Support of RWE in the Price and Reimbursement Decision-Making Process for Rare Diseases in Spain

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BACKGROUND AND OBJECTIVES

Background: Real-world data (RWD) is a promising source of complementary evidence to accelerate drug access in rare diseases (RDs). However, real-world evidence (RWE) applicability in RDs' price and reimbursement (P&R) decision-making is still limited.

Objective: Define how RWE can support the P&R decision-making process to accelerate drug access for RDs in Spain.

METHODOLOGY

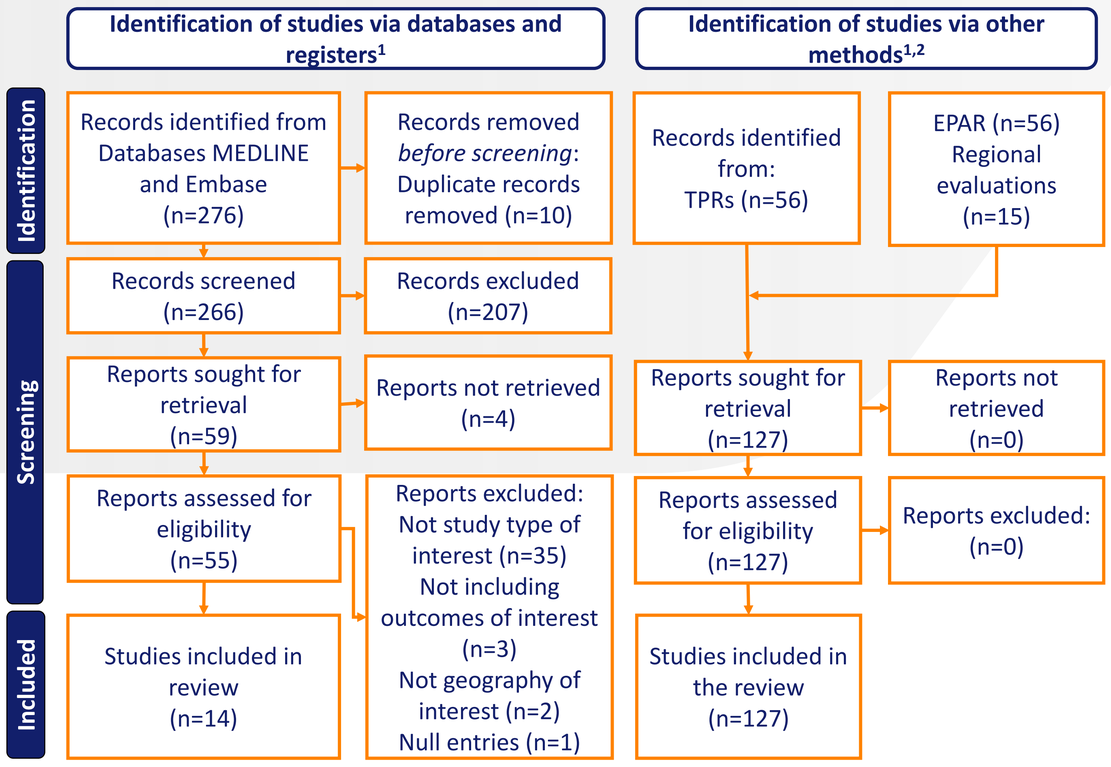
A targeted literature review (TLR) of articles and reports published between 2018-2023 to examine RWE utilization in the P&R process for RDs drugs was conducted, followed by a focus group primary market research with Spanish payers to validate literature findings and address the identified gaps.

The TLR included articles covering uncertainties, perception, and use of RWE in the P&R process. TPRs (therapeutic positioning reports) on RDs and their corresponding EPARs (European Medicine Assessment Reports) and regional evaluations were searched and included.

RESULTS

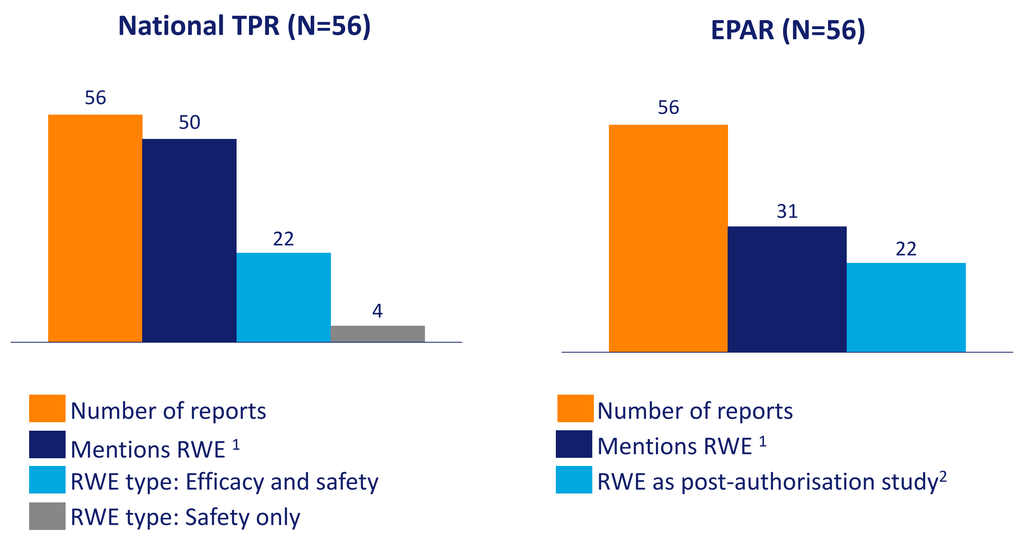
14[1-14] studies and 127 reports (56 TPRs[15-70], 56 EPAR[71-126], and 15[127-141] regional evaluations) were included.

PRISMA diagram



Date of search: May 10th, 2023
EPAR: European public assessment report; TPR: therapeutic positioning report
[1]Timeframe: 2018 – 2023; [2]TPRs on RDs published between 2018-2023 and corresponding EPARs and regional evaluations.

RWE studies in TPRs and corresponding EPARs



EPAR: European public assessment report; TPR: therapeutic positioning report; RWE: real world evidence
[1]Studies on the disease, epidemiology, efficacy, and/or safety (short-term or long-term) of the investigational drug or therapeutic alternatives or competitors; [2]Upon request by the EMA or at the promoter's proposal.

- Currently, RWE has a minor impact on the P&R decision-making for RDs in Spain, limited to a supporting tool for outcome-based agreements
- RWE can play a key role in establishing innovative dynamic and value-based pricing strategies (currently under evaluation in Spain) and in re-evaluation processes and price reviews
- Key points to consider RWE in P&R decisions include an early dialogue with payers to co-create the most suitable study design that addresses payers' uncertainties and to include RWE studies within the evidence generation plan
- Main challenges to overcome include a commitment from the public administration to set a framework for the use and applicability of RWE, commitment from pharmaceutical companies to publish generated evidence; transparency of the decision-making process; application of technologies to extract the RWD from primary sources; and data governance

Key insight	Rationale
RWE has a minor impact on the P&R decision-making for RDs	> RWE has not impacted price or decision-making of RDs in Spain; when TPRs refer to RWE studies, is mainly contextual > There is a lack of definition of the value of RWE in the health technology assessment and P&R decision-making
RWE can play a key role in establishing innovative dynamic and value-based pricing strategies	> VALTERMED and early access programs have the potential to generate RWD that can be exploited for evidence generation > RWE complements the evidence needed in P&R decisions, can act as a supporting tool for outcome-based agreements, and for the establishment of dynamic pricing strategies > New generated evidence should translate into changes in drug pricing and positioning > Dynamic pricing strategies must be agreed from the beginning of the P&R negotiation > RWE could be key in drug re-evaluation if the evidence supports the value of the drug, and to ensure real-life drug effectiveness; although currently re-evaluation processes often results in a price decrease
Key points to consider RWE in P&R decisions	> Early dialogue with payers to ensure that RWE studies' objective cover uncertainties that arise at the time of the negotiation, not covered by the evidence generated in clinical trials > Studies must have a robust study design that will be accepted by the authorities (following guidelines, registered protocols, etc) > Early Dialogue Advice with regulatory and Health Technologies Assessment agencies must be encouraged > RWE generation should be considered early in the drug development plan (to be available at the P&R process)
Main challenges of including RWE in P&R decisions	> Commitment and transparency from all parties > Apply new technologies (such as AI) to extract data > Data quality (preference for primary sources) and governance > Replication of clinical trial conditions and concerns about RWE studies methodology

CONCLUSION

Beyond its current limited use, RWE can serve as a tool facilitating dynamic and value-based pricing agreements to accelerate the P&R process and thereby the access to new RDs drugs to patients.

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