Partnering with Patients – Alignment on Terminologies and Definitions for Global Harmonization

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Background

Measuring what is most meaningful to patients is increasingly recognized as an essential component of research design and conduct. To support this imperative, health authorities have recently published guidelines for industry best practices for patient centric research (PCR).

This signifies a shift from past rhetoric and intended patient-centricity to a new model of active patient partnership for drug development.

Objective

This research aimed to landscape PCR related guidelines and recommendations. We identified and characterized published PCR related documents and extracted terminologies according to their purpose to highlight discrepancies and commonalities among terminology definitions.

This poster presents the first output of ongoing research aiming to landscape PCR related guidelines and recommendations and to identify terminologies and highlight discrepancies and commonalities among term definitions.

Methods

A targeted review searching the web-based sites of participating regulatory authorities for the International Consortium for Harmonization (ICH) identified relevant publications for inclusion. A structured extraction template was developed. Thematic analysis methods were used to code elements in terminologies and in their respective definitions to categorize into subthemes and themes. The list of sources to search was based on the ICH list of regulatory members (European Medicines Agencies (EMA) / United States Food and Drug Administration (US FDA) – Patient-Focused Drug Development ICH Reflection Paper), however we restricted the list to European, United States of America, and international organizations. The analysis was conducted with qualitative analysis software (Nvivo, RITME, v.14) to enable codes to be assigned to themes and to facilitate the organization of the data.

Results

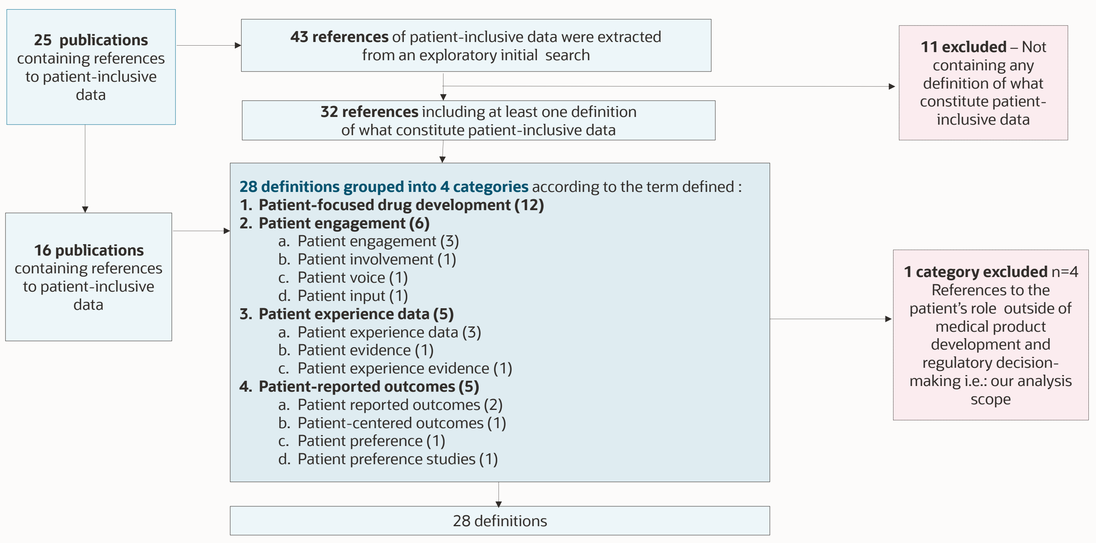
25 full text publications were screened. 16 publications (13 slide or presentation decks, 4 reports, 4 guidance documents, 1 executive summary, 1 glossary, 1 overview page and 1 statement document) were kept in the analysis (Figure 1). These publications are from various organizations:

- Eight from the US FDA, 3 from the Council for International Organizations Of Medical Sciences (CIOMS),
- Two from the EMA,
- Two from the ICH (EMA and US FDA)
- One from the European Federation of Pharmaceutical Industries and Associations (EFPIA).

The median publication year is 2022 (IQR: 2021, 2023). After the inductive analytical process (Figure 1), 28 PCR related definitions were identified, coded, and analyzed for commonalities and discrepancies:

- Nine definitions from the CIOMS,
- Eight from the US FDA,
- Six from the EMA,
- Three from the EFPIA
- Two from the ICH.

Figure 1. Inductive analytical steps diagram



These 28 definitions correspond to 12 different terms, which have been grouped into 4 thematic categories, based on the similarity of the term's meaning:

1	Patient-Focused Drug Development	PFDD	➔ 1 term:	Patient-Focused Drug Development	➔ 12 consistent definitions across 12 publications
2	Patient Engagement	PE	➔ 4 terms:	Patient engagement, Patient involvement, Patient voice, Patient input	➔ 6 definitions across 5 publications
3	Patient-Experience Data	PED	➔ 3 terms:	Patient experience data, Patient evidence, Patient experience evidence	➔ 5 definitions across 3 publications
4	Patient-Reported Outcomes	PRO	➔ 4 terms:	Patient-reported outcomes, Patient centered outcomes, Patient preferences, Patient preference studies	➔ 5 definitions across 2 publications

The US FDA is consistent with the term and associated definition used around PCR. The US FDA used only one term, PFDD, associated with the following definition (Figure 2):

"**A systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into the development and evaluation of medical products throughout the medical product life cycle.**".

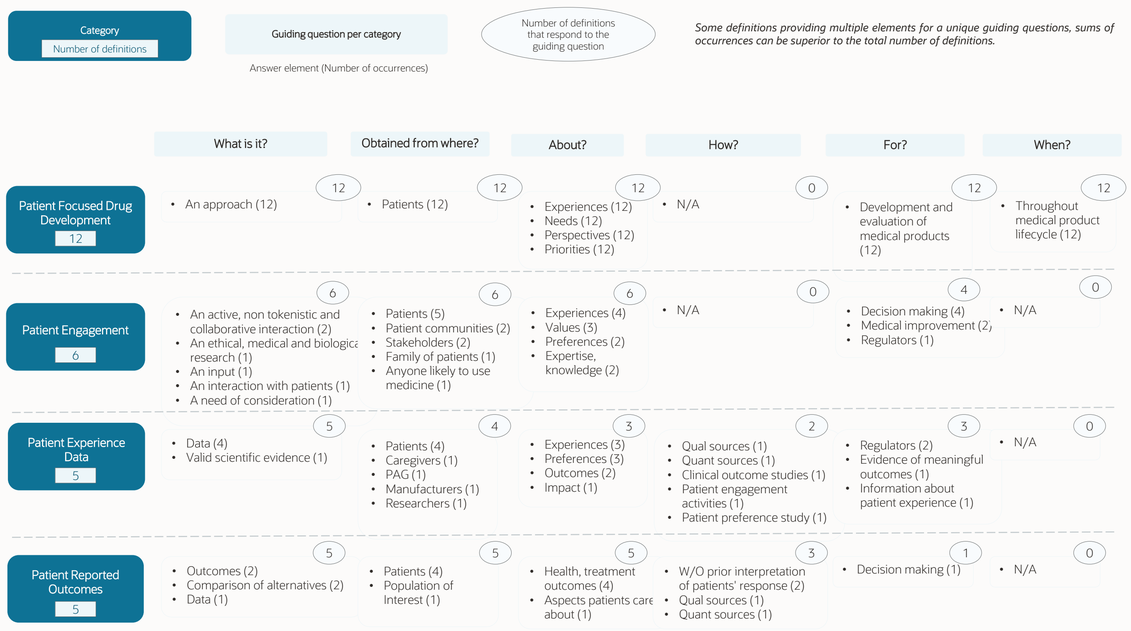
This definition has been also used by the CIOMS and the ICH but in a slightly modified definition: "A systematic approach **to capture** patients' experiences, perspectives, needs and priorities, and to incorporate them meaningfully into the development and evaluation of a medicinal product throughout its lifecycle.".

The difference between those 2 definitions resides in the positioning of the organization. The FDA proposes a definition which allows to recognize this type of data and ensure this data is collected, while the CIOMS and the ICH propose a definition which recommends collecting this type of data.

Unlike the US FDA which is consistent in using the term PFDD, the other sources use different terms and definitions. The CIOMS describes Patient Engagement as "**The active, non-tokenistic and collaborative interaction between patients, the patient community and other stakeholders, where decision making is guided by patients' contributions as partners, recognizing their unique experiences, values and expertise.**" in 2 documents published in September 2022 and January 2023. The other definitions grouped into the Patient Engagement category differ from the CIOMS's one

The subgroups of Patient Experience Data and Patient Reported Outcomes do not present any harmonized definitions.

Figure 2. Comparison for thematic analysis on the 4 categories, 28 definitions based on description questions



The thematic category PFDD was the most recurrent overall with a single term and almost consistent definition. Only the PFDD category proposes the same definition each time. None of the categories propose answers element to all the guiding questions. Only two categories (PED and PRO) specify how to collect such data. By comparison, this is the only question, for which PFDD category definition does not answer. There is a high variety of answers to the questions "what is it" and "About/ on?" between categories. In all categories, the data should be obtained from the patient. "Patient" is also the most recurring word in all the definitions (Figure 3).

Figure 3. Top 15 of words most used in the 28 definitions (number of occurrences)

Conclusion

Given the dynamic nature of patient priorities and evolving treatment landscapes, the global ICH multistakeholder collaboration to achieve alignment for PCR design and methods is essential. The US FDA proposes a consistent definition of PFDD, sometimes adopted by others. However, the diversity of definitions shows that some organizations need to specify other aspects of PCR, proposing different terms and definitions to refine the role and use of the patient centric research. If there is no exact alignment on the term and definition for PCR, the different definitions do not contradict but rather complement each other. To move forward with active patient partnership, further guidance on how to best collect PCR should be developed.

Contact

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