)
- FDA/EMA/HC Rare Disease Cluster Meetings

Conclusion

- The majority of CBER's novel approvals are for rare diseases
- CBER's Rare Disease Program aims to advance development of CBER biologics for rare diseases
- CBER collaborates with rare disease partners at FDA in many ways
- Working together helps us accomplish our shared goals!

Challenge Question

Which of the following was not noted as an example of a collaborative activity with rare disease partners at FDA?

A. Rare Disease Cluster meetings with the EMA and Health Canada

B. Training for FDA reviewers

C. Planning committee for a new visitor parking lot for public meetings

D. Guidance development

E. Stakeholder outreach

U.S. FOOD & DRUG ADMINISTRATION

Questions?

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