RegenMedEd: Empowering Patients and Advocates to Advance Rare Disease Research

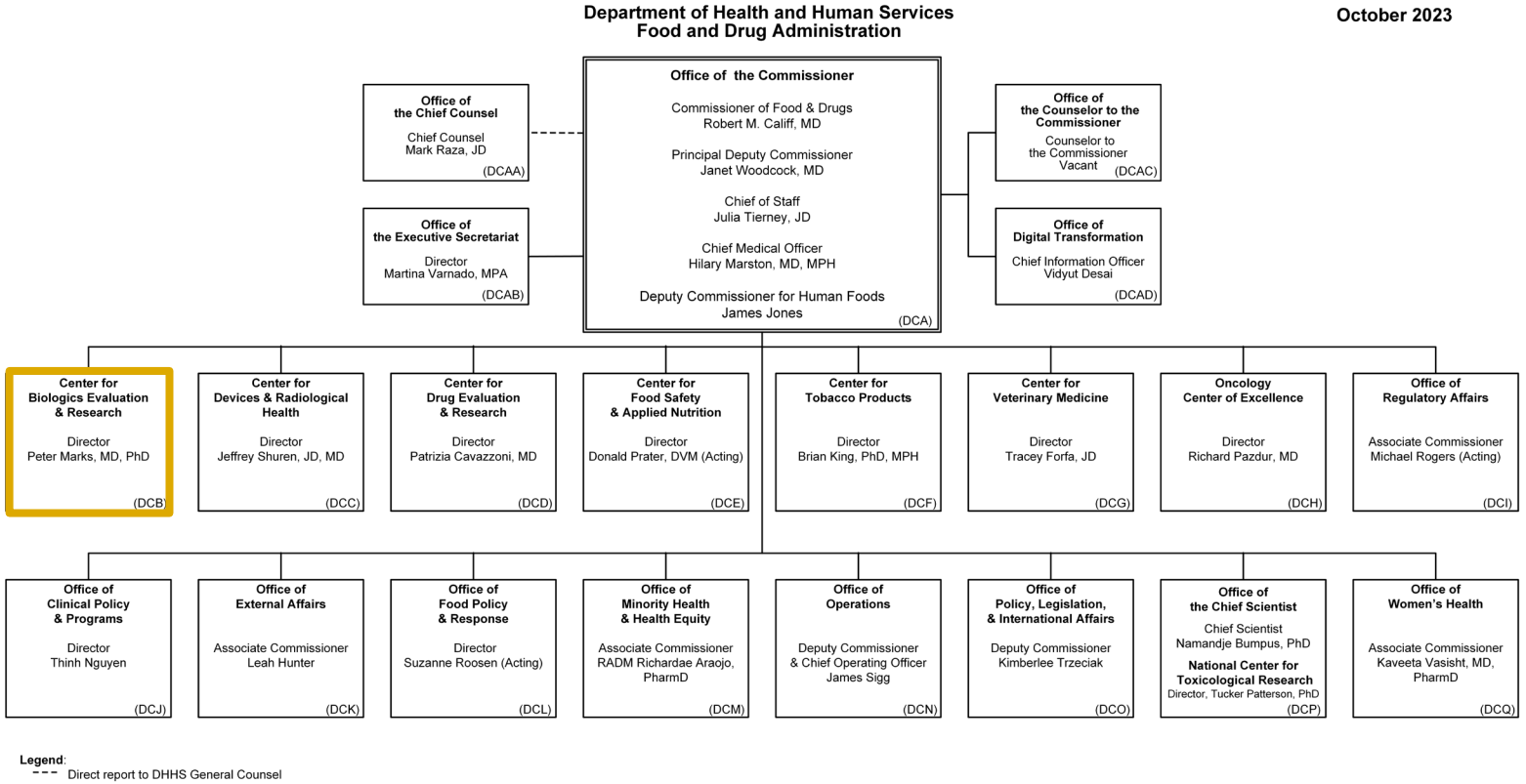
Anne Rowzee, PhD
Associate Director for Policy
Policy and Special Projects Staff
Office of Therapeutic Products
Center for Biologics Evaluation and Research

March 1, 2024

Overview

- Introduction to FDA CBER OTP
- OTP Regulated Products & Therapies
- Spotlight on Recent Patient Engagement Activities
- What's to Come

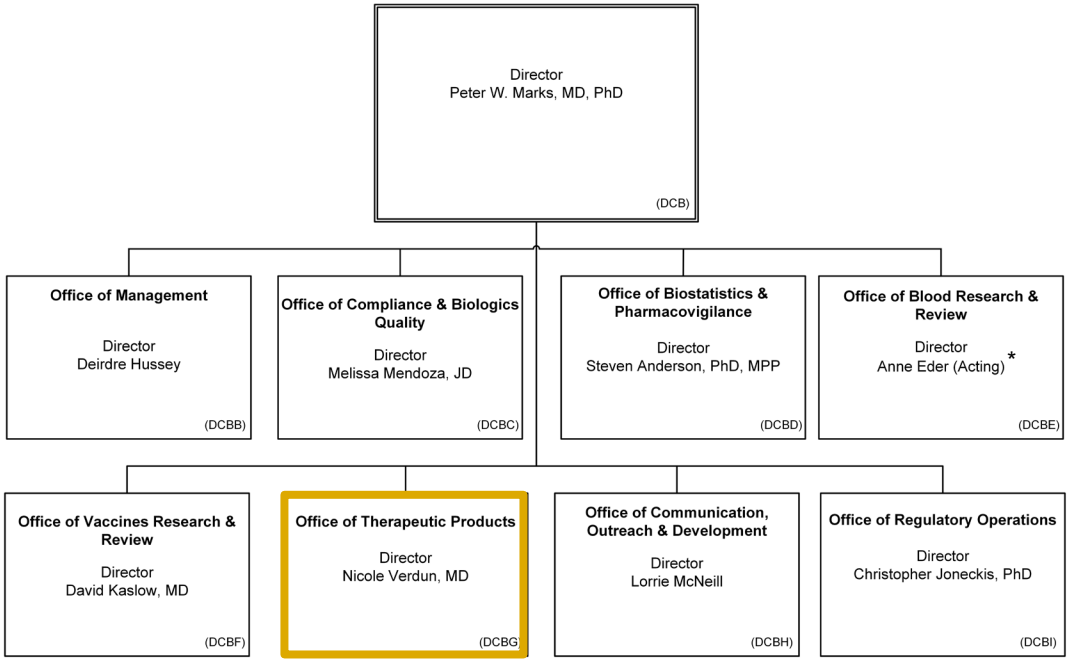
FDA Organizational Structure



CBER Organizational Structure

October 2023

Department of Health and Human Services
Food and Drug Administration
Center for Biologics Evaluation and Research



* Dr. Anne Eder was named Director of OBRR January 2024

OTP Philosophy

The Office of Therapeutic Products (OTP) promotes public health through a data-driven process which provides regulatory oversight that helps ensure medical products are safe and effective. In doing so, OTP strives to lead all regulatory decisions with data, impartiality, and compassion.

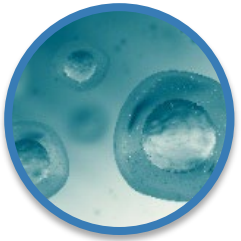
OTP Regulated Products

Devices

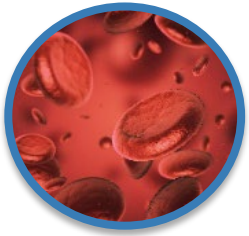
Xenotransplantation Products

Gene Therapies

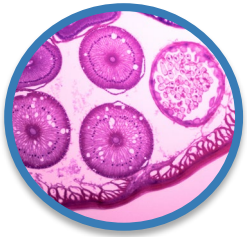
Cell Therapies



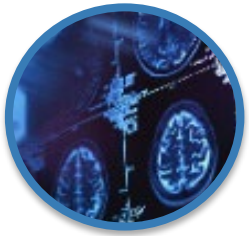
Certain Blood and Plasma Derived Products



Tissues



Combination Products



Therapeutic Vaccines and Cellular Immunotherapies

Image courtesy of Jen Albert, OTP.

OTP Regulated Products: Regenerative Medicine Therapies

OTP also regulates regenerative medicine therapies (RMTs)

- RMTs are defined in the 21st Century Cures Act: Title III, Section 3033, signed into law in 2016
- Regenerative medicine involves using stem cells, engineered biomaterials, gene editing, and other technologies to repair or replace damaged cells, tissues, or organs
- Types of RMTs:

Certain gene therapies (including gene editing)

Cell therapies

Tissues and tissue engineering products

Xenogeneic cell products

Spotlight: Recent Gene Therapy Approvals

On June 29th 2023, FDA approved **Roctavian** for treatment of adults with severe **hemophilia A**.

On December 8th 2023, FDA approved **two** gene therapy products, **Casgevy** and **Lyfgenia**, for treatment of **sickle cell disease** in patients 12 years and older. **Casgevy** is the first FDA-approved gene therapy that uses **genome editing technology**.

On January 16th 2024, FDA approved **Casgevy** for treatment of patients 12 years and up, with **transfusion-dependent beta-thalassemia**, an inherited disorder characterized by life-long anemia requiring frequent blood transfusions. In 2022, FDA approved **Zynteglo,** the first gene therapy for treatment of beta-thalassemia for adult and pediatric patients.

FDA has approved **17 gene therapy products**, most of which are for rare disorders. There are many more rare diseases for which gene therapy holds promise.

Gene Therapy Holds Great Promise

80% of rare diseases have a genetic basis.

OTP currently oversees more than **2,600** active investigational products; **approximately half** of these are gene therapy products.

Patient participation and insight are critical for clinical research.

Spotlight: RegenMedEd Series

- Launched in **November 2021**, OTP created a quarterly webinar series called “Regen-Med-Ed” in an effort to offer more frequent opportunities for patient engagement and education following our inaugural annual workshop in May 2021.
- Goals of the series are:

Discuss foundational information about regenerative medicine therapies, including gene therapy and cell therapy

Explore opportunities to engage with FDA and advance regenerative medicine research and drug development

Hear from FDA, patients, advocates, researchers, and other important stakeholders about their experiences

Spotlight: RegenMedEd Series

"Thank you all for sharing your stories! Thank you also to the FDA for organizing the meeting."

"Thank you all for enlightening us all your experiences. You've inspired me to learn more and do more."

All past RegenMedEd event materials can be found on www.fda.gov/news-events/otp-events-meetings-and-workshops

Have a question or suggestion? Use the hashtag #RegenMedEd to share!

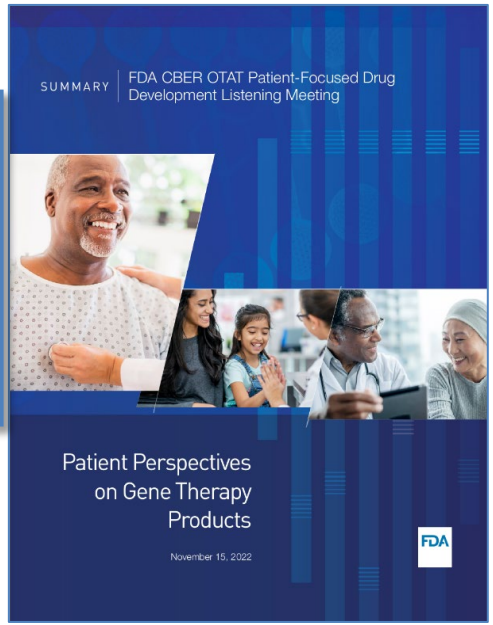
Spotlight: November 2022 Listening Meeting

On November 15, 2022, OTP hosted a virtual patient listening meeting to **gather patient perspectives on gene therapy products** and **provide a forum for their insights and expectations** on risks and benefits of gene therapies, participation in clinical studies, and the landscape of patient experience data.

The founder of Teach RARE, father to a daughter with aromatic L-amino acid decarboxylase (AADC) deficiency, an ultra-rare disorder, spoke about his and his wife's experience caring for their daughter both before and after gene therapy. He **emphasized the importance of early intervention for rare genetic disorders**.

The director of SCID Angels for Life Foundation discussed the urgent need for better treatments for SCID, also known as "bubble boy disease." She **expressed the need for financial incentives to support orphan drug status for new cellular therapies and fast-tracking successful treatments so that patients with rare diseases** such as SCID are not left behind.

A representative of the community supporting aspartylglucosaminuria, an ultra-rare neurodevelopmental disorder, **highlighted that lack of funding and commercial interest in ultra-rare diseases** has made it difficult for patients to access gene therapy. She shared her experience founding the Rare Trait Hope Fund and raising money for the pre-IND (investigational new drug) stage of drug development.

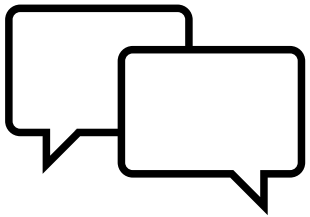


The full summarized report can be found on FDA.gov.

Future Patient Engagement Opportunities

OTP plans to continue consistent engagement and educational events with patient communities including:

- New patient-centric website forthcoming all about gene therapy
- New creative educational materials regarding gene therapies
- Continuing regular webinars and events
- Continuing to foster existing and create new stakeholder group relationships
- And more!



Let us know what you want to see from FDA CBER OTP!

Stay Connected with FDA CBER

- Visit CBER's website
- Sign up for our newsletter, "What's New @ CBER"
- Follow us on social media: @FDACBER on X
- Share your ideas on X/Twitter with #RegenMedEd
- Stay up to date on our events: https://www.fda.gov/news-events/otp-events-meetings-and-workshops

Contact Information

- **Anne Rowzee**

  Anne.Rowzee@fda.hhs.gov

- **Regulatory Questions:**

  OTP Main Line – 240 402 8190

  Email: OTPRPMS@fda.hhs.gov

- **Interactions with Office of Therapeutic Products website:**

  Interactions with Office of Therapeutic Products | FDA

- **OTP Learn Webinar Series:**

  http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm

FDA Headquarters

- **CBER website:** www.fda.gov/BiologicsBloodVaccines/default.htm
- **Phone:** 1-800-835-4709 or 240-402-8010
- **Consumer Affairs Branch**: ocod@fda.hhs.gov
- **Manufacturers Assistance and Technical Training Branch**: industry.biologics@fda.hhs.gov
- **Follow us on X, formerly Twitter:** https://www.twitter.com/fdacber

Thank you!


FDA
U.S. FOOD & DRUG
ADMINISTRATION