Current Research Status and Applications of Patient Experience Data under the Patient-Centered Care Paradigm: A Postprint

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Abstract

As individuals with firsthand disease experience, clinical trial participants, and contributors to medical practice, patients possess the most direct and explicit understanding of disease experiences and needs. Drug regulatory agencies in multiple countries have successively proposed the "patient-centered" concept, aiming to enhance patient-initiated participation in medical decision-making, drug development, and clinical trials through "patient experience data" encompassing patient experiences, perceptions, and needs. This article systematically reviews the evolution of patient experience data, collates its definition, scope, sources, and collection methods, and analyzes its application value and key aspects to provide references for future research. The study reveals that although patient experience data can be widely applied in multiple domains, including the development of clinical outcome assessment instruments, drug development and regulatory decision-making, and optimization of clinical trial design, there remains a lack of standardized collection processes, standardized identification and screening techniques, and proper methodological guidance for application. Based on these findings, future efforts require collaborative cooperation among multiple stakeholders, including researchers, patient communities, and government agencies, to jointly improve and advance the collection, identification, screening, and application of patient experience data.

Full Text

Research Status and Application of Patient Experience Data Under the "Patient-Centered" Concept

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Abstract

As direct experiencers of illness, participants in clinical trials, and engaged parties in medical practice, patients possess the most immediate and profound understanding of their disease experiences and health needs. In response, drug regulatory agencies worldwide have successively embraced the "patient-centered" concept, seeking to enhance pathways for active patient participation in medical decision-making, drug development, and clinical trials through the systematic use of patient experience data (PED)—information capturing patients' experiences, perspectives, and needs. This article systematically reviews the evolution of PED, collating definitions, scope, sources, and collection methods while analyzing its application value and critical implementation components to inform future research. Our findings indicate that although PED can be widely applied in developing clinical outcome assessment tools, supporting drug development and regulatory decision-making, and refining clinical trial design, significant challenges remain. These include the absence of standardized collection protocols, validated identification and screening methodologies, and robust methodological guidance for appropriate application. Addressing these gaps will require collaborative efforts among researchers, patient communities, government agencies, and other stakeholders to establish and promote best practices for PED collection, identification, screening, and utilization.

Keywords: Health services administration; Patient-centered; Patient experience data; Clinical decision-making; Application and challenge

1. Concept of Patient Experience Data

Different institutions have developed nuanced descriptions of PED. The 2016 U.S. 21st Century Cures Act defines PED as experience information about a disease or condition provided by "any person or entity" —including patients, family members, caregivers, or patient advocacy organizations—encompassing how the disease or its treatment affects patients' lives and their treatment preferences. China' s Center for Drug Evaluation (CDE) defines PED as "all information regarding experiences, perspectives, needs, and preferences about diseases and related treatments provided by patients, their families, guardians, or caregivers." The European Medicines Agency (EMA) describes PED as "data collected through various patient engagement activities and methods to describe

patients' experiences with their health status, symptoms, disease course, treatment preferences, quality of life, and health impacts."

The U.S. Food and Drug Administration (FDA) further elaborated on this concept through its Patient-Focused Drug Development (PFDD) guidance, characterizing PED as encompassing patients' experiences, perspectives, needs, and priorities across five domains: (1) symptoms and conditions and their impact on daily functioning and quality of life; (2) disease progression over time, including functional impacts and evolving patient experiences; (3) treatment experiences, including therapy-related symptoms and burdens; (4) perspectives on treatment outcomes and trade-offs among different results; and (5) views on disease impacts and benefit-risk trade-offs of treatments. In essence, PED represents multi-dimensional data, collected through diverse channels, that reflects patients' experiences, perspectives, needs, and preferences regarding their disease and its treatment, capturing the holistic impact on patients' lives.

2. Evolution of PED Across Countries and Institutions

As PED has gained increasing importance in regulatory review and health technology assessment, agencies including the FDA, CDE, EMA, UK' s MHRA, and Japan' s PMDA have issued guidance documents to integrate PED into medical decision-making and drug development. This section examines the developmental trajectories of PED at the FDA, CDE, and EMA.

2.1 FDA

The FDA' s journey toward incorporating patient perspectives began with the 2002 Best Pharmaceuticals for Children Act (BPCA), which aimed to enhance pediatric drug efficacy and safety by ensuring children' s needs were considered during drug review. In 2004, the FDA published "Innovation/Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products," a blueprint for modernizing drug development and regulatory processes that emphasized incorporating reliable patient insights to ensure technologies met actual patient needs.

A significant shift occurred in 2009 with the "Guidance for Industry: Patient-Reported Outcome Measures—Use in Medical Product Development to Support Labeling Claims," which encouraged early-stage drug development to extract meaningful benefit concepts reflecting patients' physiological, physical, or functional states, subsequently developing PRO instruments to evaluate whether products adequately addressed patient needs. The 2010 Patient Protection and Affordable Care Act (ACA) established the Patient-Centered Outcomes Research Institute (PCORI), institutionalizing patient engagement in healthcare reform. The 2012 Food and Drug Administration Safety and Innovation Act (FDASIA) and Prescription Drug User Fee Act V (PDUFA V) further strengthened patient involvement in drug development and regulatory decisions, mandating incorporation of patient voice into benefit-risk assessments and drug review

processes.

The landmark 21st Century Cures Act formally codified PED in 2016, requiring the FDA to develop clear guidance on using PED in regulatory decisions and mandating transparency and accountability in its application during drug reviews. Between 2020 and 2023, the FDA' s PFDD guidance series released four foundational documents detailing how to collect meaningful PED and integrate it throughout drug development and review processes. During this period, the FDA convened multiple PFDD meetings to capture patients' priorities, perspectives on benefits and risks, and desired involvement in drug development, providing platforms for stakeholders to hear patient voices directly [Figure 1: see original paper].

2.2 CDE (China)

China' s regulatory evolution has similarly embraced patient-centered principles. In 2021, CDE issued the "Guidance for Industry: Patient-Reported Outcomes in Drug Clinical Research (Trial)," emphasizing the need to capture patient perspectives, insights, and needs throughout the drug lifecycle. This was followed in 2022-2023 by a comprehensive suite of guidances: "General Considerations for Organizing Patient Participation in Drug Development (Trial)," "Technical Guidance for Patient-Centered Clinical Trial Design (Trial)," "Technical Guidance for Patient-Centered Clinical Trial Implementation (Trial)," and "Technical Guidance for Patient-Centered Benefit-Risk Assessment (Trial)." These documents marked China' s first formal integration of patient-centered concepts into regulatory guidance, proposing that "drug research, design, implementation, and decision-making from the patient perspective will facilitate the efficient development of drugs that better meet patient needs and deliver clinical value."

In 2024, CDE launched the "Patient-Centered Rare Disease Drug Development Pilot Program," a pioneering initiative to implement patient-centered principles in rare disease drug development, guiding sponsors to systematically incorporate patient voice throughout the development process. Additionally, in November 2024, CDE released the draft "Technical Guidance for Patient-Centered Clinical Research of New Traditional Chinese Medicine Drugs," marking the first integration of patient-centered concepts with traditional Chinese medicine theory. This guidance advocates incorporating patient voice and using PED to develop clinical outcome assessment tools aligned with TCM' s therapeutic characteristics, establishing diversified evaluation standards and methods to clearly articulate TCM' s clinical efficacy.

2.3 EMA

The EMA has progressively emphasized PED collection, analysis, and application in drug development and regulatory decision-making. In 2018, the European Patients' Academy on Therapeutic Innovation (EUPATI) published guidance on integrating patient needs, preferences, and opinions into drug develop-

ment and regulation. A 2022 multi-stakeholder workshop convened by EMA aimed to achieve consensus on PED, with participants agreeing on key domains including patient engagement, patient-reported outcomes, patient experience evidence, and patient preferences, while identifying challenges and strategies for improvement.

In 2023, EMA published an executive summary acknowledging that "although the EU has made good progress on PED, it has not yet been systematically integrated into all aspects of drug development and regulation." The agency announced plans to release a reflection paper discussing PED definitions, value, methodologies, data sources, implementation challenges, and EMA' s role in scientific advice and qualification of novel methodologies.

3. Scope, Sources, and Collection Methods of PED

3.1 Scope of PED

The 21st Century Cures Act describes PED scope as encompassing patient symptoms and natural history, disease impact on function and quality of life, treatment experiences, input on important outcomes, treatment preferences, and the relative importance of patient-defined issues. FDA' s PFDD guidance recommends collecting and evaluating PED across multiple dimensions: (1) disease and treatment impacts, including symptoms, most bothersome complaints, disease management burden, treatment burden, and clinical trial participation burden; (2) perspectives on potential and current treatments, including expected benefits, risk tolerance, acceptable benefit-risk trade-offs, and attitudes toward uncertainty; (3) views on unmet medical needs and available treatment options; and (4) understanding of disease natural history, including progression, severity, and chronicity.

CDE emphasizes collecting: (1) patient understanding of disease (most concerning symptoms, quality-of-life impacts); (2) perspectives on current treatments (accessibility, safety and efficacy limitations, adherence, preferences); (3) unmet clinical needs; (4) expectations for potential treatments and acceptable risks; and (5) clinical trial participation burden and engagement methods. EMA identifies five domains: (1) patient data from engagement activities; (2) scientifically validated patient experience evidence (PEE); (3) patient engagement (PE) activities; (4) patient preferences (PPs) regarding treatment outcomes; and (5) patient-reported outcomes (PRO). While agencies differ in their descriptions, PED generally includes disease-related information (symptoms, quality-of-life impact, natural history), treatment-related information (current/potential treatment views, burden, preferences), and other aspects (trial participation burden, unmet needs). However, this represents a preliminary summary, and specific scope requires further stakeholder deliberation.

3.2 Providers of PED

The 21st Century Cures Act identifies PED providers as patients, family members, caregivers, patient advocacy organizations, disease research foundations, researchers, and pharmaceutical manufacturers. FDA' s PFDD guidance recommends direct patient reporting as primary, while acknowledging that valuable information may also come from caregivers, patient advocates, clinicians, and others when direct data are limited. CDE recognizes patients (not only trial subjects but all individuals with specific diseases), family members, guardians, caregivers, and patient organizations as sources. EMA considers PED to originate from direct patient reports. In practice, collection should prioritize direct patient input, supplemented by caregivers and advocacy organizations when necessary.

3.3 Sources and Channels for PED Collection

The 21st Century Cures Act suggests obtaining PED through disease registries, natural history studies, patient focus groups, survey data, clinical outcome assessment (COA) data, and patient preference studies. FDA' s PFDD guidance recommends collection throughout the medical product lifecycle or independently, via clinical trials, observational studies, advisory committees, public meetings, and novel platforms like social media and verified online patient communities. CDE emphasizes pre-designed clinical trials, patient preference studies, natural history studies, interviews/questionnaires, expert consultations, and patient engagement meetings. EMA recognizes multiple channels including natural history studies, COAs, patient preference studies, observational studies, qualitative research (focus groups), and quantitative research (surveys). Given this diversity, FDA developed the Patient Experience Data Table as part of drug review documents to identify PED sources in NDAs, BLAs, and efficacy supplements, facilitating regulatory review [Figure 2: see original paper].

3.4 Methods for PED Collection

Regulatory agencies recommend qualitative, quantitative, and mixed-methods approaches, selected based on research objectives. Specific methods include one-on-one interviews, focus groups, patient meetings, observation, surveys, audiovisual materials, and social media data collection. For example, STAUNTON et al. used social media and patient interviews to extensively collect early-stage Parkinson' s patients' experiences and disease impacts, identifying patient-prioritized symptoms for clinical benefit assessment. YANG Zhi et al. employed qualitative methods to understand treatment burden among elderly patients with multiple chronic conditions, providing theoretical foundations for developing burden assessment tools.

4. Application Value of PED

4.1 Capturing Patient Experiences and Needs

As direct experiencers of disease and treatment, patients provide the most accurate insights into their conditions. PED offers direct information about disease impacts, enabling clinicians and researchers to develop more comprehensive understandings. Numerous studies have collected patient perspectives on specific diseases to deepen understanding. KARAGIANNIS et al. used exploratory sequential studies to capture type 2 diabetes patients' treatment preferences and perspectives on efficacy and drug attributes. Such research demonstrates how PED illuminates patient priorities and informs product development.

4.2 Developing Clinical Outcome Assessment Tools

Clinical Outcome Assessments (COAs) describe or reflect patients' symptoms, functioning, or survival status, serving as primary or secondary endpoints in clinical trials to demonstrate intervention effects, clinical value, and patient benefit-risk profiles. Both development and validation of COAs require extensive PED support. FDA's Drug Development Tool Qualification Program (DDTQP) establishes COA qualification procedures to ensure scientific rigor, with seven PROs currently qualified. CONNELLY et al. used literature reviews to capture PED in systemic lupus erythematosus, developing novel COA tools for SLE drug development and efficacy evaluation.

4.3 Drug Development and Regulatory Decision-Making

PFDD guidance describes integrating patient experiences and needs into drug review and approval processes, ensuring patient perspectives become integral to drug development and regulatory review. Several manufacturers have successfully navigated this pathway. Janssen Pharmaceuticals collected PRO and patient preference data to demonstrate the efficacy and safety of esketamine nasal spray for treatment-resistant depression, securing FDA approval and providing a new therapeutic option. KIEFFER' s evaluation of 59 FDA-approved drugs in 2018 found that 48 applications included the required PED table, with 34 (70.8%) incorporating PED into their review process.

4.4 Clinical Trial Design and Implementation

Patient-centered research focuses on integrating reliable, meaningful, and representative patient experiences into study design and implementation to enhance relevance and impact. CDE' s three guidances on patient-centered trial design, implementation, and benefit-risk assessment describe how to incorporate PED throughout research planning. The U.S. National Health Council' s patient-centered real-world evidence working group explored PED integration into real-world data (RWD) studies to generate patient-centered real-world evidence (RWE). OEHRLEIN et al. used the Patient Experience Mapping Toolbox

to collect and identify meaningful PED for real-world study design, though application methods require further development.

4.5 Other Applications

PED can improve patient experiences and promote active patient participation in care. CDE' s guidance explicitly addresses building patient engagement practices that ensure scientific rigor while optimizing trial participation experiences and reducing burden through innovative approaches like remote visits. Additionally, PED informs policy development and healthcare service improvement, driving continuous optimization of care delivery.

5. Challenges Facing PED

5.1 PED Collection Challenges

Provider-Related Issues: PED quality may be influenced by providers' emotional states, cognitive abilities, cultural backgrounds, language, and education levels. As healthcare advances, patient experiences evolve, requiring adequate self-understanding for meaningful participation. LOWELL et al.' s qualitative study of Australian First Nations speakers revealed that linguistic and cultural differences can compromise PED authenticity, leading to inaccurate data, omitted information, or erroneous conclusions.

Collector-Related Issues: Standardized collection processes are essential for data consistency and comparability. PINTO' s survey of 50 experienced researchers found that while 76% had dedicated PED collection units, only 33% had standardized protocols. Different PED types present distinct challenges: PRO tools require substantial time and cost for design and validation, while patient preference and natural history studies lack widely accepted standards. Furthermore, researchers face communication gaps with regulators—72% reported discussing PED with FDA, but challenges in comprehensive, effective communication raise concerns about meeting regulatory requirements. All respondents advocated for clear, standardized meeting pathways to discuss PED collection and use.

Patient Engagement Integration: Patient engagement (PE) involves stakeholders sharing experiences, perspectives, needs, and priorities, positioning patients as key informants for public health missions. While FDA/EMA Patient Engagement Clusters have enhanced participation, studies show integration pathways between PE and PED remain underdeveloped.

5.2 Identifying and Selecting Meaningful PED

EMA designates scientifically validated PED as Patient Experience Evidence (PEE). The critical challenge lies in identifying clinically relevant data from vast PED collections to generate PEE for patient-centered drug development and regulatory decisions. Research on collecting, interpreting, and using patient

data shows that while its importance is recognized, methods for aggregating, reporting, and demonstrating representativeness and relevance require further investigation.

5.3 Challenges in Trial Design and Regulatory Decision Integration

Trial Design Integration: Current patient-centered research must address both engagement methods and PED application to strengthen study design. While CDE' s guidances outline strategies for PED-driven research objectives, target population selection, comparator choice, and endpoint selection, the agency acknowledges these represent evolving perspectives requiring continuous refinement. NHC' s RWE guidance lacks explicit descriptions of PED application in study design, and though scholars have proposed 13 recommendations for PED integration in real-world studies, limited acceptance hinders widespread implementation.

Regulatory Decision Integration: Despite FDA, EMA, and CDE efforts to promote PED, stakeholders call for additional regulatory guidance, greater transparency in PED evaluation, and clearer standards for evidentiary use. EMA workshop participants noted ambiguity about whether PED qualifies as valid evidence for decision-making, demanding enhanced process transparency. PINTO et al. found most researchers (62%) were unclear about where to submit PED in regulatory filings or how to use it to guide decisions.

5.4 Other Challenges

Digital transformation offers opportunities for PED collection and analysis but also presents challenges. The Patient-Focused Medicine Development (PFMD) organization created the Global Patient Experience Data Navigator to facilitate PED collection, analysis, and global dissemination across all diseases and stakeholders. The National Health Council' s Patient Experience Mapping Toolbox guides real-world study design, implementation, and translation. However, EMA cautions that digital tools raise concerns about data complexity, security, and patient trust, requiring collaborative industry efforts to address these issues.

Conclusion

PED is essential for realizing patient-centered healthcare, garnering attention from global institutions and finding widespread application in COA development, drug development, regulatory decision-making, and clinical trial design. However, significant challenges persist, including provider heterogeneity, limited collector-regulator communication, absence of standardized collection processes, and inadequate methodological guidance for identification, screening, and application. Effective PED utilization demands multi-stakeholder collaboration among researchers, patients, and government agencies to establish robust frameworks for collection, identification, screening, and implementation, ultimately ensuring patient voices meaningfully shape healthcare innovation and policy.

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Note: Figure translations are in progress. See original paper for figures.