# Towards a Consensus on Patient-Centered HTA for Rare Diseases in the U.S.: Insights from Global Literature

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### INTRODUCTION

Traditional health technology assessment (HTA) often struggles to comprehensively capture representative information on outcomes and preferences that are important to patients, especially in rare diseases.

### OBJECTIVE

To review HTA-related recommendations from rare disease literature and online sources that can inform development of a patient-centered framework and checklist for patient engagement in rare disease HTA.

### METHODS

A review was conducted to identify articles on rare diseases and health technology assessment (HTA). The initial search in PubMed, restricted to English-language articles available before April 2023, yielded 279 articles. (**Figure 1**). Each article was screened for relevance to HTA tools and frameworks. Articles not directly related to these topics (n= 218) were excluded, with 61 articles remaining. An additional four relevant articles were identified through advisory board recommendations and sources encountered during the analysis phase, resulting in 65 articles for review.

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There is lack of consensus on approaches to conducting patientcentered HTA for rare diseases in the United States. This project aims to develop a patient-centered framework and checklist for enhancing patient engagement in rare disease HTA.

### RESULTS

This project reviewed published literature and recommendations to inform the development of a framework and checklist to provide insights on patient engagement in rare disease HTA.

Several recommendations have been identified for improving HTA in rare diseases. These include leveraging various data resources, integrating patient-centered and –reported outcomes, engaging stakeholders throughout, adopting a societal perspective, considering sustainability, applying economic evaluation methods, and comprehensively incorporating product value (**Figure 2**).

Figure 2. Recommendations for HTA of Rare Disease Treatments

Leverage Various Data Resources

From the remaining 65 articles, a further subset of 42 articles was excluded due to their focus on comparing HTA agencies across different countries and discussing economic evaluation methods such as conducting cost-effectiveness analysis in rare diseases, rather than general tools and frameworks.

In total, 23 articles were reviewed and summarized, focusing on their relevance to HTA tools and frameworks for rare diseases.

### Figure 1. PRISMA Flow Diagram for Literature Search



- In addition to quantitative data, incorporate qualitative data and mixed methods to identify patient-centered outcomes
- Examples of data resources include interviews, case studies, and medical record data

#### Integrate Patient-Centered Outcomes and Patient-Reported Outcomes (PRO)

 Finding the intersection of patient-centered outcomes and patient-reported outcomes is crucial for comprehensive assessment, providing information about patients' preferences, goals, and lived experience

#### **Engage Stakeholders Throughout**

- Involve patients, caregivers, and other stakeholders from pre-clinical to post-launch development stages, including clinical trials, regulation, HTA, and coverage and reimbursement processes
- Examples of stakeholders include patients, caregivers, clinical experts, health care providers, hospitals and health systems, payers, purchasers, industry, researchers, policymakers, regulators, patient advocacy groups, health economists, and training institutions

#### Adopt a Societal Perspective

- Use a societal perspective in rare disease assessments and appraisals
- Consider the economic burden for society and family, and quality of life impacts for the family (i.e., family spillover effects)



## CONCLUSIONS

This literature review highlights key recommendations for bridging gaps and addressing uncertainties in rare disease HTA. Findings will be incorporated into a patient-centered HTA framework and checklist, with the aim of improving patient engagement in rare disease assessment processes.

### REFERENCES

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#### **Consider Sustainability**

- Address sustainability in funding rare disease treatments
- Measures to ensure sustainability may include outcomes-based funding using realworld data collection

#### **Explore Non-Traditional Economic Evaluation Methods**

- Consider methods such as multi-dimensional approaches or Multi-Criteria Decision Analysis (MCDA) to assess multiple aspects of value beyond traditional CEA
- Examples of criteria considered in assessments include disease severity, rarity, manufacturing complexity, and availability of existing treatments

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### **CONTACT INFORMATION**

#### **Comprehensively Incorporate Product Value**

 Assessments of treatments for rare diseases should strive to incorporate novel value elements in a comprehensive manner while balancing increased uncertainties due to factors such as lower disease prevalence Rick Chapman: <u>rick.chapman@valueresearch.org</u>



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