

# Patient Engagement

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Innovation in Medical Product Development – May 29, 2024



# Learning Objectives

- Define patient engagement
- Describe how CBER considers patient input in product review
- Locate FDA patient engagement resources
- Integrate patient engagement strategies across the drug development process

# Patient Engagement



Activities that involve patient stakeholders sharing their experiences, perspectives, needs, and priorities that help inform FDA's public health mission.

[Patient-Focused Drug Development Glossary | FDA](#)

# Engaging with patients...

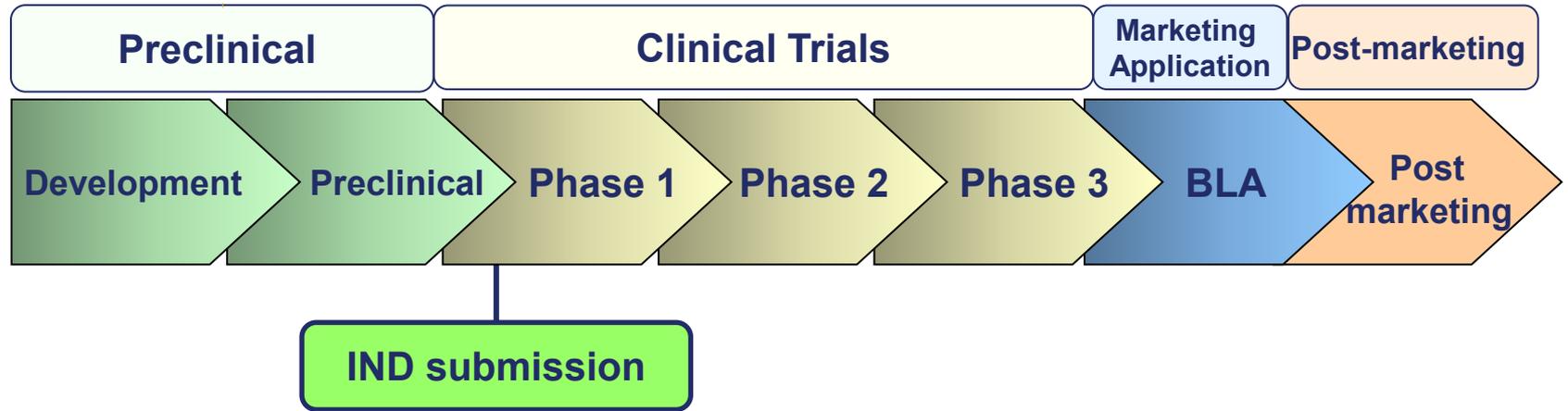
- Impact of the disease and its treatment
- Perspectives about potential and current treatments
- Views on unmet medical needs and available treatment options
- Enhance the understanding of disease natural history

# When to engage with patients

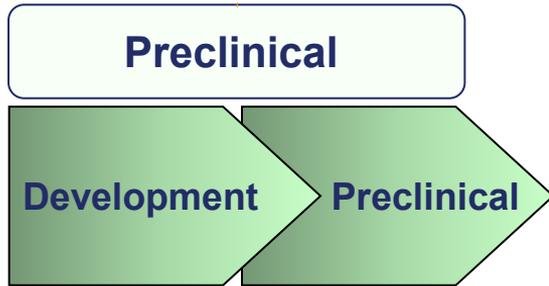


Throughout the product development process

# Drug Development Process

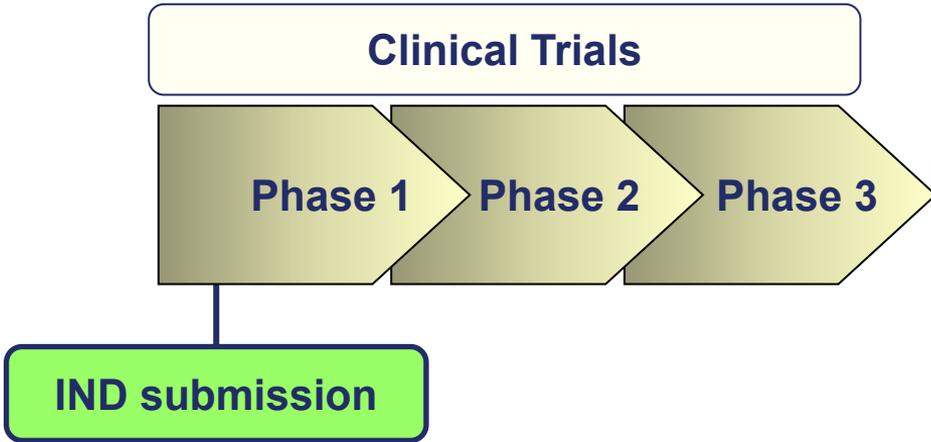


# Preclinical



What impacts  
(burden of disease  
and burden of  
treatment) matter  
most to patients  
and how do we  
measure them?

# Clinical Trials



What aspects of clinical trials can be better tailored to meet the needs of patients who (might) participate in the trial?

# Marketing Application

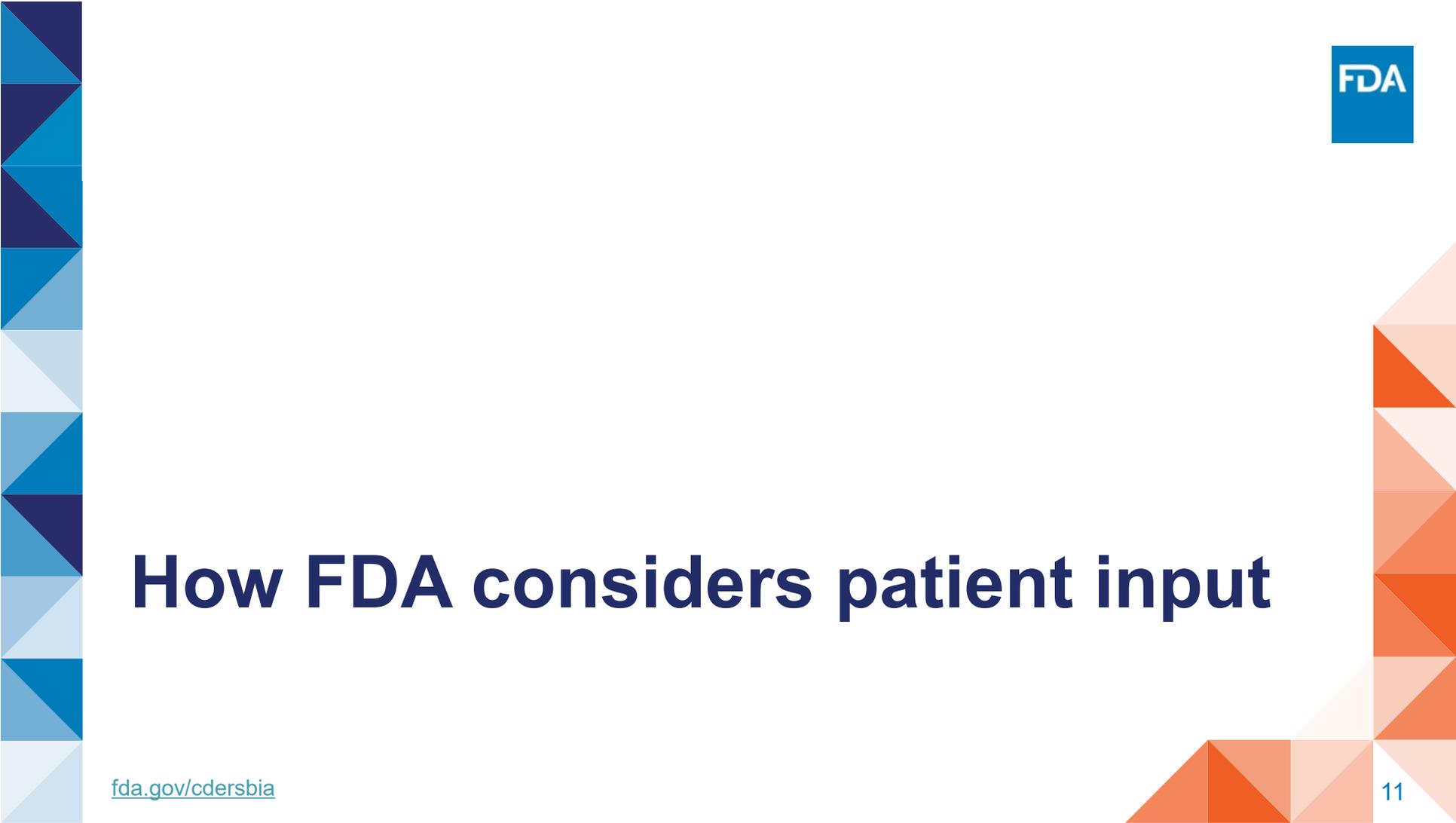


Patient reported  
outcome data or  
elicited patient  
preferences inform  
Benefit-Risk (BR)  
assessments

# Postmarketing



How do we best communicate information to patients and prescribers?

The slide features decorative geometric patterns. On the left, a vertical bar consists of a series of triangles in various shades of blue and dark blue. On the right, a larger, more complex pattern of triangles in shades of orange and light orange is visible. The main content area is white with the title text centered.

# How FDA considers patient input

# BLA Clinical Review Memo

Clinical Reviewer:  
STN:

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### Data Submitted in the Application

Check if Submitted	Type of Data	Section Where Discussed, if Applicable
<input type="checkbox"/>	Patient-reported outcome	
<input type="checkbox"/>	Observer-reported outcome	
<input type="checkbox"/>	Clinician-reported outcome	
<input type="checkbox"/>	Performance outcome	
<input type="checkbox"/>	Patient-focused drug development meeting summary	
<input type="checkbox"/>	FDA Patient Listening Session	
<input type="checkbox"/>	Qualitative studies (e.g., individual patient/caregiver interviews, focus group interviews, expert interviews, Delphi Panel)	
<input type="checkbox"/>	Observational survey studies	
<input type="checkbox"/>	Natural history studies	
<input type="checkbox"/>	Patient preference studies	
<input type="checkbox"/>	Other: (please specify)	
<input type="checkbox"/>	<b>If no patient experience data were submitted by Applicant, indicate here.</b>	
Check if Considered	Type of Data	Section Where Discussed, if Applicable
<input type="checkbox"/>	Perspectives shared at patient stakeholder meeting	
<input type="checkbox"/>	Patient-focused drug development meeting	
<input type="checkbox"/>	FDA Patient Listening Session	
<input type="checkbox"/>	Other stakeholder meeting summary report	
<input type="checkbox"/>	Observational survey studies	
<input type="checkbox"/>	Other: (please specify)	

# Patient Engagement Case Studies

The Applicant did not provide a patient experience report for the subjects enrolled in UIH-001 or UIH-002. The Applicant did, however, include testimonials from subjects who participated in the studies during the April 15, 2021 Advisory Committee Meeting.

### 1.2 Patient Experience Data

The Applicant did not provide a patient experience report for the subjects enrolled in UIH-001 or UIH-002. The Applicant did, however, include testimonials from subjects who participated in the studies during the April 15, 2021 Advisory Committee Meeting.

The FDA Science of Patient Input, Office of Biostatistics and Epidemiology (OBE) group collaborated with UCSF on a project for patient preference in islet cell therapy. The group presented a poster “Preferences of those with Type 1 Diabetes for risks and benefits of islet cell transplantation: A discrete choice experiment to inform regulatory approval” at the FDA Science Forum (2021). The authors conclusion was that their study “suggests that hard-to-control T1DM patients may be willing to accept a certain level of risk (e.g. 5% risk of serious complications) to achieve a certain extent of benefit (the possibility of having 5+ years of insulin independence).”

#### Data Submitted in the Application

Check if Submitted	Type of Data
<input type="checkbox"/>	Patient-reported outcome
<input type="checkbox"/>	Observer-reported outcome

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## 1.2 Patient Experience Data

Patient experience data relevant to this submission are summarized in Table 2.

Table 2. Patient Experience Data Relevant to This Application

Check if Submitted	Type of Data	Section Where Discussed, if Applicable
<input checked="" type="checkbox"/>	Patient-reported outcome	6.1.8, Endpoints and Criteria for Study Success
<input checked="" type="checkbox"/>	Observer-reported outcome	6.1.2, Design overview 6.1.8, Endpoints and Criteria for Study Success
<input type="checkbox"/>	Clinician-reported outcome	
<input type="checkbox"/>	Performance outcome	

<input type="checkbox"/>	FDA Patient Listening Session	
<input checked="" type="checkbox"/>	Other stakeholder meeting summary report	2.1, Disease or Health-Related Condition(s) Studied
<input type="checkbox"/>	Observational survey studies	
<input type="checkbox"/>	Other: (please specify)	

Applicable

6.1.8, Endpoints and Criteria for Study Success

6.1.2, Design overview  
6.1.8, Endpoints and Criteria for Study Success

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Check if Submitted	Type of Data
<input checked="" type="checkbox"/>	Patient-reported outcome
<input checked="" type="checkbox"/>	Observer-reported outcome
<input type="checkbox"/>	Clinician-reported outcome
<input type="checkbox"/>	Performance outcome

As part of the FDA Externally Led Patient-Focused Drug Development initiative, on April 6, 2018, a joint public meeting led by Pachyonychia Congenita (PC) Project and the Dystrophic Epidermolysis Bullosa Research Association of America (DEBRA) (Pachyonychia Congenita Project 2018) was held.

The following pertinent questions were asked during the meeting with the responses gathered from attendees (in person and online):

Are you participating in a clinical trial?

<input type="checkbox"/>	FDA Patient Listening Session	
<input checked="" type="checkbox"/>	Other stakeholder meeting summary report	2.1, Disease or Health-Related Condition(s) Studied
<input type="checkbox"/>	Observational survey studies	
<input type="checkbox"/>	Other: (please specify)	

On June 15, 2022, DEBRA held a listening session with FDA. Patients with DEB and their caregivers shared their perspectives of the disease that mattered most to them. The representatives from DEBRA stated that any wound area reduction or pain reduction would be considered important to them.

# Atidarsagene autotemcel



STN: 125758

## Reviewer comments on patient experience data

In addition to the clinician-reported outcome (ClinRO) and performance-based outcome (PerfO) evidence submitted, this reviewer also considered the following sources of PED:

1. Externally-led Patient-Focused Drug Development Voice of the Patient reports:

- a. Cure MLD (2022). Metachromatic Leukodystrophy (MLD) Voice of the Patient Report, October 21, 2022 and November 18, 2022. Accessed from: [\[link\]](#) (linking yes/no). Slowing disease progression and increasing responsiveness for patients were reported as valued aspects of a treatment for MLD. These PED further emphasize the substantial unmet treatment need and the importance of neurocognitive preservation as a treatment outcome for patients of all MLD subtypes.

2. Patient-Centered Outcomes Research Institute (PCORI) reports:

- a. Harrington, M., Whalley, D., et al. (2019). Caregiver-reported outcomes of metachromatic leukodystrophy from interviews with caregivers. Orphanet J Rare Dis 14, 89 (2019).

These additional PED helped confirm the importance of preserving cognitive functioning for all MLD patients but, in particular, for juvenile patients for whom cognitive symptoms may be an early emerging symptom. In the MLD Voice of the Patient report, caregivers indicated a top concern was decreased communication/responsiveness for MLD patients and the report described patients as “locked in” which is assumed to mean complete dependence on caregivers and no communication abilities, even minimally (e.g., blinking yes/no). Slowing disease progression and increasing responsiveness for patients were reported as valued aspects of a treatment for MLD. These PED further emphasize the substantial unmet treatment need and the importance of neurocognitive preservation as a treatment outcome for patients of all MLD subtypes.

# Hemophilia

*“This valuable input has already been used in ways that help advance overall development of gene therapy products for hemophilia.”*

[How FDA is Putting the Patient Voice at the Forefront of Gene Therapy Clinical Trials for Hemophilia | FDA](#)

Following the listening session:

- Agenda for “Product Development in Hemophilia” public workshop
- [Public summary](#) is a resource
- Reinforced public comments on the [“Human Gene Therapy for Hemophilia”](#) draft guidance

# Challenge Question #1



**In which ways can patient engagement NOT help your biologic development program?**

- A. Developing clinical outcome assessments
- B. Tailoring clinical trial protocols to patients' needs
- C. Persuading FDA to ignore your missed endpoints
- D. Understanding patients' expected benefits and tolerance for risks

# Patient Engagement Resources

# Patient Engagement Programs



- Patient-focused drug development meetings
- Listening sessions
- RegenMedEd
- Webinars and Workshops

# Patient Engagement Programs



**Reports, recordings,  
and transcripts  
are available to you!**

# Voice of the Patient and Listening Session Reports

[Condition-Specific Meeting Reports and Other Information Related to Patients' Experience | FDA](#)

## Condition-Specific Meeting Reports and Other Information Related to Patients' Experience

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This webpage provides links to certain publicly available external reports and resources relating to patient experience data. The patient community, patient advocates, researchers, drug developers, and federal agencies may find these materials useful. For specific questions related to a report or resource, FDA recommends reaching out to the point of contact listed on the cover page of the report.

This webpage hosts an alphabetical listing of condition-specific meeting reports and other information related to patients' experience. These meetings include [FDA-led Patient-Focused Drug Development \(PFDD\) meetings](#), [Externally-led PFDD meetings](#), and [Patient Listening Sessions](#). Other information includes proposed draft guidance relating to patient experience data, natural history studies, or other condition-specific background on condition and discussion of unmet medical need.

FDA's Office of Patient Affairs is responsible for managing the Agency's Patient Listening Session program. While conditions for which there were Patient Listening Sessions conducted are listed individually on this page, the link will direct you to the Patient Listening Session page where you will be able to see all the Patient Listening Session Summaries posted by the Office of Patient Affairs.

For more information regarding what types of resources may be included on this webpage, how to submit a publicly available website link to FDA, and other general questions, please

# RegenMedEd Series



## [OTP Events, Meetings, and Workshops | FDA](#)

### OTP Events, Meetings, and Workshops



The Office of Therapeutic Products (OTP) hosts a variety of events to share information about OTP-regulated products and bring together important stakeholders to discuss the development and advancement of these products. Learn more about our various event series, register for our upcoming events, or find information about previous events.

#### Learn More About Our Events

##### RegenMedEd Series

The RegenMedEd series brings together important stakeholders and FDA staff to discuss foundational information about regenerative medicine therapies, such as gene and cell therapy products, and explore opportunities for patients, caregivers, and advocates to engage with FDA to help advance product development. Recent topics include gene therapy clinical trials, the importance of natural history studies, and regenerative medicine 101.

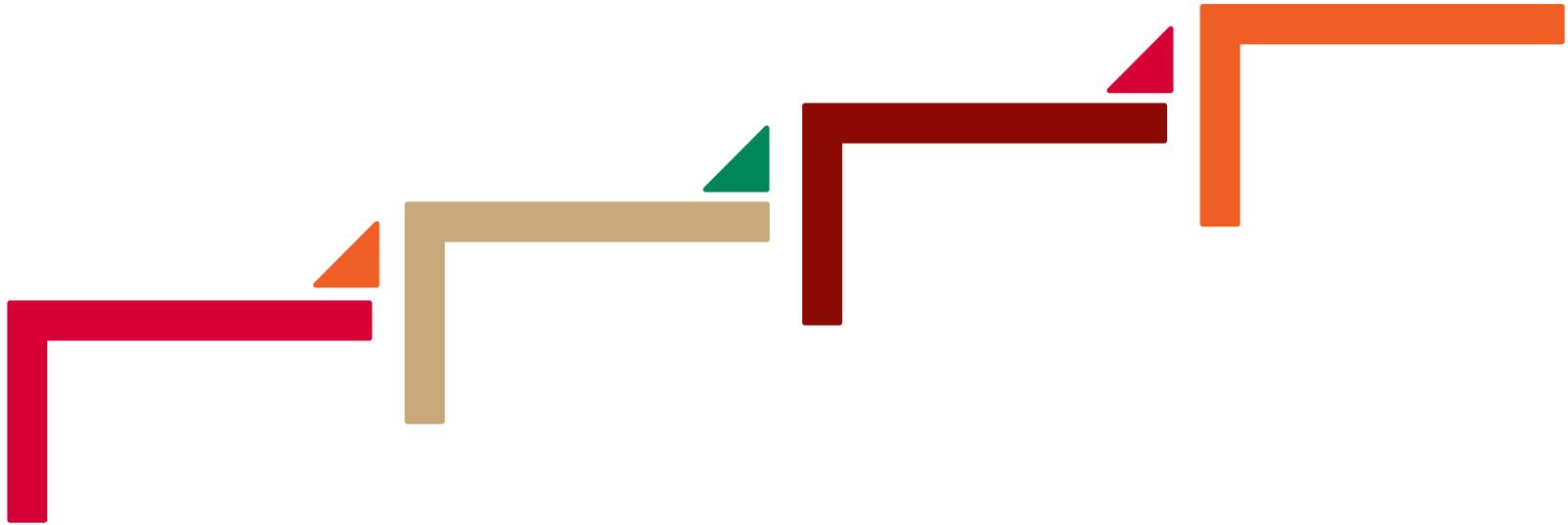
##### OTP Virtual Town Hall Series

The OTP Virtual Town Halls aim to engage with product development stakeholders and researchers. These town halls have a question-and-answer format with the goal of providing regulatory information to stakeholders to help advance development of OTP-regulated products.

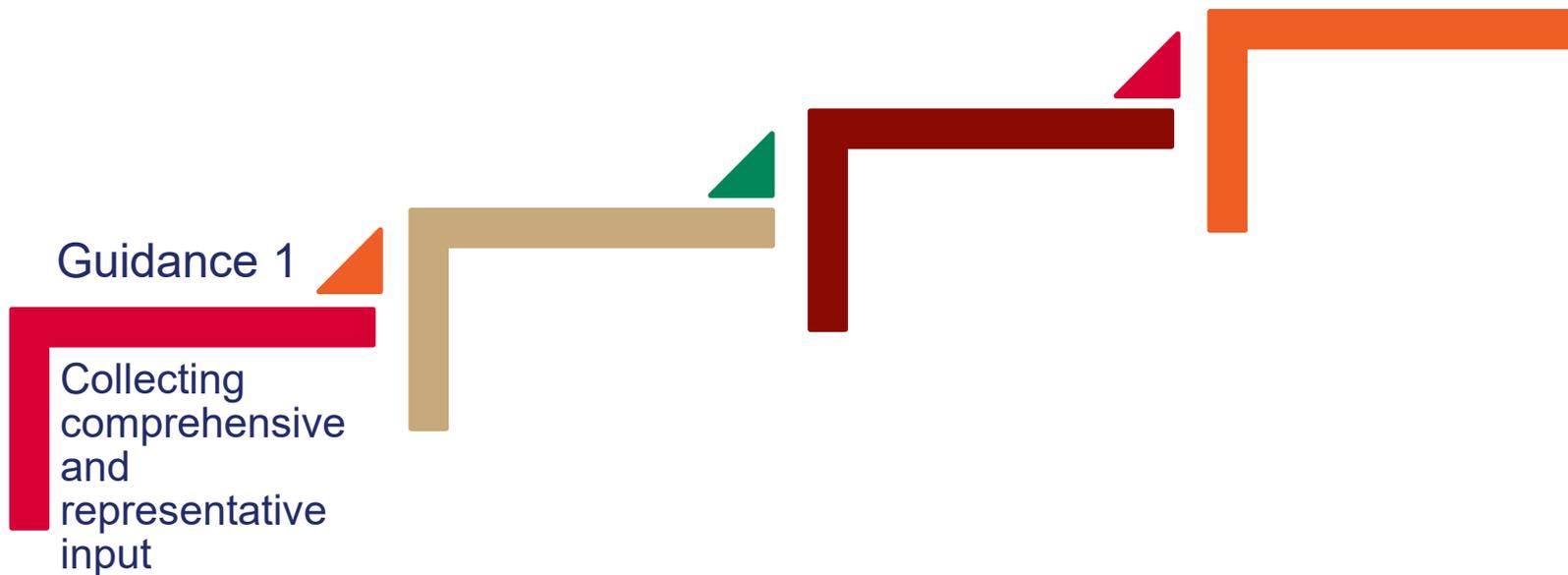
##### Additional Workshops, Meetings, and Events

In addition to our event series, OTP also hosts a number of other meetings and workshops throughout the year. Most of these events are held virtually and are free and open to the

# Patient-Focused Drug Development Guidances



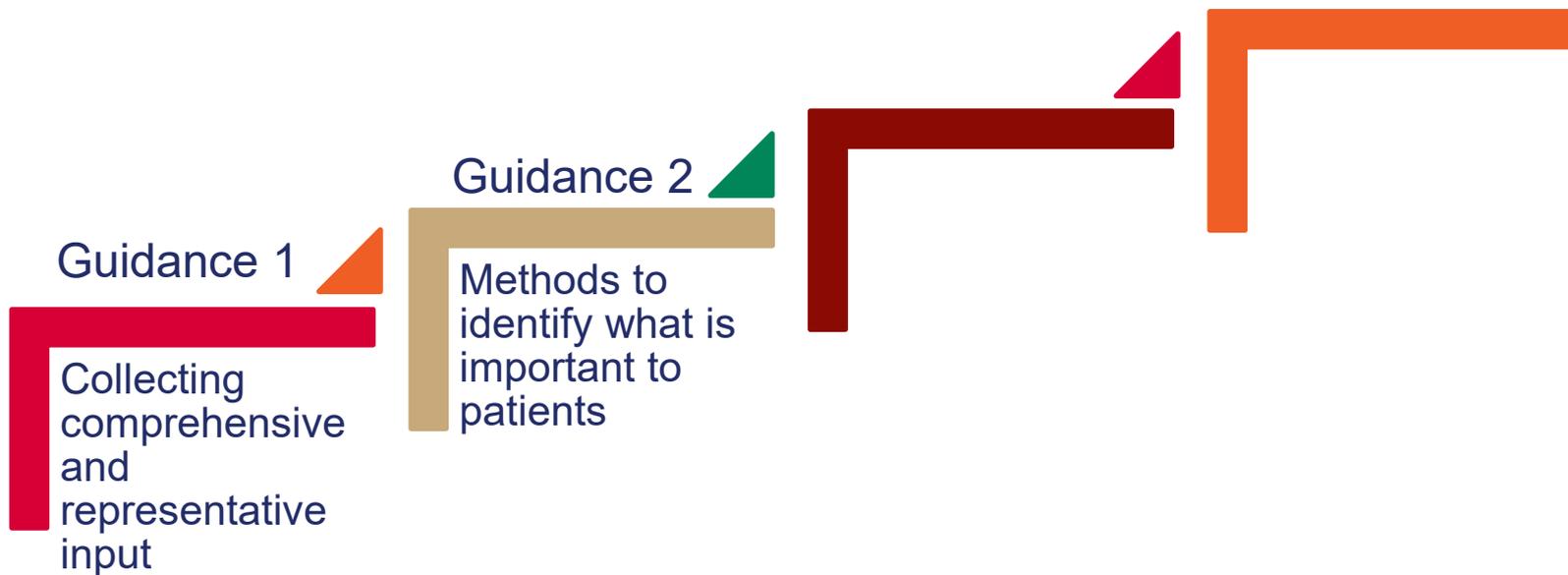
# Patient-Focused Drug Development Guidances



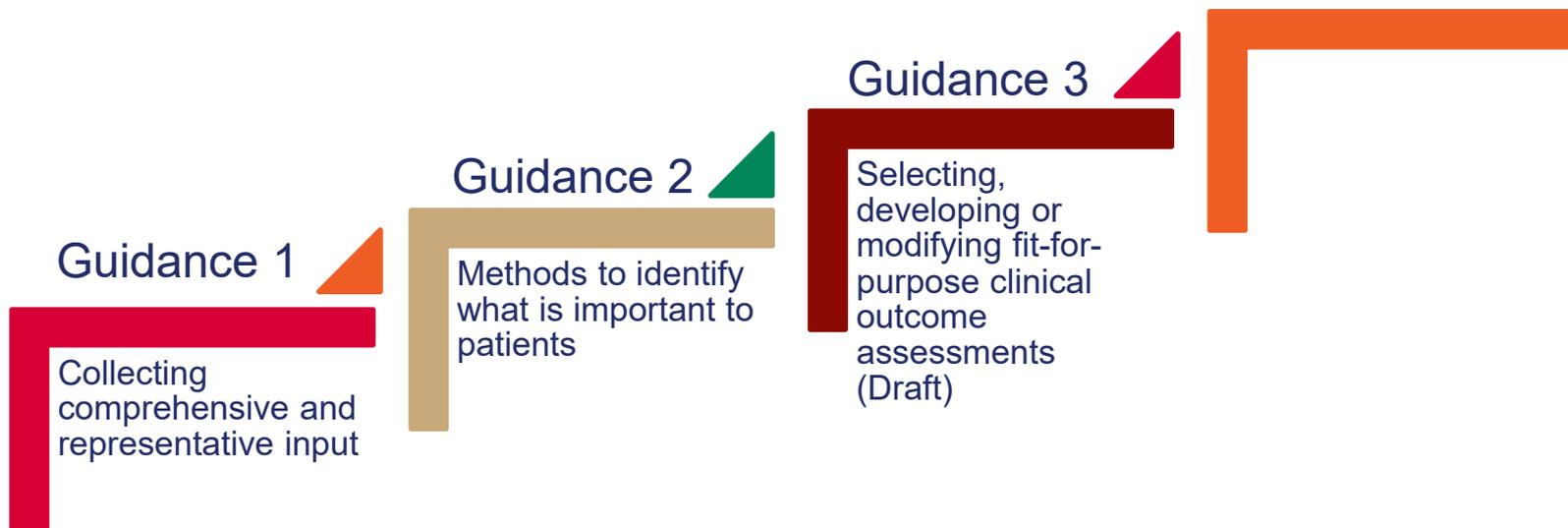
Guidance 1

Collecting  
comprehensive  
and  
representative  
input

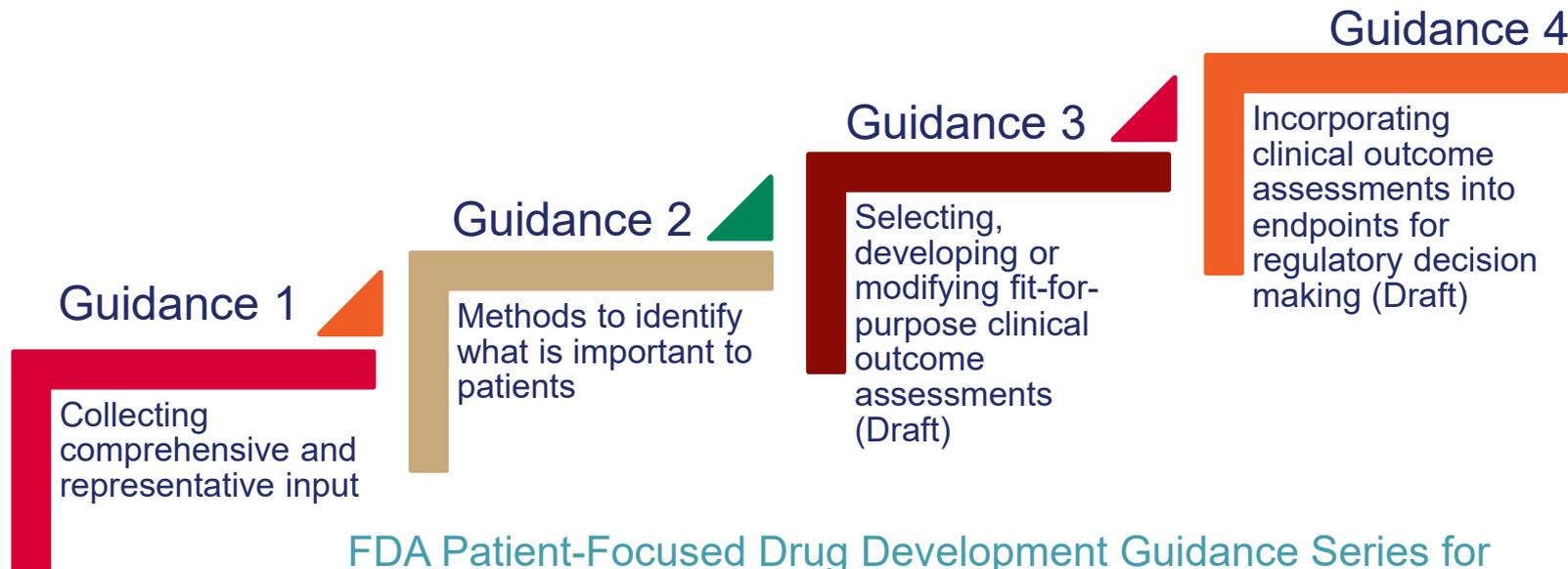
# Patient-Focused Drug Development Guidances



# Patient-Focused Drug Development Guidances



# Patient-Focused Drug Development Guidances



[FDA Patient-Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making | FDA](#)

# Challenge Question #2



## What patient engagement resources are available on the FDA website?

- A. A list of almost 200 reports and resources related to patient experience data
- B. A matchmaking service for drug developers and patient groups who want to collaborate
- C. Step-wise guidance on collecting and submitting patient experience data
- D. Publicly available documentation of how FDA considers patient input

# For more information:

- [Center for Biologics Evaluation and Research Patient Engagement Program | FDA](#)
- [OTP Events, Meetings, and Workshops | FDA](#)
- [Biological Approvals by Year | FDA](#)
- Sign up for the [CBER listserv](#) or follow us on [social media](#)

# Summary



- Patients are the experts in what it is like to live with their disease.
- Patient engagement is the first step in a patient-focused drug development program.
- FDA has resources to guide you

# Closing Thought

Engage with patients early,  
engage with patients often.