

Measurement of therapeutic adherence in orphan diseases: A Scoping review



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INTRODUCTION

Orphan diseases (OD) are chronic and degenerative pathologies with a low global prevalence that affect the quality of life. Due to their late and difficult diagnosis there is a need to initiate timely and continuous treatment according to the prescription. The compliance and adherence to the treatment is critical to achieve health outcomes.

OBJECTIVE

To evaluate the state of art related to adherence scales in OD for intravenous treatment (IVT) administered at home or at specialized centers.

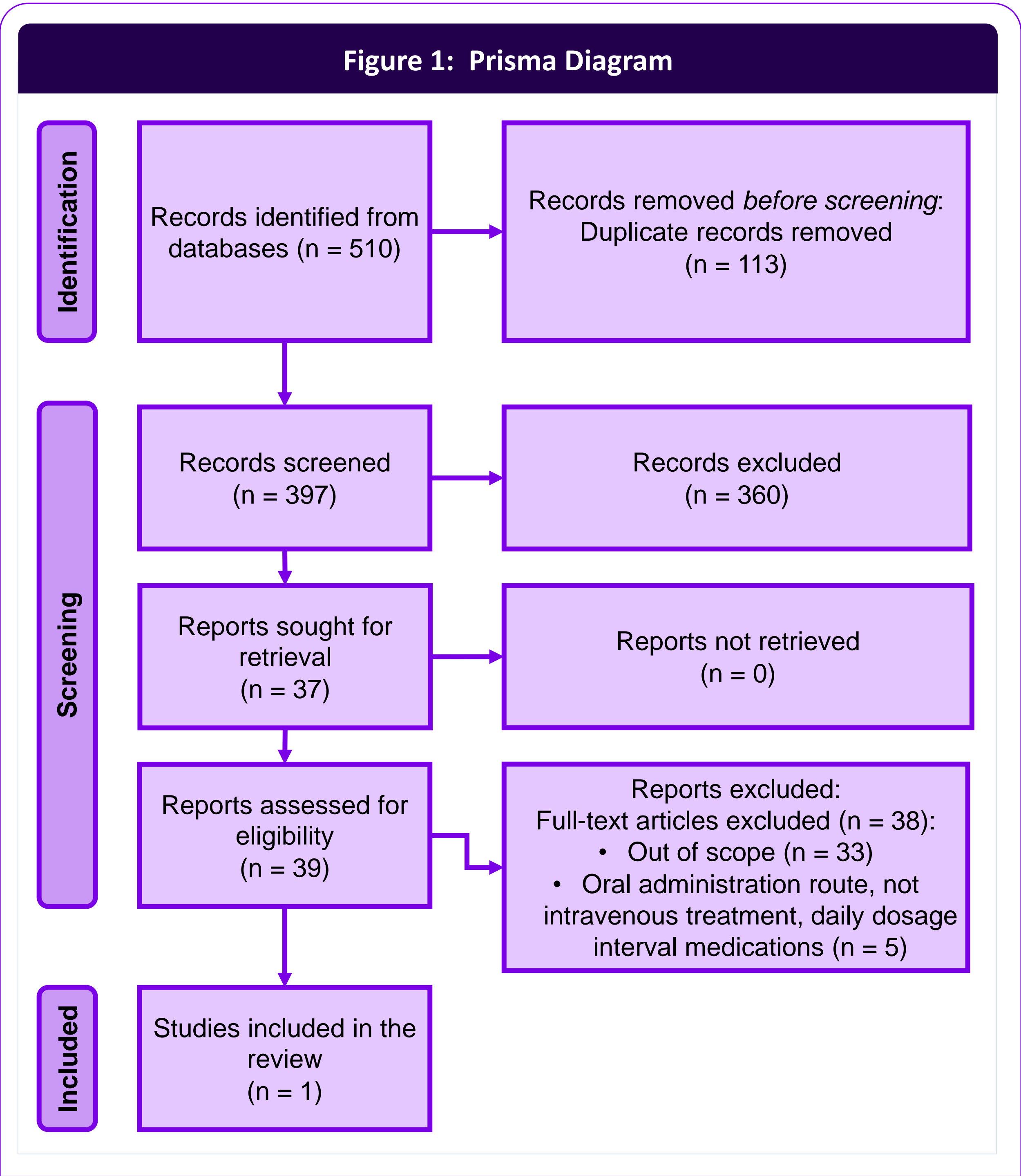
POSTER HIGHLIGHT: Having adequate instruments to measure objectively the adherence in orphan diseases, can improve the treatment effectiveness, identify access barriers, impact patient quality of life, and reduce both disease complications and associated healthcare costs.

METHODS

- A scoping literature review of published research articles of adherence scales in OD was carried out in MEDLINE, Cochrane, LILACS, PubMed, Ovid, Embase, SciELO and Redalyc.
- The search strategy included the keywords: adherence, compliance to treatment, rare diseases, home infusion therapy, surveys, and questionnaires.

RESULTS

- 510 articles were found, only 1 accomplished the inclusion criteria (Figure 1). The VERITAS PRO¹ scale for hemophilia meets the characteristics to evaluate OD adherence. However, the publication did not describe the questionnaire components.
- Additional instruments with potential to be adapted and used for IVT in OD were identified according to the disease used to test them and the inclusion of therapeutic adherence in their components. These used indirect measurement methods and/or a patient-reported outcomes approach.
- The MMAS-8², BMQ³, ARMS⁴, and MAS⁵ instruments, amongst others, were described in different studies for high-prevalence of chronic diseases, outpatient treatment, daily dosage interval medications and/or an oral administration route.



CONCLUSIONS

- There are several methods available to measure adherence. However, not all methods can be applied as the standard measurement for each disease and treatment scheme. In this literature review, an optimal method for OD was not found.
- There are opportunities to develop alternatives that evaluate objectively the IVT adherence in OD. Such instrument could allow for improved treatment effectiveness and to identify access barriers.
- It would also have a positive impact on the patient's quality of life, reduced disease complications and healthcare costs.

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