

OTAT's Stakeholder Outreach and Patient Education Program:

Bringing the Patient Voice to the Forefront

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Overview and Outline

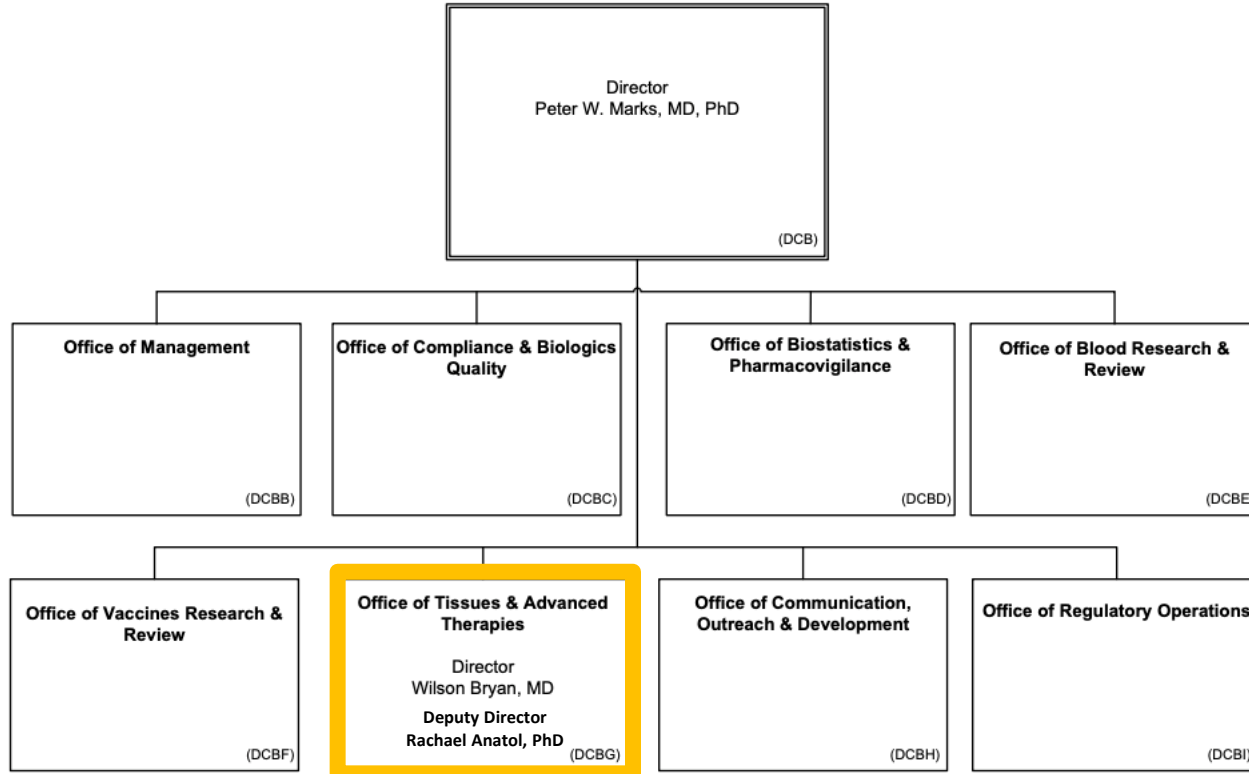


- Introduction to OTAT and regenerative medicine
- Overview of OTAT's stakeholders
- Focus on patient education and outreach in the regenerative medicine space

Learning Objectives

- Describe OTAT's stakeholders
- Illustrate the purpose and value of patient engagement for advancing regenerative medicine therapies
- List the various initiatives OTAT is leading and/or participating in to engage with patients and gather patient input and feedback

CBER Organizational Structure



Our Mission – OTAT



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
- FDA interpretation includes gene therapies that lead to sustained effects, and certain xenogeneic cell products
- Types of RMTs:



Cell therapies



Tissues and
tissue
engineering
products



Gene Therapy
(including gene
editing)



Xenogeneic cell
products

The 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:

- Providing advice and education to product developers, including academicians and industry



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

OTAT's Stakeholders

OTAT's Stakeholders



Academic Groups



**Trade Organizations
& Public, Private
Partnerships**



**Patients & Advocacy
Organizations**

Work with Academic Stakeholders



- Help to organize & provide regulatory perspective for workshops
- Participate in working groups and on steering committees related to emerging scientific topics

Work with Trade Organizations & PPPs



- Attend annual liaison meetings with trade organizations to hear positions on high-priority topics
- Conduct listening sessions with product sponsors and trade organizations to expedite advances in cell and gene therapy
- Public Private Partnerships



Upcoming outreach efforts



- Updating and consolidating web-based resources related to RMTs
- Updating content on OTAT Learn to support productive engagement with sponsors
- Collaborate with trade organizations to facilitate mutual learning on high- priority topics
- Fall 2022 Public meetings
 - Methods and approaches for capturing post-approval safety and efficacy data
 - Patient perspectives on gene therapy products



**Patients & Advocacy
Organizations**

The Critical Role of Patients in Advancing Regenerative Medicine

What is Patient Engagement?



Activities that involve patient stakeholders sharing their **experiences, perspectives, needs, and priorities**



Patient engagement helps to inform the **FDA's public health mission**



Overall goal: **Learn directly from patients**



Why Does Patient Engagement Matter?



Patients are at the heart of the FDA's work



Patients are experts in their own experience of their disease or condition.



Patient input can inform medical product development and enhance regulatory decision-making to address patients' needs.

How Does OTAT Receive Patient Input?



Advisory Committee Meetings

During development as SGE consultant



Public meetings, webinars, and workshops

Patient-Focused Drug Development Meetings



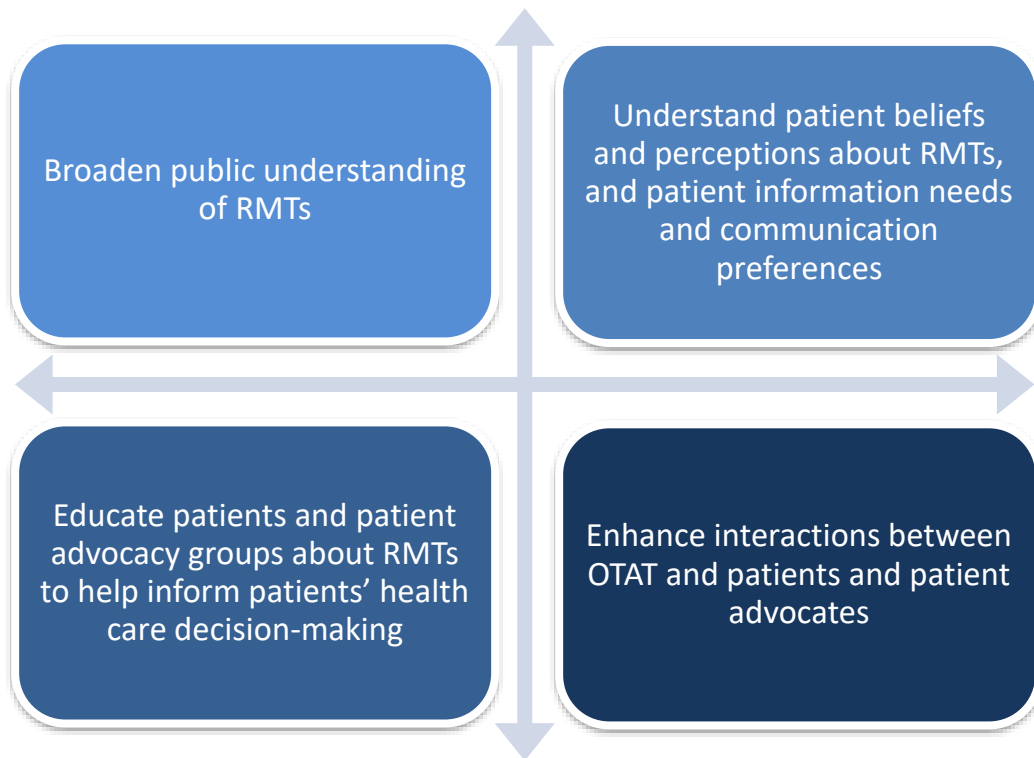
Meetings with patient organizations

FDA/NORD Rare Disease Listening Sessions



OTAT's RMT Stakeholder Outreach & Education Campaign

RMT Stakeholder Outreach & Patient Education Campaign



Spotlight: OTAT's Annual Patient Engagement Workshop



Virtual half-day event held in Spring



Bring together patients, caregivers, advocates, and others to discuss ways to work with the FDA to help advance regenerative medicine



Spotlight: OTAT's RegenMedEd Webinars



1-hour virtual, public webinars held quarterly



Discuss a specific topic related to regenerative medicine and patient education; feature patients and caregivers and their stories

The graphic is a promotional banner for an FDA webinar. On the left, it features the FDA logo, the word "Webinar" in blue, the title "The Critical Role of Patients in Advancing Gene Therapy Treatments for Rare Diseases", the date and time "March 9, 2022 | 11:00 AM – 12:00 PM EST", and a call to action "Join FDA in recognizing the contributions of patients in advancing treatments for rare diseases." Below this is the hashtag "#RegenMedEd" and a Twitter icon. On the right, there are three circular photographs: a young girl in a pink shirt, a family of three, and a young man.

FDA

Webinar

The Critical Role of Patients in Advancing Gene Therapy Treatments for Rare Diseases

March 9, 2022 | 11:00 AM – 12:00 PM EST

Join FDA in recognizing the contributions of patients in advancing treatments for rare diseases.

#RegenMedEd

Spotlight: Meetings With Patient Groups



Types of work products that patient organizations could submit to the FDA

Meeting reports summarizing patient perspectives on disease and treatment burden

Methodologically-sound patient surveys

White papers or peer-reviewed journal articles describing topics such as background on disease and considerations for clinical trials in a disease area

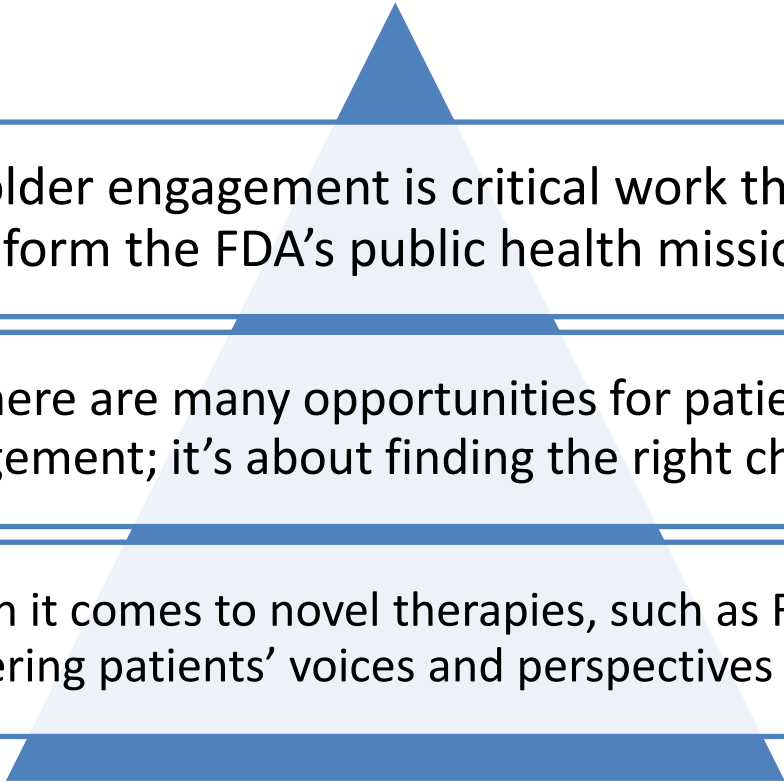
Natural history study report

Proposed draft guidance relating to patient experience data

Resources

- Follow [CBER on Twitter](#) for the latest information about webinars, workshops, news
- Find [past webinars and workshops](#)
- Visit our [Cell and Gene Therapy web pages](#) for more information

Summary



Stakeholder engagement is critical work that helps inform the FDA's public health mission

There are many opportunities for patient engagement; it's about finding the right channel

When it comes to novel therapies, such as RMTs, centering patients' voices and perspectives is key

CE Question 1



Which of the options below would NOT be considered a relevant type of patient input for the FDA?

- A. Patient perspectives on the most burdensome/difficult symptoms of a disease
- B. Patient perspectives on risk tolerance of a potential treatment
- C. Patient perspectives on the risks of participating in a clinical trial
- D. Patient perspectives on drug pricing

CE Question 2



True or False: OTAT's RegenMedEd Webinar series is held annually.

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- **OTAT Learn Webinar Series:**
<http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm>
- **CBER website:** www.fda.gov/BiologicsBloodVaccines/default.htm
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