

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.

There is lack of consensus on approaches to conducting patient-centered 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.

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.

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.

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.

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.

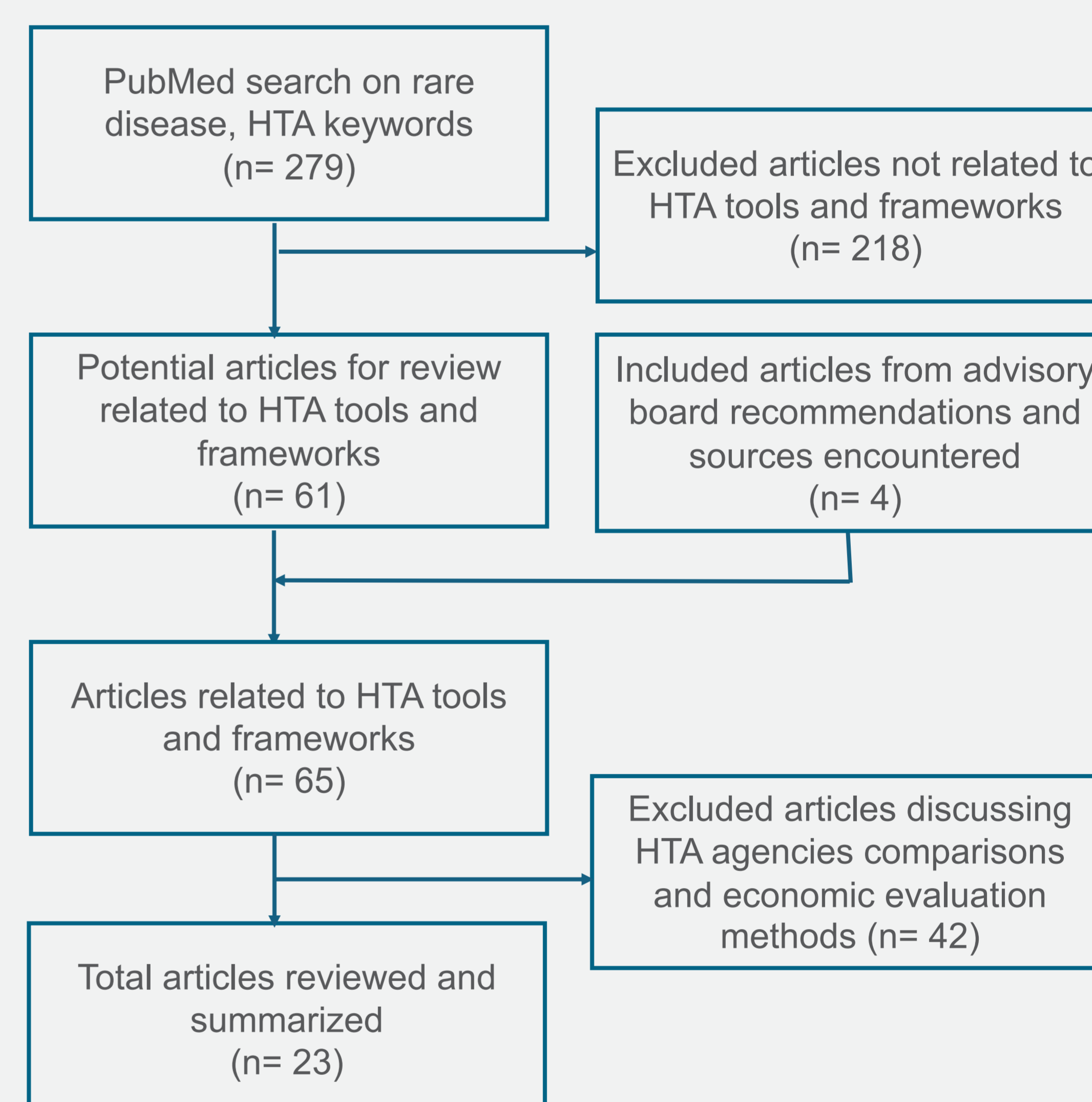
RESULTS

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



Figure 1. PRISMA Flow Diagram for Literature Search



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.

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