Patient Engagement open forum Into Reality

Patient Experience Data in Regulatory Processes:

Informing regulatory communication toward medical product development

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

PATIENT FOCUSED MEDICINES DEVELOPMENT

EUPATI
European Patients' Academy on Therapeutic Innovation

REC **Disclaimer:** This meeting is going to be recorded.

What is the Patient Engagement Open Forum

Patient Engagement open forum Into Reality

A series of virtual events (in 2020) where we will work together, in a multi-stakeholder context, **to turn patient engagement from an aspiration into reality**.

The Forum aims to provide **a holistic perspective** of patient engagement, the **landscape and actors**, and **foster collaboration** and **co-creation** while **breaking down fragmentation** that are often present in patient engagement work.

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Patient Engagement open forum Into Reality

Meet our panelists

Nicholas Brooke
Panelist
Founder and Executive Director, ***The Synergist.org***

Eleanor Perfetto, PhD, MS
Panelist
Executive Vice President, ***National Health Council***

Jessica Scott, MD, JD
Panelist
Head of R&D Patient Engagement, ***Takeda Pharmaceuticals***

Wendy Sanhai, PhD, MBA
Moderator
Specialist Leader, ***Deloitte Consulting Government & Public Services***

PATIENT FOCUSED MEDICINES DEVELOPMENT

Patient Engagement open forum Beyond Aspiration

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Agenda

Item	Speaker	Time
Introduction	Nicholas Brooke *Founder and Executive Director, The Synergist.org* Eleanor Perfetto, PhD, MS *Executive Vice President, National Health Council*	**10’**
Setting the stage	Jessica Scott, MD, JD *Head of Global R&D Patient Engagement, Takeda Pharmaceuticals*	**15’**
Interview Findings **Interactive Session**	Wendy Sanhai, PhD, MBA *Specialist Leader, Deloitte Consulting*	**40’**
Panel Discussion **Interactive Session**	Nicholas Brooke Eleanor Perfetto, PhD, MS Jessica Scott, MD, JD Wendy Sanhai, PhD, MBA (moderator)	**45’**
Thank you and Next Steps	Eleanor Perfetto, PhD, MS	**10’**

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Patient Engagement open forum Beyond Aspiration

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Patient Engagement open forum Into Reality

Patient Experience Data (PED) and patient engagement to shape, deliver and analyse it.

Why is this important?

A patient community perspective:

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Patient Engagement open forum Into Reality

Before we get started, we ask you…

Be present & engaged

Stretch your thinking

Turn on video and smile if you can (pets and kids welcome!)

Together let’s make it interactive

Note: this session will be recorded

Session Objectives

The participants will:

Learn more about ongoing patient engagement efforts to inform regulatory decision making

Explore challenges and emerging good practices on the use of Patient Experience Data (PED) in regulatory submissions

Contribute to prioritizing the PED challenges and related emerging good practices that emerged from feedback to date

Contribute to next steps for the enhanced use of PED in regulatory decision making, in collaboration with patient organizations, biopharma and regulators

We will be using PollEverywhere to capture your insights

How to provide your answers:

1 Navigate to **www.pollev.com/ped2020**

2 Please enter your name (responses are anonymous)

3 Keep an eye our for this image which will indicate a polling question

4 Provide your response and see what others think!

Let's give it a try…

How are you feeling today?

excited | 😀

tired | 😔

frustrated| 😓

ready | 😎

With which stakeholder group do you most align?

Patient Organizations

Biopharmaceutical

Not-for-Profit

Regulator

Consultant

Other

Setting the Stage

Jessica Scott, MD, JD

Head of Global R&D Patient Engagement, Takeda Pharmaceuticals

Takeda R&D

Patient Engagement Office

PEOF Workshop

June 25, 2020

Jessica Scott, MD, JD
Takeda Head of Patient Engagement Office

From developing medicines for patients...
to developing medicines with patients

Moving from Developing Medicines *for* Patients to Developing *with* Patients

Patient-Focused Culture

Patient-themed Activities (2018)

Patient Engagement Activities & Plans* (2019-2020)

* Patient Engagement Plan (PEP) is a proactive road map for how and when the patient/community perspective will be incorporated into the development of the asset

Patient Engagement In Friedreich's Ataxia

New Primary Endpoint

FARA Friedreich's Ataxia Research Alliance

Patient Engagement Celiac Disease

Gluten Challenge

Initial Stage Catalyzed by Several Organizations

Takeda

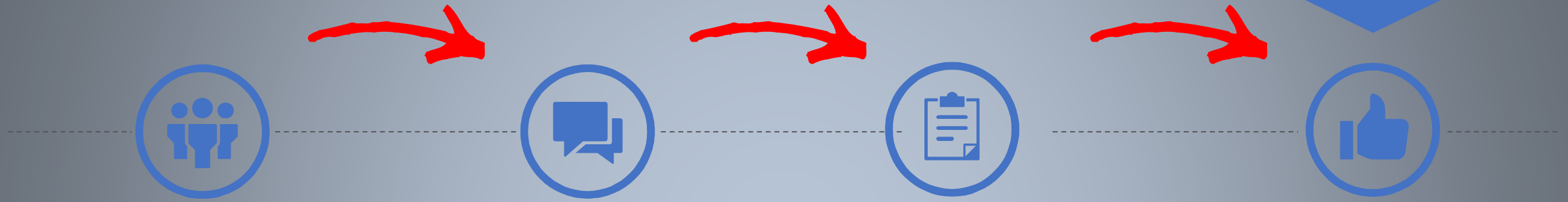
Deloitte.

PATIENT FOCUSED MEDICINES DEVELOPMENT

PUTTING PATIENTS FIRST®
NATIONAL HEALTH COUNCIL

Stakeholder Engagement to Better Understand Current State

Today



Identify Stakeholders

60+ stakeholders identified

Conduct Interviews

Conducted 35 interviews

Document Findings

Identified themes, and aggregated findings into categories

Share initial Findings Validate Current State

Identify gaps and level of interest progressing further

Interview Findings

Wendy Sanhai, PhD, MBA

Specialist Leader, Deloitte Consulting

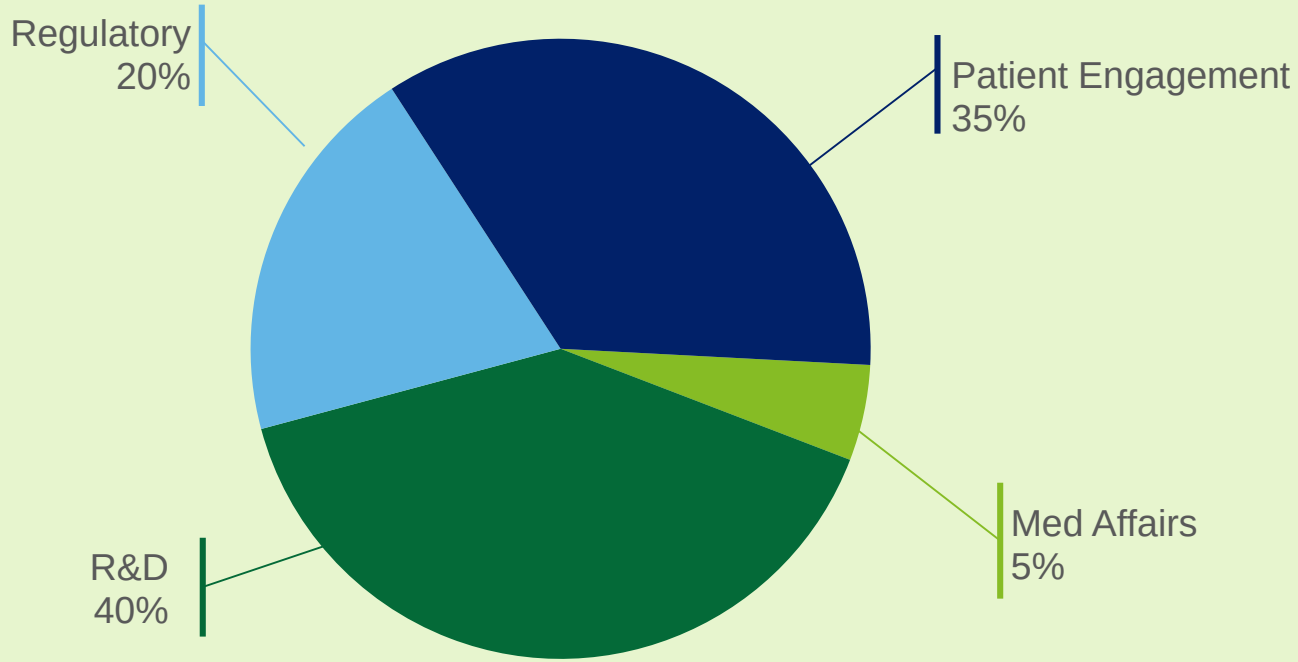
35 stakeholders interviewed across multiple stakeholder groups

5 Patient Organizations

4 Other Not-for-Profit Stakeholders

10 Biopharmaceutical Companies

Functional breakdown of biopharma interviews:



Obtained insights on the current practices, existing gaps and future opportunities

Dimension		Definitions
Focus for today		
Current state	**Challenges**	• Inconsistencies/hurdles in patient engagement practices, PED (qualitative and quantitative) across industry and in communication with regulators
	Emerging Good Practices	• Approaches/methodologies within biopharma where patient engagement teams felt empowered to include the voice of the patient in the drug development • Approaches that facilitated communication with regulators
Future state	**Potential Future Practices**	• Opportunities to increase patient engagement in drug development • Considerations for communication and collaboration with regulators whereby information can inform regulatory decision-making

Note: Future State is not in scope for this session

We identified challenges, emerging good practices and recommendations to improve the use of Patient Experience Data (PED)

Dimension		Sampling of what we heard
Current state	**Challenges**	"It appears that more finite **(smaller) populations are getting more attention** from regulators compared to more prevalent diseases, like Alzheimer's Disease, for example"
		"The PRO pathway is well developed, whereas other clinical outcome assessment measures are less developed and less 'popular' so it's a **bigger hurdle to get support for new tools"**
		"Still a grey area in **understanding what regulators want in terms of PED**. Beyond PROs, it would be helpful knowing what they would find helpful"
	Emerging Good Practices	"We need to look at how we are engaging with **patients as partners** rather than as data points"
		"We have defined corporate level procedures and educational resources to provide teams with guidance on engaging patients - who to engage, **how to engage**, numbers to engage, payments, mandatory attendees from the company, etc."
		"FDA has been very complimentary about **sponsors going to the agency early**"
Future state	**Potential Future Practices**	"Regulators need to hear it for themselves so that they change their thinking. To do this, early engagement with regulators and early buy-in from company leadership are important in de-risking the tools and approaches developed and accepted for regulatory decision-making "

Note: Future State is not in scope for this session

Four major themes emerged from the interviews

Theme	Definitions
Culture	Assumptions/notions that influence PE and use of PED
Cross-organizational Alignment	Infrastructure within R&D/other functions that facilitate or impede collaboration on PE and PED development
Process	Frameworks to integrate PE and develop PED in R&D and for submissions
Tools	Instruments/platforms/techniques for PE and generation of PED in clinical trials and for submissions

Regulators: Taking actionable steps to include the patient's voice and PED in submissions

FDA Announces Public Meeting on Independent Assessment of Communications Between FDA and Sponsors During Drug Development

On August 11, 2020, the U.S. Food and Drug Administration (FDA) will hold a public meeting to discuss Eastern Research Group, Inc.'s (ERG) assessment of communication practices between FDA and sponsors during the investigational new drug (IND) phase of new drug/biologic development. Completion of the assessment and the public meeting satisfies FDA's commitment to use an independent third party to assess FDA-sponsor communication practices during the IND stage of drug/biologic development, mandated by the Prescription Drug User Fee Act for fiscal years 2018 through 2022 ("PDUFA VI").

https://www.fda.gov/media/139088/download

FDA's first patient-focused drug development guidance now final

Posted 17 June 2020 | By Kari Oakes

Final guidance for drugmakers on gathering comprehensive and representative input from patients is now available from the US Food and Drug Administration (FDA).

The document is the first in a series of four that will outline patient-focused drug development (PFDD) guidance "to address, in a stepwise manner, how stakeholders (patients, researchers, medical product developers and others) can collect and submit patient experience data and other relevant information from patients and caregivers for medical product development and regulatory decision making," said the FDA. (RELATED: *Patient focused drug development: FDA issues guidance on gathering patient input*, *Regulatory Focus* 12 June 2018)

The *21st Century Cures Act* includes requirements for PFDD, and the guidance also meets one of the commitments made by the agency under the most recent *Prescription Drug User Fee Act* (PDUFA VI).

The guidance outlines what constitutes patient experience data and reviews general considerations for patient experience data collection. Some specific topics presented are questions that can be put to stakeholders to define which research questions are of interest, what the study population should be, and what to consider in designing a study.

Also, the guidance suggests specific quantitative and qualitative methods that can be used to collect

https://www.fda.gov/drugs/news-events-human-drugs/public-meeting-independent-third-party-assessment-ind-fda-sponsor-communication-practices-pdufa-vi

We will want to hear from you. How relevant are our findings to you?

Current state

Challenges

Select top three **Challenges** based on your perspective

Emerging Good Practices

Are there additional **Emerging Good Practices** that you have found?

Please drop any questions for our panel in the chat window!

Current State

Challenges & Emerging Good Practices

CULTURE

Assumptions/notions that influence PE and use of PED

Culture | Sponsors (Internal)

Challenges

Sticking to Tradition

- Across R&D, TAs use traditional approaches and standard operating procedures (SOPs)
- Not willing to incorporate the patient's voice in drug development

Isolated PED and Differences in Perceived Value

- The value placed on early PE and on the use of PED varies by each TA

Being Risk-Averse

- TAs are unsure of what to provide regulators beyond PROs
- New/untested approaches could risking a rejection by regulator

Emerging Good Practices

Leadership buy-in

- Support from senior leadership imperative
- Consistent cross-R&D adoption of PE practices and PED development

Cross R&D use of PED

- Development of PE strategies and PED across drug lifecycle
- Driving greater awareness and adoption

PE Roadmap Development

- Development of clear/proactive PE roadmap
- Collection/analysis of PED, with the goal of regulatory submissions

Culture | Sponsors (External)

Challenges

Rules of Engagement for Patients and Sponsors

- Regulatory rules for patients/sponsor interaction
- Challenging for rare diseases given limited number of patients

Challenging the Status Quo

- Reluctance to push boundaries with regulators
- No new approaches for PE and COA measures and tools

Perceived Inconsistency in Sponsor Findings

- Apparent hesitation from regulators in accepting PED
- Inconsistent regulatory feedback for appropriate PED submissions

Emerging Good Practices

Understanding the Patient Perspective

- Development of "neutral ground territory" for regulators
- Attending patient listening sessions to hear top-of-mind needs for patients and caregivers

Contributing Novel Perspectives

- Leveraging inputs from both social and clinical scientists to define qualitative and quantitative measures on the use of PED

Direct Inclusion of Patients

- Evolution in willingness of regulators to accept patient insights
- Regulators more open to collaborating with patients and patient advisory committees

Culture | Patient Organizations

Challenges

Understanding the Benefit of Everyday Activities

- Sponsors struggle understanding the benefit/risk beyond measurable insights, where the burden of a disease will influence patient's willingness to take risks

Need Unity of Purpose

- Developing registries without regulatory submission in mind
- Siloed collection, annotation, and analysis
- Lack of interoperability of data systems/registries

Managing the Dialogue and Use of PED

- Some sponsors struggle in communicating with patients due to the emotional conversations

Emerging Good Practices

Early and Frequent Patient Input

- Development of early/ongoing working partnership and to allow patient input early

Opportunity to Act as Intermediaries

- Collaborating with sponsors on defining valuable COAs for patients
- Connecting with regulators/divisions early encourage PE and "HOW" and "WHAT" PED to use

Culture | Challenges Poll Question

Sponsor – Internal Operations

Sticking to Tradition

Isolated PED and Differences in Perceived Value

Being Risk-Averse

Sponsor – External Engagement

Rules of Engagement for Patients and Sponsors

Challenging the Status Quo

Perceived Inconsistency in Sponsor Findings

Patient Organizations

Understanding the Benefit of Everyday Activities

Needing Unity of Purpose

Managing the Dialogue and Use of PED

Select your top three culture-related challenges

Select your top three culture-related challenges:

Sticking to Tradition

Isolated PED and Differences in Perceived Value

Being Risk-Averse

Rules of Engagement for Patients and Sponsors

Challenging the Status Quo

Perceived Inconsistency in Sponsor Findings

Understanding the Benefit of Everyday Activities

Needing Unity of Purpose

Managing the Dialogue and Use of PED

Select your top three culture-related challenges:

- Sticking to Tradition
- Isolated PED and Differences in Perceived Value
- Being Risk-Averse
- Rules of Engagement for Patients and Sponsors
- Challenging the Status Quo
- Perceived Inconsistency in Sponsor Findings
- Understanding the Benefit of Everyday Activities
- Needing Unity of Purpose
- Managing the Dialogue and Use of PED

Are there additional culture-related challenges?

Include additional culture-related challenges:

CROSS-ORGANIZATIONAL ALIGNMENT

Infrastructure within R&D/other functions that facilitate or impede collaboration on PE and PED development

Cross-organizational Alignment | Sponsor (Internal)

Challenges

Operations Siloed by Function

- Lack of collaboration/ communication between teams

Lack of Cross-Functional PE Leadership

- Lack of coordination across R&D and TAs resulting in disjointed PE efforts
- Absence of integrated PE plan/roadmap

Autonomous TAs

- Each TA drives its own clinical trial processes
- No centralization of PE activities
- Varied engagement of patients and disparate development of PED

Emerging Good Practices

Cross-Functional Collaboration

- Frequent consults between functions/business units on defining COA measures, tools, etc. based on PED
- Creation of submission packages/briefing books for regulators early

PE Specific Role

- Creation of a centralized role with primary responsibilities
- Coordinating PE efforts: generation/use of PED in regulatory submissions, product labeling and post-launch activities

Routine Evaluation

- Set up of PE taskforce to identify opportunities to increase the use of PED in development of medical products

Cross-organizational Alignment | Sponsors (External)

Challenges

Inconsistent Use of PED

- Lack of clarity in what regulators will accept for PED
- Unclear value of PED for regulatory decision-making

Differences Across Divisions

- Varying degrees of willingness to engage early with sponsors and accept PED in submissions

Inconsistent Communication

- Different levels of willingness to engage with sponsors across reviewing divisions and regulatory leadership

Emerging Good Practices

Collaboration Across Regulatory Bodies

- Sessions between regulatory agencies to discuss PE activities
- Agreement on consistency and quality of PED in regulatory decisions

Consistency in Internal Messaging

- More consistent communication/messaging across regulatory offices and divisions
- Adoption of uniform practices across regulatory agencies

Cross-organizational Alignment | Patient Organizations

Challenges

Individual Patient Bias

- Patient's view is specific to their individual experience
- Limited patient input can impact trial design, and broader understanding of the disease

Disease Agnostic Trial Design

- Sponsors develop trial designs without patient input
- Generic trial designs not tailored to specific disease needs
- Different benefit/risk metrics

Misaligned Metrics

- Overreliance on PROs make patients feel unheard as there are other measures/tools

Emerging Good Practices

Community Engagement

- Engaging patient community regularly to understand impacts from events
- Engaging representative patient populations to ensure better understanding of drugs' efficacy and safety

Establish Formal Patient Communication Pathways

- Obtaining patient's input early in Sponsors' program/asset development

Centralized Effort to Facilitate PE

- Willingness across industry: identify and discuss opportunities to improve PE efforts, align efforts

Cross-organizational Alignment | Challenges Poll Question

Sponsor – Internal Operations

Operations Siloed by Function

Lack of Cross-Functional PE Leadership

Autonomous TAs

Sponsor – External Engagement

Inconsistent Use of PED

Differences Across Divisions

Inconsistent Communication

Patient Organizations

Individual Patient Bias

Disease Agnostic Trial Design

Unaligned Metrics

Select your top three cross-organizational alignment-related challenges

Select your top three cross-organizational alignment-related challenges

Operations Siloed by Function

Lack of Cross-Functional PE Leadership

Autonomous TAs

Inconsistent Use of PED

Differences Across Divisions

Inconsistent Communication

Individual Patient Bias

Disease Agnostic Trial Design

Unaligned Metrics

Select your top three cross-organizational alignment-related challenges

Operations Siloed by Function

Lack of Cross-Functional PE Leadership

Autonomous TAs

Inconsistent Use of PED

Differences Across Divisions

Inconsistent Communication

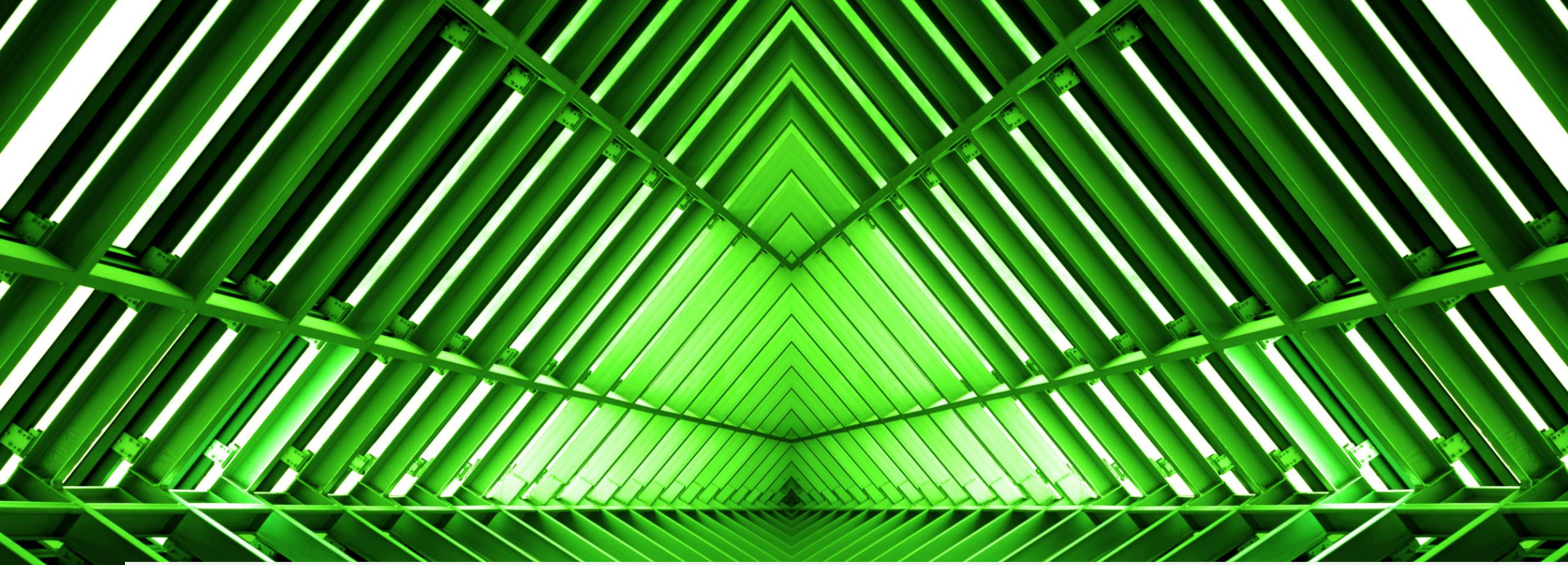
Individual Patient Bias

Disease Agnostic Trial Design

Unaligned Metrics

Are there additional cross–organizational alignment-related challenges?

Include additional cross-organizational alignment-related challenges:



PROCESS

Frameworks to integrate PE and develop PED in R&D and for submissions

Process | Sponsors (Internal)

Challenges

Need for Internal Education within R&D

- Siloed approaches across TAs
- Varying levels of adoption of PE efforts
- Inconsistent development/use of PED in submissions

Inconsistent Interpretation of Guidances

- Differences in recommendations for type/quality/quantity of PED collected
- Differences in how/whether recommendations are adopted

Emerging Good Practices

Consistency in Communication Across TAs

- Increased communication within R&D and TAs to allow consistent adoption of type/quality of PE practices
- PED incorporated in regulatory submissions

Enterprise-wide PE Strategy

- Centralized function across R&D
- Consistent approaches across TAs and regulatory function
- Consistency whether/which PE activities are implemented and whether/which PED are included in regulatory submissions

Process | Sponsors (External)

Challenges

Lack of Dedicated Communication Pathway for PE and PED

- Absence of effective communication pathways to discuss PE or PED collection that is "fit for purpose" between sponsors and regulators

Omission of Patient Preference Data in Regulatory Review

- Regulator sticking to SOP in clinical trial approaches; contrasts with patient feedback to sponsors

Understanding of Patient Preference Data (PPD)

- Lack of understanding of meaningful PPD
- Patients may prefer quality of life measures vs. survival in assessing benefit/risk
- Evaluation of safety and efficacy vary by preconceived perspective on the severity of a disease vs perceived patient perspective

Emerging Good Practices

Develop "fit for purpose" Guidance/ Opportunities

- Guidances provide current thinking from regulators
- Early "fit for purpose" discussions between sponsors and reviewing divisions

Continuous Evolution of PE

- Frequent dialogue between sponsors, regulators, patient organizations and patients
- Sponsors incorporating dynamic patients' perspective and responses throughout R&D and post-launch

Process | Patient Organizations

Challenges

Different Patient Identification and Recruitment Strategies

- Each organization has different approach to identifying and recruiting patients
- Lack of uniformity for regulators and sponsors

Traditional Approaches Outweigh Innovation

- Innovative solutions are being compared to old models / tools

Emerging Good Practices

Proactively Identify and Engage Patients

- Forming patient cohorts to support recruitment efforts in clinical trials, disease state understanding, etc.

Focused Collaboration

- Identify opportunities for organizations across the industry to work with the end in mind

Disease Coalition in Collaboration with Sponsors

- A collaborative initiative between sponsors and patient organizations to better understand and incorporate patient needs

Process | Challenges Poll Question

Sponsor – Internal Operations

Need for Internal Education within R&D

Inconsistent Interpretation of Guidances

Sponsor – External Engagement

Lack of Dedicated Communication Pathway for PE and PED

Omission of Patient Preference in Regulatory Review

Lack of Understanding of Patient Preference

Patient Organizations

Different Patient Identification and Recruitment Strategies

Traditional Approaches Outweigh Innovation

Select your top three process-related challenges

Select your top three process-related challenges:

Need for Internal Education within R&D

Inconsistent Interpretation of Guidances

Lack of Dedicated Communication Pathway for PE and PED

Omission of Patient Preference in Regulatory Review

Lack of Understanding of Patient Preference

Different Patient Identification and Recruitment Strategies

Traditional Approaches Outweigh Innovation

Select your top three process-related challenges:

- Need for Internal Education within R&D
- Inconsistent Interpretation of Guidances
- Lack of Dedicated Communication Pathway for PE and PED
- Omission of Patient Preference in Regulatory Review
- Lack of Understanding of Patient Preference
- Different Patient Identification and Recruitment Strategies
- Traditional Approaches Outweigh Innovation

Are there additional process-related challenges?

Include additional process-related challenges:

TOOLS

Instruments/platforms/techniques for PE and generation of PED in clinical trials and for submissions

Hot off the press…new "tools" for patients, caregivers and clinicians

FDA U.S. FOOD & DRUG ADMINISTRATION

FDA Announces First of Its Kind Pilot Program to Communicate Patient Reported Outcomes from Cancer Clinical Trials

The U.S. Food and Drug Administration today launched Project Patient Voice, an initiative of the FDA's Oncology Center of Excellence (OCE). Through a new website, Project Patient Voice creates a consistent source of publicly available information describing patient-reported symptoms from cancer trials for marketed treatments. While this patient-reported data has historically been analyzed by the FDA during the drug approval process, it is rarely included in product labeling and, therefore, is largely inaccessible to the public.

"Project Patient Voice has been initiated by the Oncology Center of Excellence to give patients and health care professionals unique information on symptomatic side effects to better inform their treatment choices," said FDA Principal Deputy Commissioner Amy Abernethy, M.D., Ph.D. "The Project Patient Voice pilot is a significant step in advancing a patient-centered approach to oncology drug development. Where patient-reported symptom information is collected rigorously, this information should be readily available to patients."

Patient-reported outcome (PRO) data is collected using questionnaires that patients complete during clinical trials. These questionnaires are designed to capture important information about disease- or treatment-related symptoms. This includes how severe or how often a symptom or side effect occurs.

Patient-reported data can provide additional, complementary information for health care professionals to discuss with patients, specifically when discussing the potential side effects of a particular cancer treatment. In contrast to the clinician-reported safety data in product labeling, the data in Project Patient Voice is obtained directly from patients and can show symptoms before treatment starts and at multiple time points while receiving cancer treatment.

Your SparkNotes:

- "Project Patient Voice" initiative was announced June 23rd
- The pilot, being conducted through the Oncology Center of Excellence (OCE)
- On-line platform for patients/caregivers/HCPs
- A catalog of publicly available **P**atient-**R**eported **S**ymptom **D**ata (**PRSD**) from trials for marketed treatments
- Intended to be used by HCP when discussing symptoms of treatment: side effects across time,
- How are these data different from "drug labeling?"
  - Labeling: **static,** from CRFs, clinicians and reports of Adverse Reactions, not accessible to public
  - PRSD: benefits/risks from the patient's voice directly (**same trial**), real-time, complements other data

https://www.fda.gov/about-fda/oncology-center-excellence/project-patient-voice

Tools | Sponsors (Internal)

Challenges

Limited Generation and Application of PED

- Choose to develop and use limited COAs
- Not willing to innovate, less understood/accepted study endpoints and COA measures

Design Studies that Provide Usable PED

- Despite PFDD efforts and guidances, lack of clarity about type, quality and quantity of PED that should be collected
- Lack of willingness of reviewing divisions to accept these data

Emerging Good Practices

Develop Well-Designed PE Activities

- Engaging patients early in developing meaningful outcomes of clinical benefit for regulators
- Engaging patients in generation of meaningful PED

PED Development and Assessment Dashboard

- Interactive dashboard including elements such as:
  - Symptoms of and natural history disease
  - Impact of on functioning and quality of life
  - Experience with treatment(s) and meaningful outcomes
  - Relative importance of other issues defined by patients

Tools | Sponsors (External)

Challenges

Lack of Efficiency for Qualification of COA and Drug Development Tools (DDTs)

- Established pathways for qualification of DDTs and COAs are cumbersome and time-consuming

Overreliance on PROs

- Patient insights are predominantly measured by PROs, and other COAs and DDT are not being pursued
- Regulators appreciative of incorporation of concept of PED, but default to traditional regulatory requirements and outcome measures

Emerging Good Practices

Develop Timely and Efficient Qualification Pathways

- Re-evaluation of qualification and procedures for DDTs and COAs using early PE activities and PED

PED Strategy Template

- Providing feedback to sponsors on:
  - Proposed PE strategy and expected value in determining clinical benefit
  - Planned PE activities, purpose and how the activities reflect the expected value
  - Proposed regulatory engagements through the development process

Tools | Patient Organizations

Challenges

Lack of Standardized Toolkit

- No SOP for recruitment
- Limited available tools to support recruitment efforts

Individual Data Governance

- Platforms/registries built with limited interoperability
- Existing data do not facilitate product development

Emerging Good Practices

Standardized Toolkit

- Patient registries to support recruitment, clinical endpoint definitions, clinical outcome measures, product development efforts, etc.

Apply Emerging Technology

- Collecting data and applying emerging technologies such as Natural Language Processing (NLP) to generate additional insights

Use Flexible Targeted Communications

- Direct communication with patients (e.g. social media platforms), offering a dedicated community space

Tools | Challenges Poll Question

Sponsor – Internal Operations

Limited Generation and Application of PED

Designing Studies that Provide Usable PED

Sponsor – External Engagement

Lack of Efficiency for Qualification of COA and Drug Development Tools (DDTs)

Overreliance on PROs

Patient Organizations

Lack of Standardized Toolkit

Individual Data Governance

Select your top three tools-related challenges

Select your top three tool-related challenges:

Limited Generation and Application of PED

Designing Studies that Provide Usable PED

Lack of Efficiency for Qualification of COA and Drug Development Tools (DDTs)

Overreliance on PROs

Lack of Standardized Toolkit

Individual Data Governance

Select your top three tool-related challenges:

Limited Generation and Application of PED

Designing Studies that Provide Usable PED

Lack of Efficiency for Qualification of COA and Drug Development Tools (DDTs)

Overreliance on PROs

Lack of Standardized Toolkit

Individual Data Governance

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Are there additional tools-related challenges?

Include additional tool-related challenges:

Please add any additional Emerging Good Practices that you have found:

A poll of the polls

Select your top priority now:

Culture
Assumptions/notions that influence PE and use of PED

Cross – Organizational Alignment
Infrastructure within R&D/other functions that facilitate or impede collaboration on PE and PED development

Process
Frameworks to integrate PE and develop PED in R&D and for submissions

Tools
Instruments/platforms/techniques for PE and generation of PED in clinical trials and for submissions

Which theme do you believe is a top priority for your organization?

Culture

Cross-Organizational Alignment

Process

Tools

Panel Discussions

Meet our panelists

Nicholas Brooke
Panelist
Founder and Executive Director, The Synergist.org

Eleanor Perfetto, PhD, MS
Panelist
Executive Vice President, National Health Council

Jessica Scott, MD, JD
Panelist
Head of R&D Patient Engagement, Takeda Pharmaceuticals

Wendy Sanhai, PhD, MBA
Moderator
Specialist Leader, Deloitte Consulting

When poll is active, respond at **PollEv.com/ped2020**

Questions for our panel

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Next Steps

Eleanor Perfetto, PhD, MS

Executive Vice President, National Health Council

When poll is active, respond at **PollEv.com/ped2020**

In one word, please state what comes to mind when you think of PE and PED:

Start the presentation to see live content. For screen share software, share the entire screen. Get help at **pollev.com/app**

…over coming months we will explore opportunities to enhance the use of PED…

Up next…

Identify Stakeholders

60+ stakeholders identified including:
- patient organizations,
- not-for-profit organizations
- biopharma

Conduct Interviews

Conducted 35 interviews across representatives from stakeholder groups

Document Findings

Identified themes, and aggregated findings into categories:
- current challenges
- emerging good practices
- potential future practices

Share and Validate Current State Findings across Broader Stakeholder Group

Sharing findings of current state during PEOF Summit assess gaps and level of interest progressing further

Stand up working group for optimal future state

Create working group of key stakeholders to identify and prioritize PE topics/gaps and begin discussions on how to address these

Goal: To develop better communications pathways between stakeholders and regulators

Looking beyond

Sept 24

Stand up working group

Create working group of key stakeholders to identify and prioritize PE topics/gaps and begin discussions on opportunities to address these

Share and Discuss Recommendations for a Future State

Create and Align on Roadmap

Launch Pilot Program

Future Steps

As we continue to dive into opportunities to enhance patient engagement efforts, to what degree would you like to be involved

Interested in being part of the co-creation efforts

I can provide input as needed

Keep me on the distribution list

Not involved

If you would like to stay involved going forward, please provide us with your name and email:

Last comments from panel members…

Thank you all for your engagement and participation today!

Stay tuned for a follow up session on September 24th

Let’s work together to spread the word

#PEOF2020

@imi-paradigm

@eupatients

@PFMDwithPatient

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Patients Active in Research and Dialogues for an Improved Generation of Medicines

PATIENT FOCUSED MEDICINES DEVELOPMENT

EUPATI
European Patients’ Academy on Therapeutic Innovation

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