Developing a Checklist for Patient Engagement in Rare Disease Value Research

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OBJECTIVE

Rare diseases present significant challenges to comparative clinical effectiveness research (CER) and health technology assessment (HTA). As advancements in identifying, diagnosing, and treating these diseases accelerate, there is an increasing demand for innovative research approaches. This project builds on prior work at the Center for Innovation & Value Research, emphasizing the integration of meaningful patient engagement into rare disease value research.

METHODS

In 2024, a Steering Committee of 15 members was formed to identify gaps in CER, HTA, and related methodologies that may create barriers to accessing emerging therapies (Figure 1). To inform the development of a checklist, we conducted eight stakeholder discussions with 46 participants, including patients, caregivers, payers, manufacturers, employers, regulators, and researchers. A targeted and gray literature review was also conducted to help identify the gaps, leading to a set of key recommendations for patient engagement in CER and HTA, particularly in the context of understanding outcomes important to rare disease patients.

In 2025, we built upon these findings by developing a systematic framework and checklist to ensure meaningful patient engagement in rare disease value research. A 19-member, multi-stakeholder Advisory Board, consisting of both original and new members, was convened. Several approaches were used to develop and refine the checklist, including six meetings, post-meeting surveys, and multiple rounds of review. Discussions focused on key issues such as the context of the checklist (e.g., identifying gaps or overlaps), format, intended audiences, wording, and dissemination plans.

To validate and improve its applicability, case studies on three different rare diseases will be conducted, allowing for iterative refinements to enhance its relevance and impact. The goal is to develop a core checklist that can be applied across most rare diseases, assessing its applicability in diverse contexts by selecting diseases that differ from one another (Figure 1).

RESULTS

We developed a checklist to support value researchers in integrating patient engagement throughout the processes, data analyses, and methods of patient-centered value research in rare diseases. The checklist includes both CER and HTA components, reflecting their interconnected nature.

To ensure broad applicability across rare diseases, the checklist offers high-level yet actionable guidance, accompanied by brief explanations, relevant examples, and links to supporting tools and resources where available. The structure of the checklist aligns with the typical research process and is organized into four key phases: 1) Initiation & Planning, 2) Execution, 3) Monitoring, and 4) Dissemination & Assessment (Figure 2).

Within each phase, key considerations are outlined with corresponding questions designed to guide reflection and assessment of patient engagement practices. For example, under the Initiation & Planning phase, one question focuses on “Budgeting for patient engagement activities,” emphasizing the importance of allocating resources for fair compensation, expense coverage, and training for patients.

Questions are formatted using rating scales (e.g., Likert-type) to support structured assessment. Additional items address areas such as co-creation with patients and bi-directional feedback mechanisms to foster meaningful and sustained engagement (Table 1).

Figure 1. Study Approach

Stakeholder Discussions and Literature Review

1. Steering Committee of 15 members was formed
2. Three roundtable discussions
3. Target and gray literature review

→ Result: Report with key recommendations for patient engagement in CER and HTA

Framework and Checklist Development

1. Advisory Board of 19 members was convened
2. Six meetings held to convert recommendations
3. Survey after each meeting to gather feedback
4. Checklist developed via multiple rounds of review

Case Studies for Validation

1. Advisory Board developed disease selection criteria
2. Working group selecting specific rare diseases for checklist pilot testing
3. Case studies on 3 different rare diseases will be conducted

CONCLUSION

This checklist underscores the importance of integrating patient engagement into the patient-centered value research process and serves as a tool for researchers and other stakeholders to systematically evaluate engagement. Moving forward, it can be used to identify gaps in rare disease research, promote continuous improvement in patient engagement, and foster a culture of accountability. By sharing this checklist, we hope to enhance rare disease value research, emphasizing the need for ongoing assessment and improvement of patient engagement processes.

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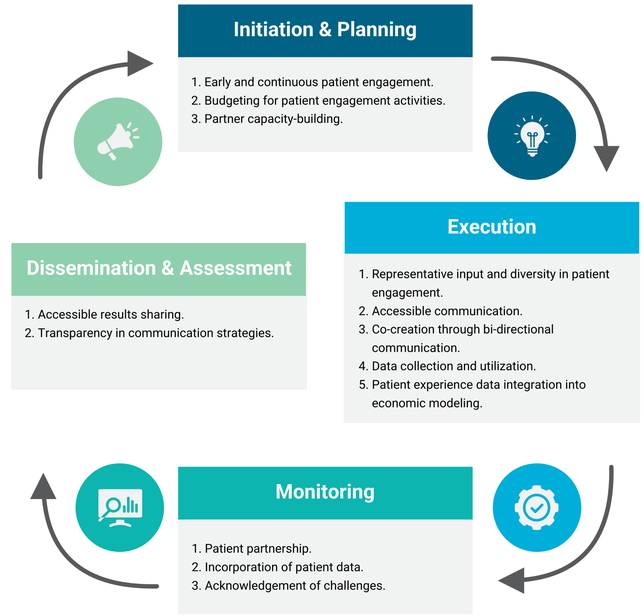
Table 1. Key Feedback for Checklist Development

Key Discussion Points	Results
Key audiences we should consider for this checklist	1) Researchers 2) Value Assessors 3) Policy Makers 4) Patient Groups
Format	1) Rating/Scoring Scale 2) Likert Scale (e.g., Strongly Agree, Somewhat Agree, Disagree) 3) Yes/No Questions
Level of detail	Moderate detail (key points + options/check boxes)
Key criteria to consider for selecting rare diseases for checklist case studies	1) Cause of disease 2) Age of onset 3) Clinical trajectory (including symptoms) 4) Diagnostic journey & barriers 5) Treatment development pipeline, resources & data 6) Health equity issues & barriers 7) Patient & caregiver burden

Figure 2. Overview of the Checklist



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