



# **Incorporating the patient voice into clinical trial design: More than just checking the box**

**Clinical Outcome Assessment Program Annual Meeting  
April 16-17, 2026  
Washington, D.C.**

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- **Moderator**

- *Elizabeth (Nicki) Bush, MHS* - Executive Director and Global Head, Endpoint Strategy and COA Measurement, OPEN Health

- **Presenters**

- *Pujita Vaidya, MPH* - Director, Regulatory Science & Policy, GRA, Sanofi
- *Amanda Decoker, MA* - Head, Patient Engagement, Experience and Recruitment, Takeda

- **Additional Panelists**

- *Samantha Parker, MBA* - Vice Chair International Rare Disease Research Consortium, Patient Engagement Lead, Italfarmaco
- *Dominique Pichard, MD, MS* - Chief Scientific & Medical Officer, International Rett Syndrome Foundation
- *Cynthia (Cyndi) Grossman, PhD* - Director, Division of Patient-Centered Development, Center for Devices and Radiological Health (CDRH), U.S. Food and Drug Administration

# Session Agenda



- Overview of session (5 minutes)



- Level-setting presentations (25 minutes)



- Panel discussion (25 minutes)



- Open discussion and Q&A (20 minutes)

# Session Objectives

- Discuss strategic considerations around enhancing patient-focused medical product development
- Share practical considerations for executing patient engagement in clinical research
- Provide regulatory and patient perspectives related to incorporating the patient voice into clinical trial design

# Introduction



# Enhancing Patient-Focused Medical Product Development: Strategic Considerations

Pujita Vaidya, MPH

Director, Regulatory Science and Policy, GRA

Sanofi

# Regulatory Landscape: Global Regulatory Efforts to Advance Patient-Focused Drug Development (PFDD)

*PFDD is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into medical product development and evaluation - FDA*

FDA (US)	EMA (EU)	MHRA (UK)	PMDA (Japan)	NMPA (China)
<ul style="list-style-type: none"> <li>• FDA-led PFDD Meetings</li> <li>• Externally-led PFDD meetings</li> <li>• FDA Patient Listening sessions</li> <li>• Series of PFDD Guidances</li> <li>• CDRH Patient Preference initiative</li> <li>• CDER Patient Preference guidance</li> </ul>	<ul style="list-style-type: none"> <li>• Engagement Framework</li> <li>• EMA PED multi-stakeholder workshop</li> <li>• Reflection paper on the best EU approach to generate, collect and analyze PED</li> <li>• Improve transparency in the Assessment Report.</li> </ul>	<ul style="list-style-type: none"> <li>• Pilot program to ensure that pharma companies and research teams harness the power of the patient voice.</li> <li>• Submissions for new active substances/ indications are received, the applicant company is expected to show evidence of the ways in which patients were engaged in the drug's development.</li> </ul>	<ul style="list-style-type: none"> <li>• PMDA Guidance on Patient Participation</li> <li>• Patient Centricity WG</li> </ul>	<p>Chinese Technical Guidelines:</p> <ul style="list-style-type: none"> <li>• Patient-Centered Clinical Trial Design</li> <li>• Benefit Risk Assessment for Patient Centered Clinical Trials</li> <li>• The Implementation of Patient Centered Clinical Trials</li> </ul>

# Strategic Value of Patient Voice Across Stakeholders

**Table 1: How Patient Experience Data Could Enhance Medical Product Development and Decision Making**

Type of Patient Experience Data	Type of Stakeholder		
	Patient Stakeholders*	Medical Product Developers/Researchers	Regulators
<b>Patient registry or natural history study data</b>	<ul style="list-style-type: none"> <li>Inform communications, education and outreach efforts for the patient community</li> <li>Inform future research</li> <li>Provide basis for recruitment in clinical trials</li> </ul>	<ul style="list-style-type: none"> <li>Help identify biomarkers and clinical outcome measures that will show how well a patient responds to a treatment in clinical trials</li> <li>Inform clinical trial design</li> <li>Support clinical trial recruitment</li> </ul>	<ul style="list-style-type: none"> <li>Enhance the understanding of the course of disease over time, identifying demographic, genetic, environmental, and other factors that correlate with its development and outcomes in the absence of treatment (or while on available therapies)</li> </ul>
<b>Study report or survey data on the therapeutic context (severity of condition and unmet medical need), including perspectives on disease background, severity of condition, and available treatment options</b>	<ul style="list-style-type: none"> <li>Identify burden of disease and unmet medical needs that warrant further scientific discussion</li> <li>Identify opportunities and gaps where further development and research may be needed</li> <li>Identify considerations for clinical endpoints and clinically meaningful outcomes</li> <li>Inform patients on possibilities to participate in development and validation of clinical trial endpoints and patient-reported outcomes</li> </ul>	<ul style="list-style-type: none"> <li>Inform Target Product Profile</li> <li>Identify clinical domains (e.g., most bothersome symptoms) of the condition that could be targeted for new treatment development</li> <li>Identify how the condition may vary by sociodemographic factors, subgroups, culture, and disease severity</li> <li>Inform the selection, development and modification of meaningful clinical endpoints and outcomes, and tools that measure what matter most to patients</li> <li>Inform clinical trial design, including appropriate inclusion and exclusion criteria</li> </ul>	<ul style="list-style-type: none"> <li>Inform FDA decision-making throughout medical product lifecycle</li> <li>Enhance the understanding of the therapeutic context for benefit-risk assessments</li> <li>Enhance understanding of meaningful endpoints and outcomes to patients to appropriately advise sponsors on a medical product development plan in early phases of development</li> <li>Inform FDA guidance on disease-specific clinical, scientific and regulatory matters</li> <li>Inform FDA assessments of medical product development programs</li> </ul>
<b>Clinical trial experience data, including perspectives on trial visits and assessments</b>	<ul style="list-style-type: none"> <li>Help clinical trial participants better prepare for the trial, including the informed consent process</li> <li>Inform patients on opportunities to participate in clinical trials and improve overall recruitment</li> <li>Help individual decision making on whether to enroll in a trial</li> </ul>	<ul style="list-style-type: none"> <li>Enhance recruitment and retention for clinical trials</li> <li>Inform development of informed consent documents</li> <li>Provide insight into clinical trial participant burden, including frequency and conduct of trial visits and assessments</li> </ul>	<ul style="list-style-type: none"> <li>Enhance understanding of patient's experience with clinical trial design and inclusion/exclusion criteria to better advise sponsors</li> </ul>
<b>Patient input on benefits and risks</b>	<ul style="list-style-type: none"> <li>Inform future research</li> <li>Identify unmet medical needs that warrant further scientific discussion</li> <li>Enhance the understanding of benefits and risks for patients</li> </ul>	<ul style="list-style-type: none"> <li>Enhance the understanding of patient input on benefits and risks to inform benefit-risk assessment</li> </ul>	<ul style="list-style-type: none"> <li>Enhance the understanding of patient input on benefits and risks to inform benefit-risk assessment</li> </ul>

## Submission Dossier and FDA Reviews

<input type="checkbox"/> The patient experience data that were submitted as part of the application include:	Section of review where discussed, if applicable
<input type="checkbox"/> Clinical outcome assessment (COA) data, such as	
<input type="checkbox"/> Patient reported outcome (PRO)	
<input type="checkbox"/> Observer reported outcome (ObsRO)	
<input type="checkbox"/> Clinician reported outcome (ClinRO)	
<input type="checkbox"/> Performance outcome (PerfO)	
<input type="checkbox"/> Qualitative studies (e.g., individual patient/caregiver interviews, focus group interviews, expert interviews, Delphi Panel, etc.)	
<input type="checkbox"/> Patient-focused drug development or other stakeholder meeting summary reports	
<input type="checkbox"/> Observational survey studies designed to capture patient experience data	
<input type="checkbox"/> Natural history studies	
<input type="checkbox"/> Patient preference studies (e.g., submitted studies, scientific publications)	
<input type="checkbox"/> Other: (Please specify):	
<input type="checkbox"/> Patient experience data that were not submitted in this review:	
<input type="checkbox"/> Input informed from participation in meetings with patient stakeholders	
<input type="checkbox"/> Patient-focused drug development or other stakeholder meeting summary reports	
<input type="checkbox"/> Observational survey studies designed to capture patient experience data	
<input type="checkbox"/> Other: (Please specify):	
<b>Patient experience data was not submitted as part of this application</b>	

**Patient Experience Data (PED) Tables in Health authority documents**

## EMA Assessment Report

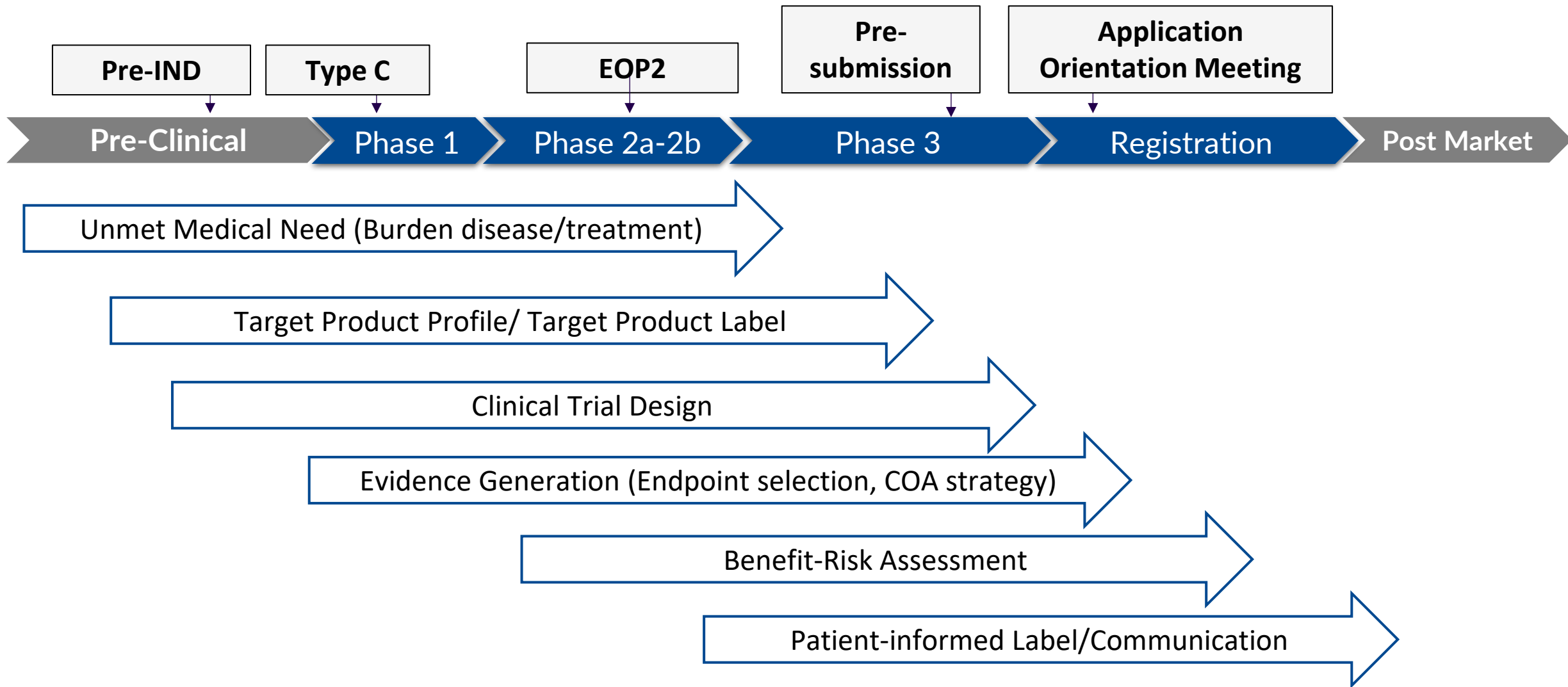
The following table with tick boxes provides an overview on the type of Patient Experience Data (PED) submitted in support of this application. Please tick the option that applies and mention section where this is further referenced in the AR:

Table 2: Patient experience data relevant to the application

Patient experience data submitted with this application	Section where discussed (if applicable)
<input type="checkbox"/> Patient experience data submitted by the applicant:	
<input type="checkbox"/> Clinical outcome assessments (COAs) such as	
<input type="checkbox"/> Patient-reported outcomes (PRO)	
<input type="checkbox"/> Other	
<input type="checkbox"/> Patient preference studies	
<input type="checkbox"/> Observational studies/RWD designed to capture patient experience data	
<input type="checkbox"/> Qualitative information or studies (e.g. summaries/analysis from patient engagement activities such as individual patient/caregiver interviews, focus group interviews, expert interviews, etc)	
<input type="checkbox"/> Other (please specify)	
<input type="checkbox"/> Other patient experience data not submitted by the applicant but considered in this evaluation:	
<input type="checkbox"/> Input informed from participation in meetings or public hearings with patient stakeholders	
<input type="checkbox"/> CHMP early dialogue with patient organisations	
<input type="checkbox"/> Third party interventions from patients and patient groups	
<input type="checkbox"/> Other (such as medical literature, summaries/analysis from patient engagement activities - please specify)	

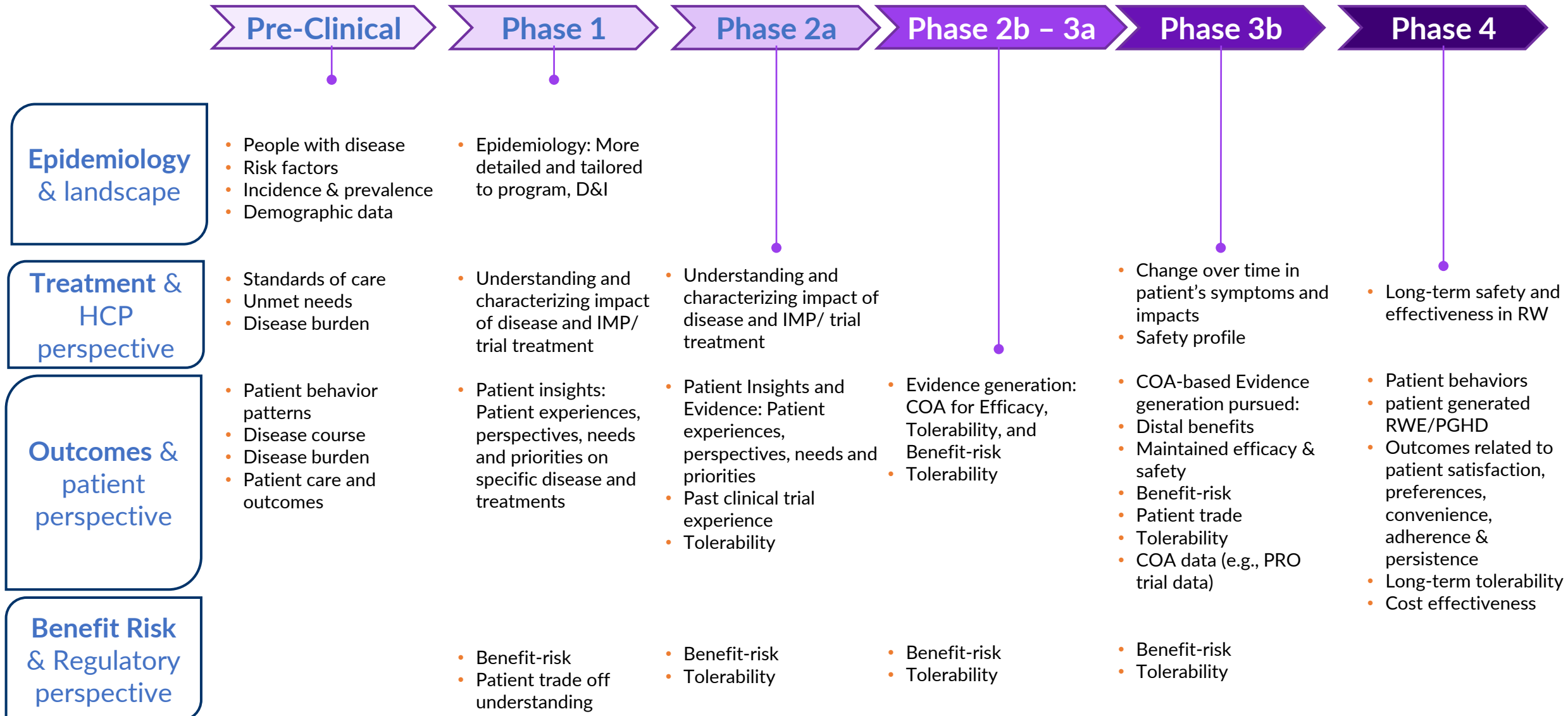
Source: FDA Guidance “Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data Guidance for Industry and Other Stakeholders”

# Consider Patient Voice Early & Throughout the Development Process



**Early integration of patient engagement strategies, patient experience data collection & cross-functional partnerships can help drive internal strategy on how patient voice can inform clinical trial design and regulatory decision making.**

# Intentional & Consistent Milestones On Which We Can Build Patient Disease Strategies To Increase Health Value



# Our Priority is Developing Medicines that Patients Want

The **Integrated Evidence Generation Plan (iEGP)** is our roadmap for gathering that data through clinical research.

To help patients interpret the results of our science, and make informed treatment decisions, we establish a patient relevant drug label strategy.

**100%**  
iEGPs advised by patient advocacy groups

**100%**  
Phase 3 indications have a patient relevant label strategy

Sanofi's integrated patient engagement framework ensures the holistic health related experience of patients is part of the Development process



**100%**  
Phase 1-3 programs have a patient informed TVP

**98%**  
Phase 1-3 indications in development have a patient-informed sBRA

The **Target Value Proposition (TVP)** outlines these *expected characteristics* of a new medicine as it relates to how it addresses what patients, HCPs, payers, and regulatory expect.

**Structured Benefit-Risk Assessment (sBRA)** is the continuous, structured evaluation of whether a drug's therapeutic benefits outweigh its risks, and is convenient to take, for a specific patient population.

# Patient Priorities Driving Development

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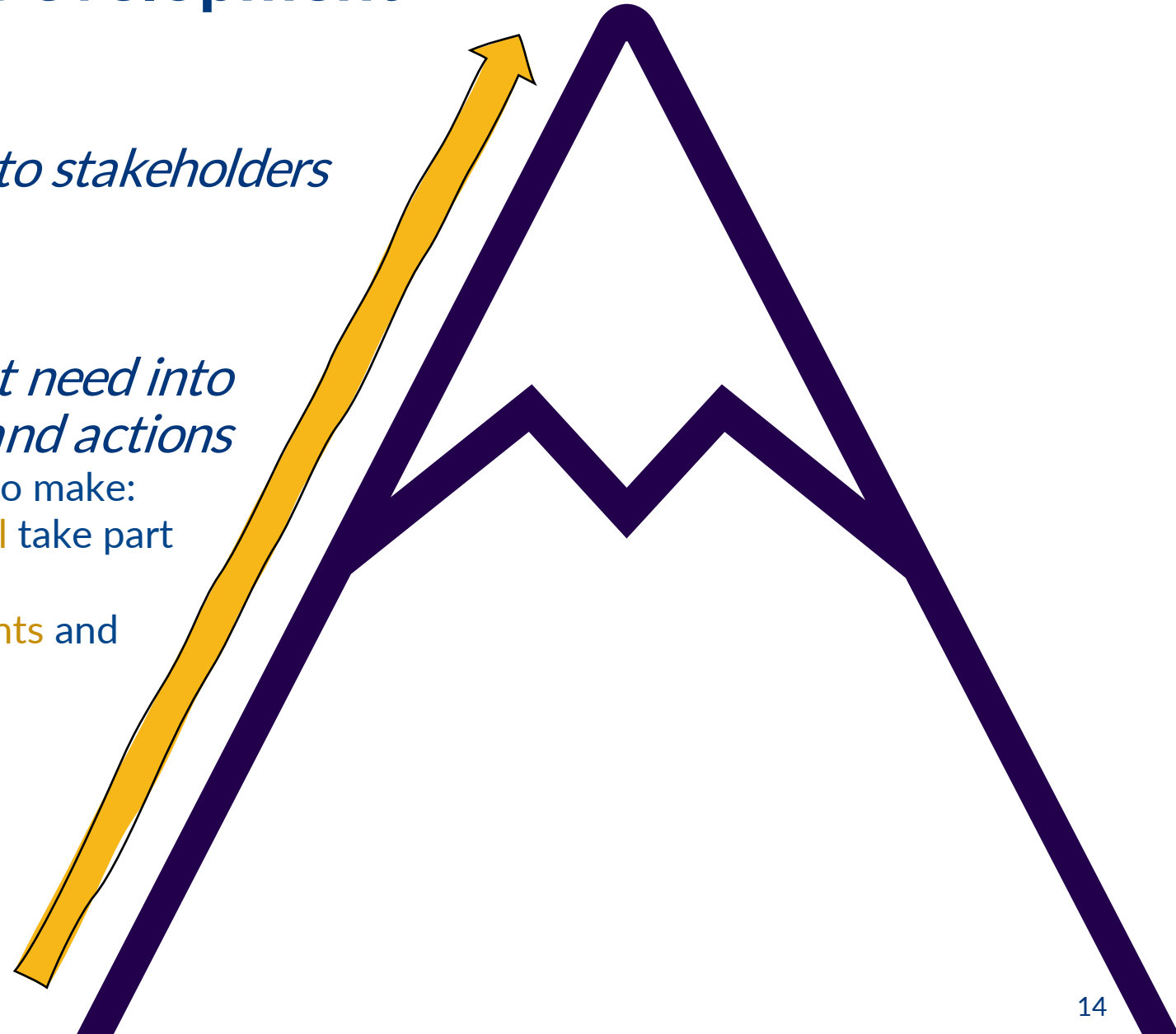
*Step 3. Show value to stakeholders*

*Step 2. Interpret need into  
priorities and actions*

Identify the most impactful ways to make:

- A **trial** that patients **can and will** take part in
- A **treatment** that **reaches patients and patients want to take**

*Step 1. Understand patient  
needs*



# Case Study Patient Priorities Driving Development: Long-Term PRO Data Collection in Dermatology Trials

## Step 1: Understand patient needs

- **PRO measures** for daily symptoms are **critical in dermatology trials** for chronic conditions like Hidradenitis Suppurativa (HS) and Atopic Dermatitis (AD). However, **long-term extension studies typically eliminate daily PRO measure** creating gaps in understanding sustained treatment benefit.
- **Patient advisory board** to pressure test protocol

## Step 2: Interpret need into priorities and actions

- Issues identified with proposed daily PRO collection approach using provision device:
  - Remembering to use a separate device intermittently
  - Infrequent use could lead to battery depletion between assessment periods
  - Maintaining an additional device could add unnecessary complexity

**ACTION:** Bring Your Own Device (BYOD) option proposed for PRO diary completion in extension studies, 1 week out of every 4 weeks.

## Step 3: Show value to stakeholders

- Daily symptoms are **core to treatment benefit**, and sustained treatment benefit is **essential to stakeholders** (e.g., payers).
- **Patient burden is minimized** with a patient-driven design solution, and **value outweighs this minimal added trial complexity** of daily PRO measures.



# Case Study Patient Priorities Driving Development: Elevating Fatigue Endpoint in Multiple Sclerosis Trials

Step 1:  
Understand  
patient needs

- **PRO measures essential** in multiple sclerosis (MS) clinical trials to capture treatment benefits that matter most to patients.
- **Traditional endpoint hierarchies may not fully reflect** patient priorities for treatment.
- **Patient Preference Study** conducted to understand what matters most to patients living with MS.

Step 2:  
Interpret need  
into priorities  
and actions

- **Identification of patient-relevant endpoints** in MS trials presented a critical opportunity.
- **Fatigue emerged as the most burdensome symptom** impacting patients' daily life, yet **not adequately prioritized** in traditional endpoint hierarchies

**ACTION: Elevate fatigue to a higher-ranking endpoint** in the trial design to reflect patient-identified priority of meaningful improvement.

Step 3: Show  
value to  
stakeholders

- Used **mix of evidence-grade data** from the Preference Study and insights and alignment from patient advisors to show that fatigue is a priority treatment outcome.
- Demonstrate **relationship of fatigue to treatment and accepted outcomes:**
  - Upstream (e.g., Mechanism of action)
  - Downstream (e.g., disease outcomes, HRQoL, health care resource use)



# Measuring Impact: Sanofi Patient Promise Develop Medicines That Reflect Patient Priorities

Integrated Patient Engagement at Sanofi is driven by our Patient Community Promise, which was co-created with patients, caregivers, and patient advocacy groups around the world.

We develop medicines that reflect patient priorities

—  
*Patient input has been integrated in 100% of our Research & Development programs*

We improve and adapt our medicines through real-world patient community insights

—  
*92% of clinical trials completed in 2024 included remote and digital solutions that make it easier for patients to participate*

We take a comprehensive approach to our partnership with the patient community

—  
*100% of clinical trials in the U.S. have Diversity & Inclusion goals to ensure our science is representative of the patient community*

We advocate for people-centered health care systems

—  
*100% of global regulatory filings<sup>1</sup> had Patient Experience Data submitted as evidence*

In 2025, 44 drug programs were informed with patient insights – affecting 59 disease indications across 163 studies<sup>2</sup>

1. Submitted in 2024 in US, China, Japan and Europe.  
2. End of Year 2025 data.

# We're helping make studies less burdensome and more meaningful for patients

**Why?** Because we want our study protocols to truly reflect patient experiences and make it easier for individuals to participate in clinical research with the goal of improving **speed**, **access**, **retention**, and **patient representation**.

## Key Success Factors

- Early engagement
- Real-world device testing
- Collaborative approach with PAGs
- Committed study teams willing to adapt protocols based on patient feedback
- Preserving scientific integrity by balancing patient needs with regulatory compliance



... were the **key protocol elements** we refined based on patient perspectives.

100%

Patient input has been considered in all new studies

# Key takeaways

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**Patient priorities driving development is not optional, its essential** – incorporating patient experience data into development ensures treatments address what matters most to patients, and patients can access the trial and treatments.

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**Be intentional and strategic in patient and health authority engagement** – this starts with cross-functional planning internally, with the end in mind, to align efforts and maximize impact.

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**Communication and transparency are essential** – ensuring stakeholders understand how patient experience data (PED) is collected and used by sponsors and regulators creates a vital feedback loop that keeps PED a priority across the industry.

# Resources

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FDA:

[FDA CDER PFDD Webpage](#) (Glossary, Guidances, Voice of the Patient Summary reports, etc)

EMA:

[Patient experience data \(PED\) reflection paper | European Medicines Agency \(EMA\)](#)

MHRA:

[Patient Involvement Strategy: an assessment of progress - GOV.UK](#)

PMDA:

[PMDA Guidance on Patient Participation](#)

CDE:

[Patient-Centered Clinical Trial Design](#)

ICH E22 Draft Guideline:

[ICH\\_E22\\_Step2\\_draftGuideline\\_Assembly\\_Endorsed\\_FINAL\\_2025\\_1119.pdf](#)

# Practical Considerations for Executing Patient Engagement in Clinical Research

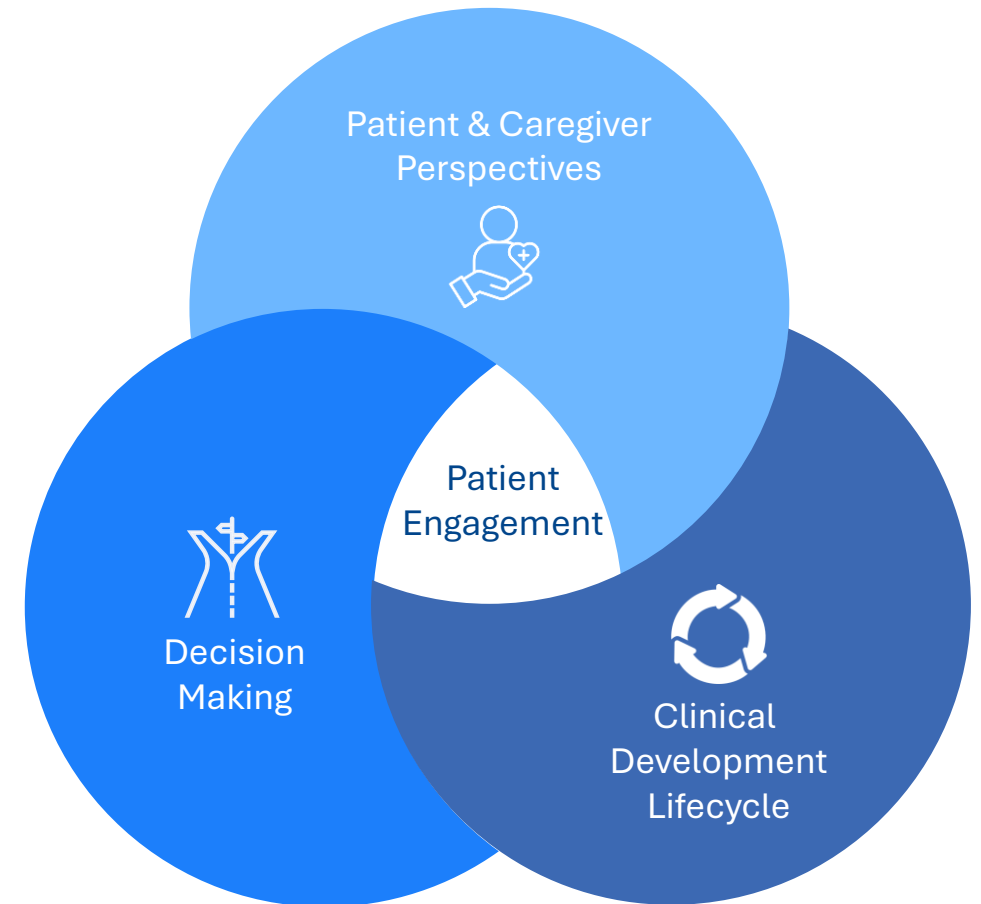
Amanda Decoker

Head of Patient Engagement, Experience and Recruitment (PEER)

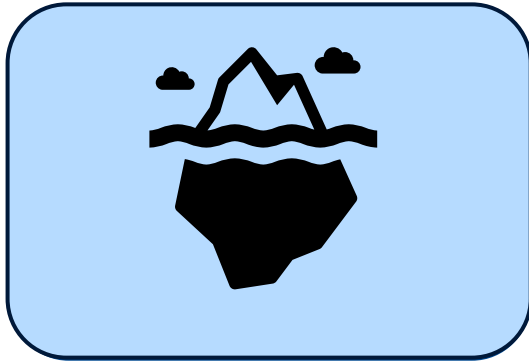
Takeda

# What We Mean by Patient Engagement

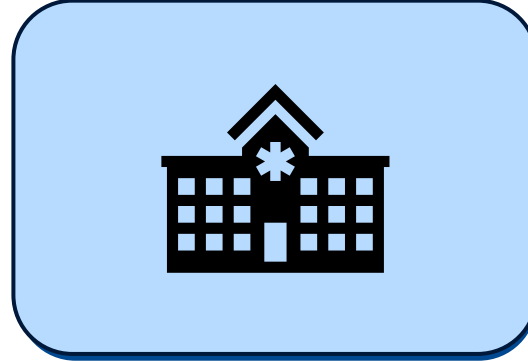
- **Patient engagement in clinical research includes:**
  - Involving patients and caregivers *outside* of trial participation
  - Seeking input across the development lifecycle
  - Using insights to inform real decisions
- **Common applications:**
  - Protocol and visit design
  - Endpoint alignment between clinicians and patients
  - Recruitment and retention strategies
  - Patient-facing materials
- **What it is not:**
  - Marketing
  - Recruitment alone
  - One-off anecdotes without action



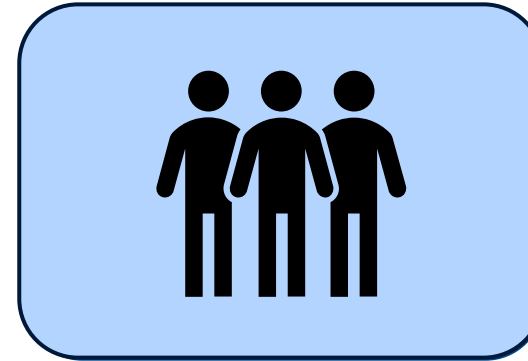
# Why Patient Engagement Matters



Increasing protocol complexity and patient burden



Recruitment and retention challenges persist across indications



Regulators, payers, and sponsors expect patient-centric approaches



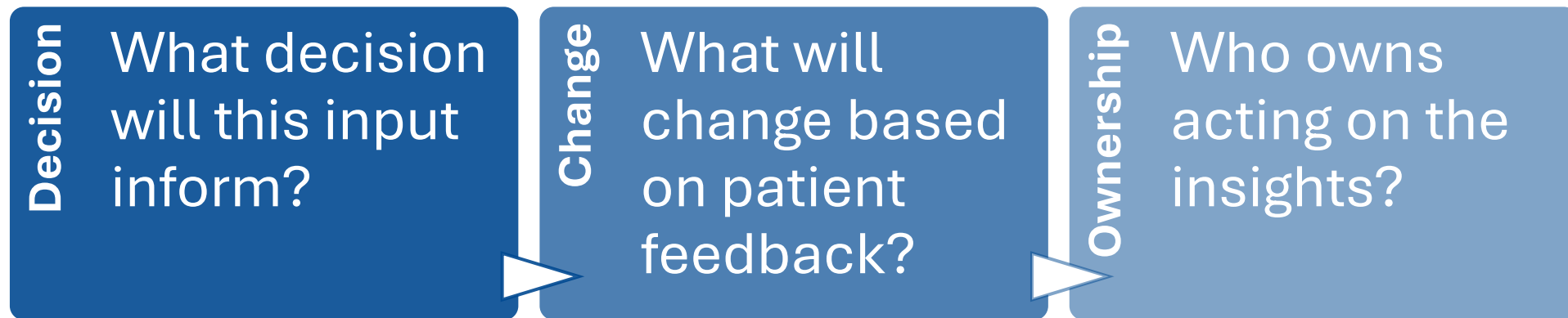
Engagement is no longer *whether* — it's *how*

## Key challenge

Many teams support patient engagement in principle, but struggle with execution.

# Start with the “Why”

Before engaging patients, teams must answer



- **Common pitfall:** Engagement activities launched without a clear purpose or decision pathway.
- **Best practice:** Design engagement with the *end decision* in mind.

# Choosing the Right Engagement Method

Goal	Practical Method
Early Concept Feedback	Advisory Boards, In-Depth Interviews
Study Burden Assessment	Surveys, Diary Reviews
Recruitment Optimization	Message Testing, Caregiver Interviews
Ongoing Trial Experience	Pulse Surveys, Patient Check-ins

## Key consideration

More engagement ≠ better engagement.  
Right-sized, targeted engagement leads to more actionable insights.

# Operational & Logistical Considerations



## TIMING

Engage early enough to influence decisions



## COMPENSATION

Fair, transparent, and compliant



## ACCESSIBILITY

Language, health literacy, disability accommodations



## RESOURCING

Internal execution vs. external vendors



## COORDINATION

Alignment with clinical, operations, and study timelines

**Reality check:** Engagement fails when treated as an add-on instead of core work.

# Legal, Regulatory & Compliance Realities



## Common concerns teams raise:

- Fair market value and compensation
- Privacy and informed consent
- Promotional boundaries
- Documentation and audit readiness



## Practical enablers:

- Pre-approved engagement frameworks
- Standard templates (contracts, consent language)
- Early involvement of legal and compliance partners
- Clear distinction between insight-gathering and trial participation

# Turning Insights Into Action



## HEAR

The biggest risk:  
Collecting insights that  
don't influence  
decisions.



## TRANSLATE

Translate findings into  
decision-ready  
summaries



## ACT

Clearly link insights  
to:

- Protocol changes
- Recruitment  
strategies
- Patient materials



## COMMUNICATE

Communicate outcomes  
internally

- What we heard
- What changed
- What couldn't change  
— and why

**Credibility comes from impact, not activity.**

# Patient Engagement Case Study: Pediatric Psoriasis



## Scope of Patient Engagement Work:






PEER organized a Patient Engagement Planning Workshop that brought together functional leads from clinical and commercial teams, including Patient Advocacy, Global Project Team leadership, Clinical Science, and Feasibility. During the workshop, unmet needs were identified, particularly around the experiences of children and adolescents living with psoriasis.



PEER then partnered with a patient engagement vendor to develop an electronic survey of 130 parents and caregivers in the US and EU to gather insights on the condition. These surveys were followed by in-depth interviews with 15 caregivers to further explore key themes.

# Patient Engagement Case Study: Pediatric Psoriasis

## Patient Insights: Key findings from the Patient Engagement work and actions taken:

<p><b>Caregivers reported they were willing to spend up to 2 hours</b> onsite for visits and the fewer onsite visits the better</p>		<p><b>Action:</b> Clinical Science considered this when planning the number of assessments for each visit and changed some planned visits to telephone visits instead of onsite</p>
<p><b>Caregivers are more likely to have their child participate</b> in a clinical trial if visits can take place before/after school or on weekends, limiting time out of school and work.</p>		<p><b>Action:</b> Feasibility included a question on early/late and weekend hours in Site Feasibility Questionnaire</p>
<p><b>Caregivers expect monetary compensation for clinical trial participation;</b> many caregivers would expect reimbursement for travel expenses. In US, caregivers expect around \$100 stipend per visit.</p>		<p><b>Action:</b> Visit stipend was set at \$125 for a standard visit and increased to \$160 per visit for a longer 4-hour post-dose PK visit.</p>
<p><b>Caregivers expressed childcare for their other children as a barrier</b> to trial participants</p>		<p><b>Action:</b> Childcare reimbursement included in site budgets and with Participant Stipend, Reimbursement and Travel vendor</p>
<p><b>Caregivers expressed concerns about placebo</b></p>		<p><b>Action:</b> PEER ensured that study brochures and other recruitment materials emphasized that all patients receive active treatment after the initial 4 months of the study</p>



**PEER also received budget from the Global Project Team to create a Patient Story video as an educational resource** within Takeda and as part of Takeda’s commitment to being patient-centric in its approach to developing medicines. The video will be shared internally with Takeda employees as well as externally on the Takeda website and social media.

# Measuring Success

## Move beyond counting activities

- Meaningful indicators include:
  - Were insights used to inform a decision?
  - Did engagement influence protocol or study design?
  - Impact on recruitment, retention, or patient experience
  - Internal stakeholder confidence and adoption



Shift thinking from “*We engaged patients*” to “*We changed X because of patient input.*”

# Key Takeaways

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Patient engagement must be **purpose-driven**

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Successful execution requires **cross-functional planning**

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Impact depends on **closing the loop**

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When done well, engagement leads to **better science** and **better experiences**

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Patient engagement isn't extra work — it's **smarter** work.



# Expert Panel Perspectives

# Panel Discussion and Q&A

- **Moderator**
  - *Elizabeth (Nicki) Bush, MHS* - OPEN Health
- **Presenters**
  - *Pujita Vaidya, MPH* - Sanofi
  - *Amanda Decoker, MA* - Takeda
- **Additional Panelists**
  - *Samantha Parker, MBA* - Italfarmaco
  - *Dominique Pichard, MD, MS* - International Rett Syndrome Foundation
  - *Cynthia (Cyndi) Grossman, PhD* - CDRH, FDA



**Thank You!**

Advancing Drug Development. Improving Lives. Together