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Criteria adopted in different models of public healthcare systems for the evaluation of reimbursement recommendations of orphan drugs: a scoping review.

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Introdução: Access to drugs for rare diseases constitutes a challenge to healthcare systems, especially those with public funding. The difficulty of conducting robust clinical trials limits the quality of evidence and elevates the cost of development, later translated into the drug's prices. Thus, it is necessary for the Health Technology Assessment (HTA) agencies to have differentiated criteria for the evaluation of reimbursement recommendations when dealing with such drugs. **Objetivos:** The objective of this research is to identify and summarize the specific criteria used when evaluating reimbursement recommendations for orphan drugs that are adopted by HTA agencies in countries with different models of public healthcare systems. **Material e Método:** A comprehensive literature search was performed on the databases PubMed, LILACS, Scopus and Embase up to March 2022. We included any publication type (opinion articles, commentaries, editorials, original articles and reviews) that addressed the criteria used by HTA agencies in countries with public healthcare systems when evaluating reimbursement recommendations for orphan drugs. **Resultados:** This scoping review summarizes the identified criteria for 18 countries and ranks them within three models of healthcare systems (NHS, NHI and SHI). We identified that NHS countries, such as the UK, Sweden, and Italy, lean towards innovation, the collection of real-world data, and the impact on organizational aspects of the system. Meanwhile, SHI countries, such as Germany, France and the Netherlands, often employ budget ceilings and expedited evaluation processes. All models shared concern over unmet need and disease nature. The 16 included studies range from 2015 to 2022 and the majority consists of reviews of HTA reports and original articles. **Discussão e Conclusões:** This review provides a good basis for the understanding of each model's classification and general tendencies when creating differentiated criteria to accommodate and compensate for the lack of evidence and investment around rare diseases.

Palavras-Chave: HTA; ATS; Rare Diseases; Criteria To HTA.

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