Center for Innovation
& Value Research

Rare Disease Patient Engagement Guidance and Checklist

The checklist focuses on integrating patient engagement and patient-centered outcomes into CER and HTA for rare diseases. It is designed to help users systematically address key elements and best practices for patient engagement, including in study design, data selection, analysis, and stakeholder engagement, both when initiating planning and throughout the research process. HTA-specific items in the checklist table are highlighted in orange.

INITIATION & PLANNING

Sub-Section	Objective	Checklist Questions	Response Options	Notes
1. Early and Continuous Patient Engagement	Ensure patient and caregiver experiences are integrated from the start and sustained throughout the research lifecycle, shaping design, implementation, and decision-making.	Have patients, caregivers, and patient advocacy groups (PAGs) been engaged early and throughout the research process (e.g., from early research planning phase to dissemination & assessment phase)?	☐ Yes ☐ Partially ☐ No	
		Are key outcomes selected for the research shown to be relevant to patients and caregivers, reflecting lived experiences?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
2. Budgeting and Patient Burden Considerations	Ensure resources are allocated to support patient engagement, including fair compensation, expense coverage, and training, while keeping engagement credible, feasible, transparent, and minimizing unnecessary burden on patients and caregivers.	Is there a budget to support fair compensation, reimbursement of expenses, and stakeholder training, including training for patients and patient representatives?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Have transparency, frequency/mode of engagement, and strategies to reduce burden and bias been addressed?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
3. Partner Capacity-Building	Ensure patients, researchers, and others involved have the skills and knowledge for effective and meaningful engagement in the value research process.	Have you participated in training programs to support effective patient engagement?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Have you used training programs designed to help rare disease patients understand and engage in the value research process?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	

EXECUTION

Sub-Section	Objective	Checklist Question	Response Options	Notes
1. Representative Input and Diversity	Ensure diverse patient and caregiver representation to capture comprehensive experiences and needs.	Considering relevant target population demographics (socioeconomic status, race/ethnicity, sex/gender, culture, geography, age, disability), have a diverse group of patients been included?	☐ Not at All ☐ Somewhat[a] ☐ Moderately[b] ☐ Substantially[c] ☐ Reasonably Representative	
		Are caregivers or proxies engaged for patients who cannot themselves be engaged (e.g., too young, severely cognitively impaired, other health reasons)?	☐ Yes ☐ Partially ☐ No ☐ Not Applicable	
2. Accessible Communication	Ensure clear communication channels for all stakeholders, especially patients and caregivers from diverse backgrounds, so they are easily able to contribute to the value research process.	Are communication methods designed using plain language, translations, or accessible formats (font size, color contrast, numeracy support) to clearly communicate research purposes and potential outcomes to patients?	☐ Yes ☐ Partially ☐ No	
		Have communications been adapted for geographic, cultural, or accessibility barriers?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Have patients or patient representatives been engaged in designing communications, including content, methods, and dissemination plans (e.g., lay summaries and early sharing with communities)?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
3. Co-Creation through Bi-Directional Communication	Ensure co-creation and bi-directional communication between patients and researchers.	Is there a structured process for interactive collaboration between patients and researchers throughout the study?	☐ Yes ☐ No ☐ Planned	
		Have study materials and methods been co-created with patient partners wherever applicable?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	

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[a] Representation on 1 dimension, but not others
[b] Representation on 2-3 dimensions, but not all
[c] Representation on >3 dimensions, but not all

EXECUTION (Continued)

Sub-Section	Objective	Checklist Question	Response Options	Notes
4. Data Collection and Utilization	Ensure transparency in data usage and ensure data used meaningfully benefits the rare disease patient community.	Are tailored approaches used to collect and apply patient experience data at different stages (pre-, during, post-treatment)?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are patients and caregivers informed about how their data are used?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are patient preferences or economic impacts (e.g., cost, access, and utilization) integrated into the research process?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are longitudinal data incorporated to track outcomes over time wherever applicable?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Have you engaged patients, caregivers, or other experts to help identify appropriate data sources and ensure that the data were patient-focused and representative?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are biomarker data considered, where relevant to disease progression and treatment response?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
5. Patient Experience Data in Economic Modeling	Ensure patient experience data are integrated and meaningfully inform the value assessment process.	When disease-specific outcomes are missing, have you explored using common outcomes across rare diseases, with disease-specific customization where needed?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are robust methods (mixed methods, PROMs, DCEs) being utilized and appropriately incorporated into the HTA process?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are patient preferences consistently integrated into the economic evaluation framework, ensuring alignment with what matters most to patients?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	

HTA-specific items in the checklist table are highlighted in orange.

MONITORING

Sub-Section	Objective	Checklist Question	Response Options	Notes
1. Continued Patient Partnership	Document meaningful patient partnership in value research and adherence to guidelines, ensuring it is monitored and adapted throughout.	Have you documented what you did or did not do (with a rationale) regarding patient engagement activities?	☐ Yes ☐ Partially ☐ No	
2. Incorporation of Updated Patient Data	Ensure that patient experience data is effectively integrated into value research and regularly updated as new data become available.	To what extent is evidence derived from patient experience data incorporated into value research?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Is there a plan to review and update findings as new patient experience data become available, or to conduct sensitivity analyses if updates are not feasible?	☐ Yes ☐ Partially ☐ No ☐ Not Applicable	
3. Acknowledgment of Challenges and Evidence Gaps	Acknowledge the unique challenges throughout rare disease value research and take specific actions to address these challenges.	Are the unique challenges of rare disease research clearly acknowledged in reports/ manuscripts/publications?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Have specific strategies been implemented to alleviate these challenges?	☐ Yes ☐ Partially ☐ No ☐ Not Applicable	
		Have regular check-in or updates of the monitoring items been planned/ made to support sustained engagement and adaptation as research progresses?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	

HTA-specific items in the checklist table are highlighted in orange.

DISSEMINATION & ASSESSMENT

Sub-Section	Objective	Checklist Question	Response Options	Notes
1. Accessible Results Sharing	Ensure that research results are shared back with patient and caregiver partners in meaningful, understandable, and culturally relevant ways, using multiple accessible formats (e.g., plain language, storytelling, videos, group discussions, or written summaries).	Have you documented what you did or did not do (with a rationale) regarding patient engagement activities?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are patient and caregiver partners appropriately recognized and acknowledged as authors, in acknowledgments, as presenters, and through other methods?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are dissemination outputs shared in a timely manner to ensure findings reach patients, caregivers, and stakeholders as early as feasible?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
2. Transparent Communication and Continued Feedback	Ensure clear, open, and timely communication of research goals, progress, findings, and the role of patient engagement to all stakeholders.	Are research goals, methods, and roles of patient engagement clearly communicated to all stakeholders at the start and through timely updates during and after the research process?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are data gaps, data privacy, and conflict-of-interest disclosures included in communication and dissemination plans?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	
		Are patients and caregivers actively involved in dissemination activities and is feedback from them sought and integrated to improve relevance, clarity, and trustworthiness?	☐ Not at All ☐ Somewhat ☐ Moderately ☐ Substantially ☐ Fully	