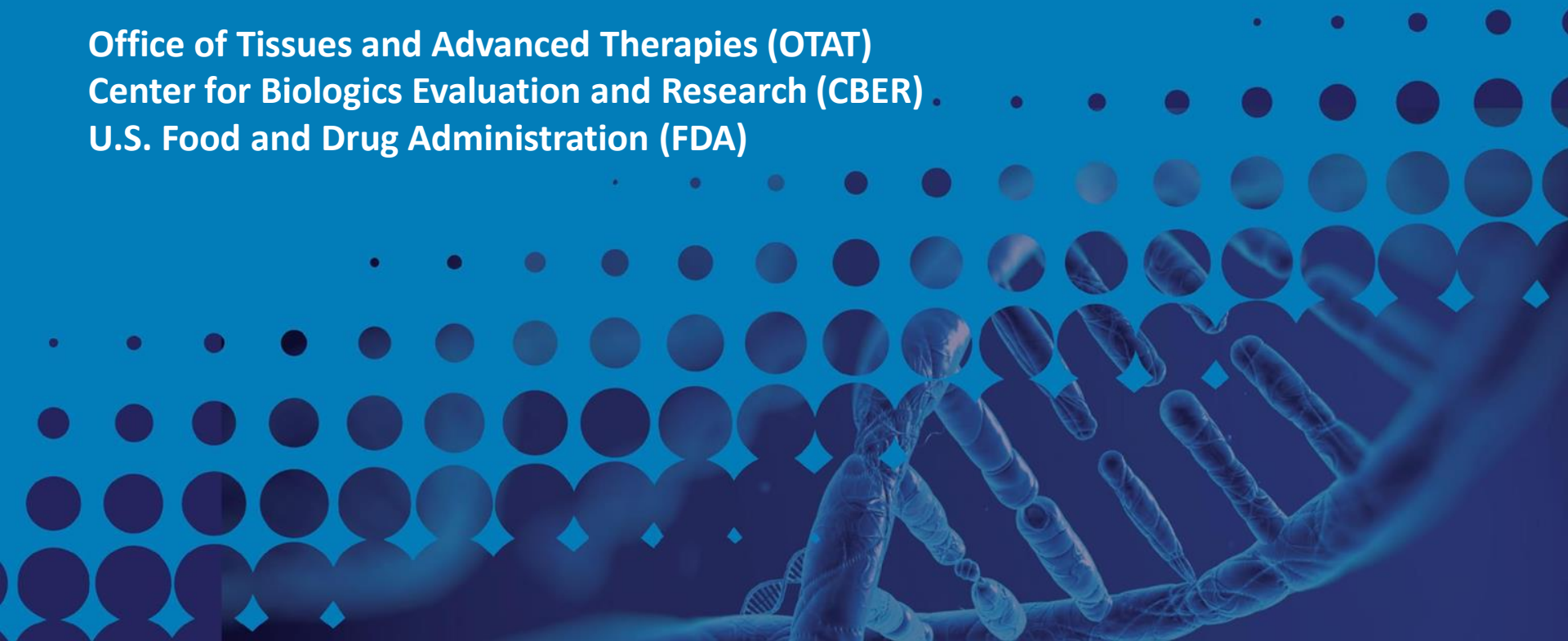
U.S. FOOD & DRUG ADMINISTRATION

Regenerative Medicine 101: Information for Patients, Caregivers & Advocates

Tuesday, November 16, 2021, 11 a.m. – 12 p.m. ET

Office of Tissues and Advanced Therapies (OTAT)
Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration (FDA)



Virtual Meeting Considerations

- The webinar will be recorded and available online after the event
- Use the Q&A box to submit questions throughout the webinar
- For any technical difficulties, please use the chat box

What Is the RegenMedEd Webinar Series?

- OTAT is launching a new webinar series about regenerative medicine
- Goals of the webinar series:

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

Use #RegenMedEd to Share Questions & Ideas

- Do you have a question about regenerative medicine for FDA?
- Do you have an idea for a future RegenMedEd webinar?
- Are you a patient, caregiver, or patient advocate interested in sharing your experience with regenerative medicine?

#RegenMedEd

Regenerative Medicine 101: Information for Patients, Caregivers & Advocates

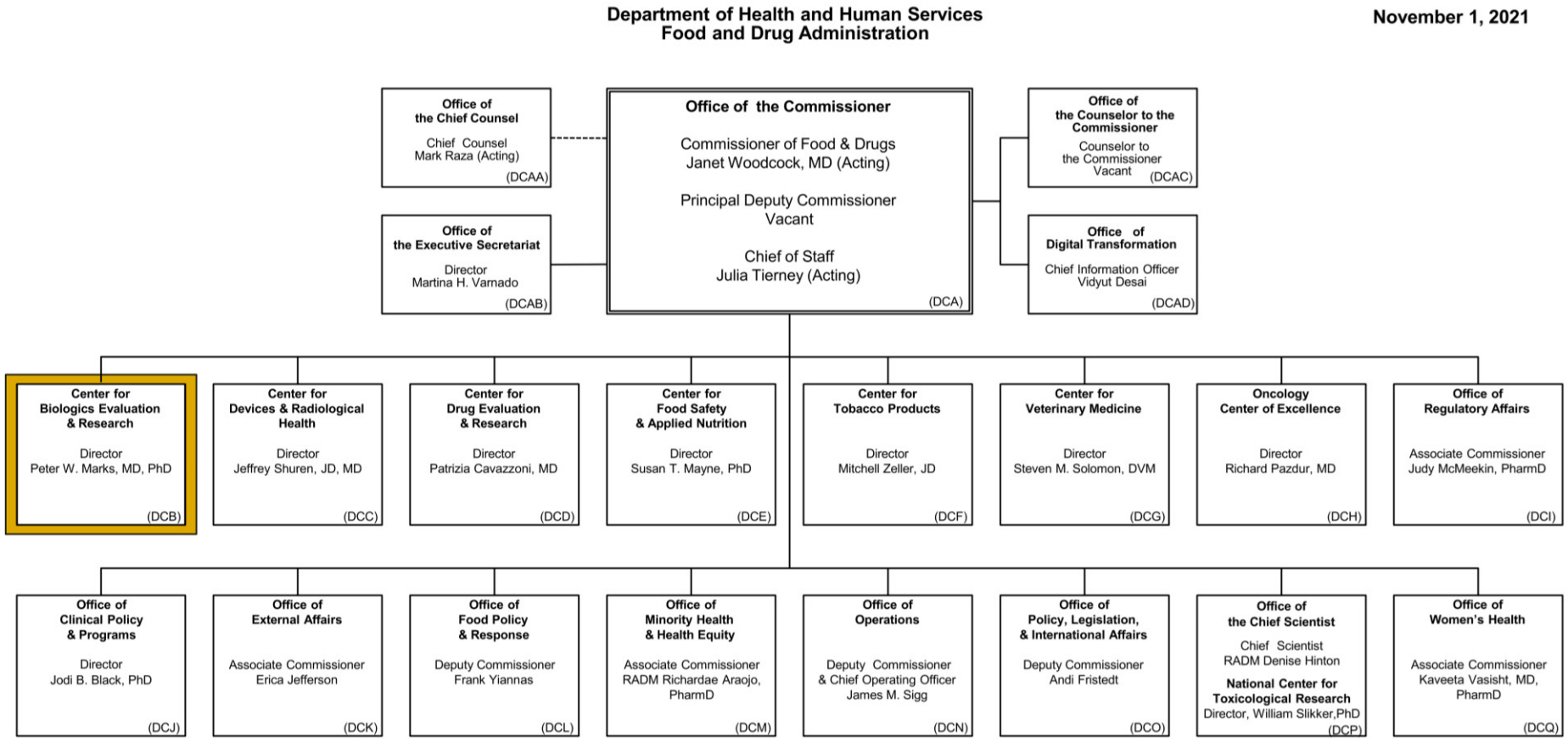
Speaker:
Wilson Bryan, MD
Director
Office of Tissues and Advanced Therapies (OTAT)
Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration (FDA)

Our Mission – FDA

FDA Mission

The Food and Drug Administration is responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and by ensuring the safety of our nation's food supply, cosmetics, and products that emit radiation.

FDA Organizational Structure

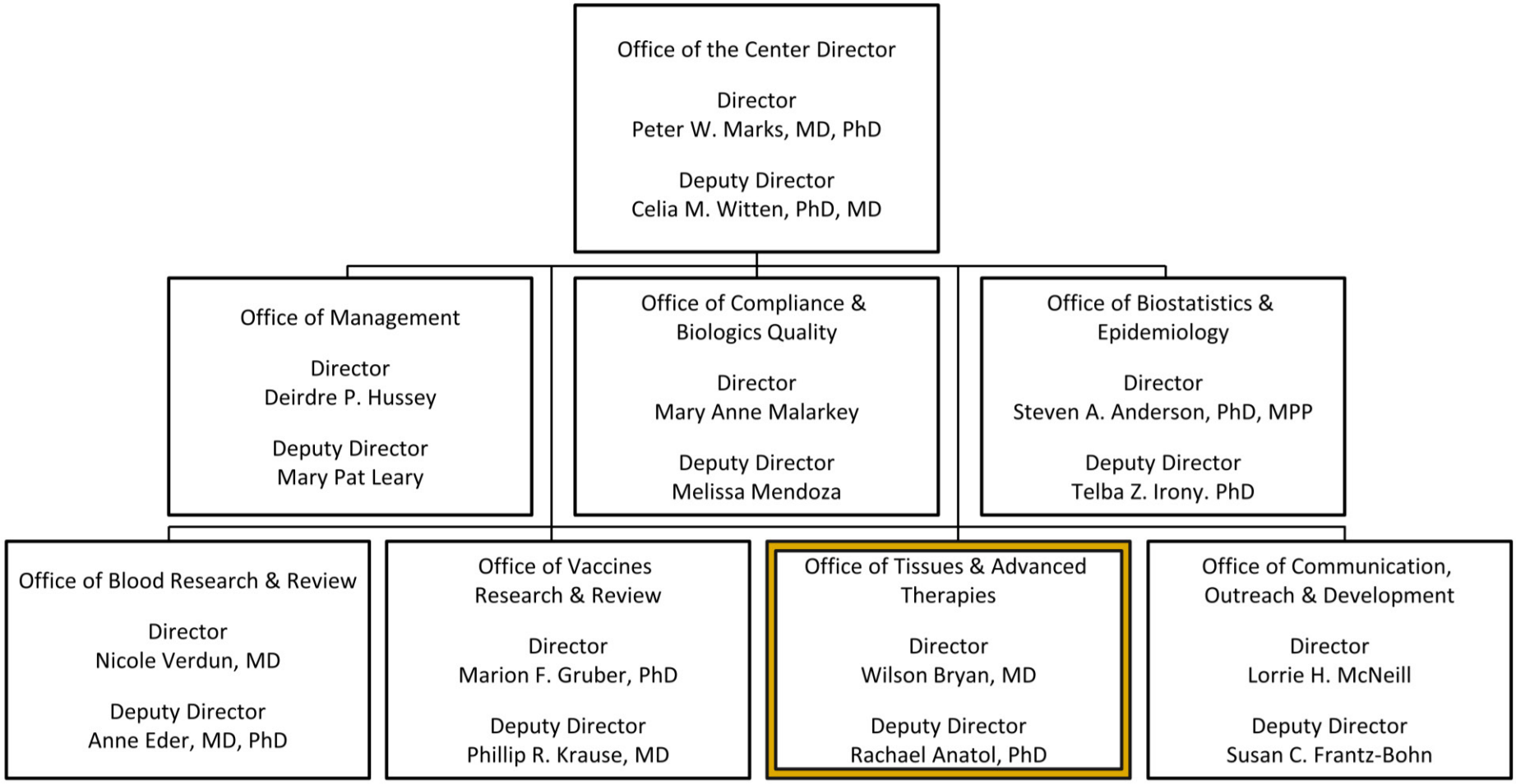


Our Mission – CBER

CBER Mission

Ensure the safety, purity, potency, and effectiveness of biological products including vaccines, allergenics, blood and blood products, cells, tissues, and gene therapies for the prevention, diagnosis, and treatment of human diseases, conditions, or injury.

CBER Organizational Structure



Our Mission – OTAT

OTAT Mission

The Office of Tissues and Advanced Therapies (OTAT) promotes the public health through collaborative, science-based regulation of medical products. This includes facilitating drug development and ensuring safety of individuals. OTAT's regulatory decisions are data-driven, impartial, and compassionate.

Regenerative Medicine Therapies

- OTAT 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:

Gene therapies (including gene editing)

Cell therapies

Tissues and tissue engineering products

Xeno-transplantation products

Diversity of OTAT-Regulated Products

Gene Therapies

- **Gene editing**
- **Ex vivo genetically modified cells**
- **Non-viral vectors** (e.g., plasmids)
- **Replication-deficient viral vectors** (e.g., adenovirus, adeno-associated virus, lentivirus)
- **Replication-competent viral vectors** (e.g., measles, adenovirus, vaccinia)
- **Microbial vectors** (e.g., *Listeria*, *Salmonella*)

Stem Cells/Stem Cell-Derived

- **Adult** (e.g., hematopoietic, neural, cardiac, adipose, mesenchymal)
- **Perinatal** (e.g., placental, umbilical cord blood)
- **Fetal** (e.g., neural)
- **Embryonic**
- **Induced pluripotent stem cells** (iPSCs)

Diversity of OTAT-Regulated Products (Continued)

- **Functionally mature/differentiated cells** (e.g., retinal pigment epithelial cells, pancreatic islets, chondrocytes, keratinocytes)
- **Therapeutic vaccines and other antigen-specific active immunotherapies**
- **Combination products**
  - Engineered tissues/organs
- **Devices**
- **Products for xenotransplantation**

- **Tissues**
- **Blood- and plasma-derived products**
  - Coagulation factors
  - Fibrin sealants
  - Fibrinogen
  - Thrombin
  - Plasminogen
  - Immune globulins
  - Anti-toxins
  - Venom antisera for scorpions, snakes, and spiders

FDA's Role in Regulating RMTs

Regulate products over their entire lifecycle–during development and after approval

Provide oversight of clinical trials to protect patient safety and rights

Advance development by:

- Publishing guidance documents
- Providing advice and education to product manufacturers

Engage stakeholders to facilitate development of innovative products that meet patient needs

Advancing Development: Today, Tomorrow & Beyond

- Gene and cell therapies hold great promise for patients with difficult diseases and few treatment options
- 900+ investigational new drug (IND) applications for ongoing clinical studies for gene and cell therapy treatments
- Gene therapy has great potential for patients with rare diseases:
  - An estimated 80% of rare diseases are caused by a single-gene defect
- New: Bespoke Gene Therapy Consortium (BGTC)
  - Partnership between National Institutes of Health (NIH), FDA, pharmaceutical companies and nonprofit organizations
  - Goal: Accelerate development of gene therapies for the 30 million people in the United States who suffer from a rare disease

To view a list of FDA-approved regenerative medicine therapy products, visit:
www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

Unapproved RMTs Are Dangerous and Illegal

- RMT products must go through clinical trials with FDA oversight and must receive FDA licensure/approval before they can be marketed to consumers
- FDA has received reports of blindness, tumor formation, infections, and more adverse medical events due to the use of unapproved RMTs
- RMTs have **not been approved** for the treatment or prevention of:
  - COVID-19
  - Autism
  - Blindness
  - Pain
  - Fatigue
  - Any neurological disorder (e.g., Alzheimer's disease, ALS, epilepsy)
  - Any cardiovascular or lung disease (e.g., heart disease, COPD)
  - Any orthopedic conditions (e.g., knee pain, tendonitis)

FDA's Actions to Prevent Unapproved RMT Marketing

- FDA has repeatedly notified, warned, and taken legal enforcement actions against manufacturers, clinics, and individuals administering unapproved RMTs

- Contact FDA at ocod@fda.hhs.gov if:
  - You are being offered any RMT products outside of a clinical trial for which FDA has oversight
  - You are considering treatment with an RMT product
  - You have been treated with an RMT product and wish to report any adverse effects or file a complaint

OTAT Patient Engagement: To Learn From Patients

Impact of disease and treatment	• Chief complaints (e.g., most bothersome signs/symptoms) • Burden of living with and managing a disease or condition • Impacts on activities of daily living and functioning
Perspectives about current and potential treatments	• Expectations of benefits • Tolerance for harms or risk • Preferences when receiving treatment • Unmet medical needs
Clinical trial considerations	• Burden of participating in clinical studies • Known and unknown risks of participating in clinical studies • Designing clinical studies to be better tailored for patients and caregivers

How Does OTAT Receive Patient Input?

- Advisory Committee Meetings
- Special Government Employee Consultants
- Public Meetings and Workshops
- Patient-Focused Drug Development Meetings
- FDA/NORD Rare Disease Listening Sessions
- Meetings with Patient Organizations

Enhancing Collaboration

There are many ways stakeholders can work together to advance regenerative medicine.

FDA encourages:

- Drug developers to invite patients/advocates to their meetings with OTAT
- Patient groups to take on translational science activities, such as natural history studies and patient registries
- Patient groups to work together
- Groups to begin collaboration early

Helpful Resources

- **Visit the CBER website:** http://www.fda.gov/vaccines-blood-biologics
- **Follow us on Twitter:** @FDACBER
- **Get email updates:** https://public.govdelivery.com/accounts/USFDA/subscriber/new
- **Find a list of approved gene and cell therapy products:** https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products
- **Visit the "What is Gene Therapy?" webpage for consumers:** https://www.fda.gov/consumers/consumer-updates/what-gene-therapy-how-does-it-work

Q&A Session

Please type your questions into the Q&A box.

#RegenMedEd

Thank you!

Webinar materials will be available in a few weeks on FDA.gov.

Stay tuned for our next webinar to be held in Winter/Spring 2022!