

EVALUATING REAL LIFE CRITERIA THAT COULD DRIVE PRICING & REIMBURSEMENT APPROVAL OF ORPHAN DRUGS IN SPAIN

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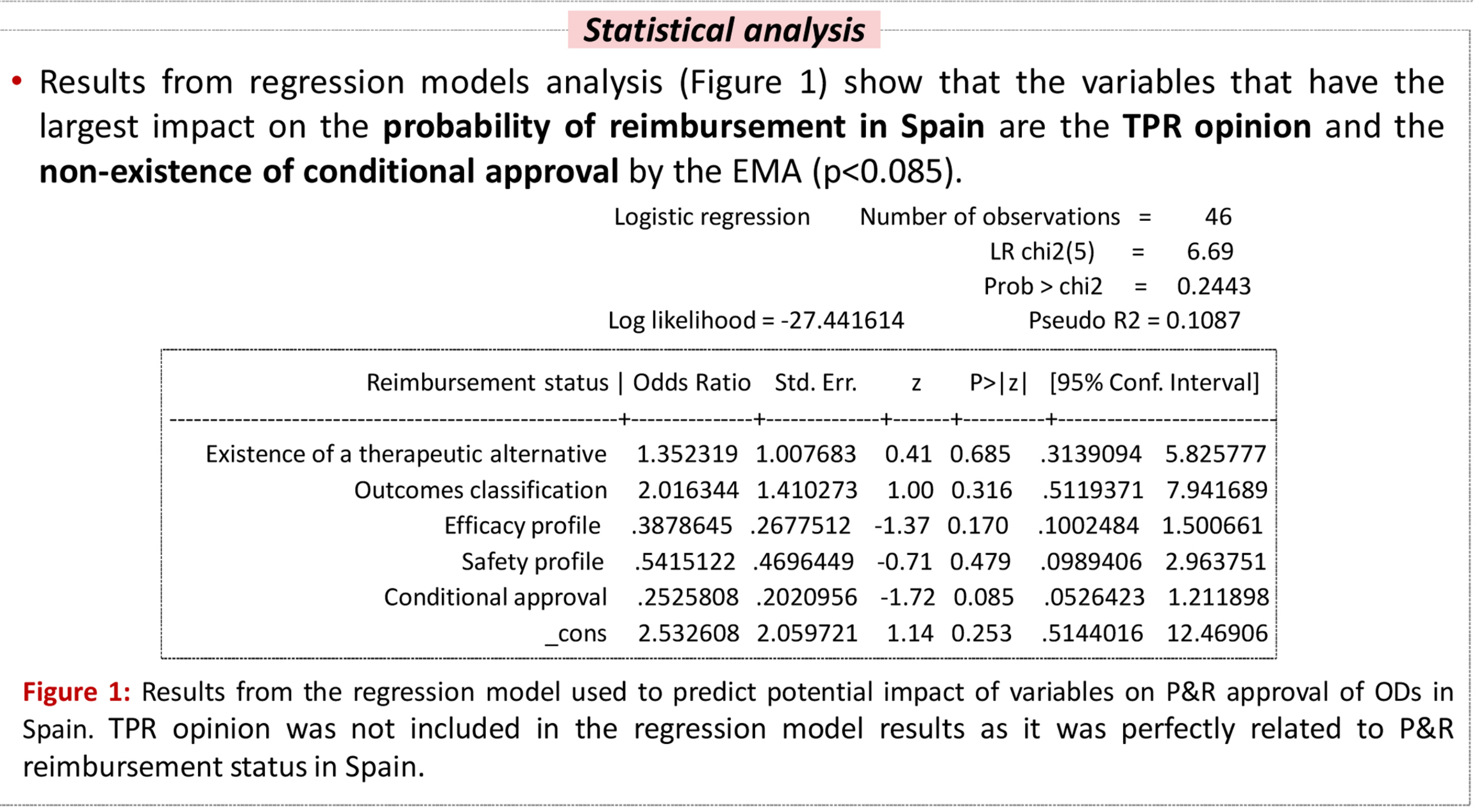
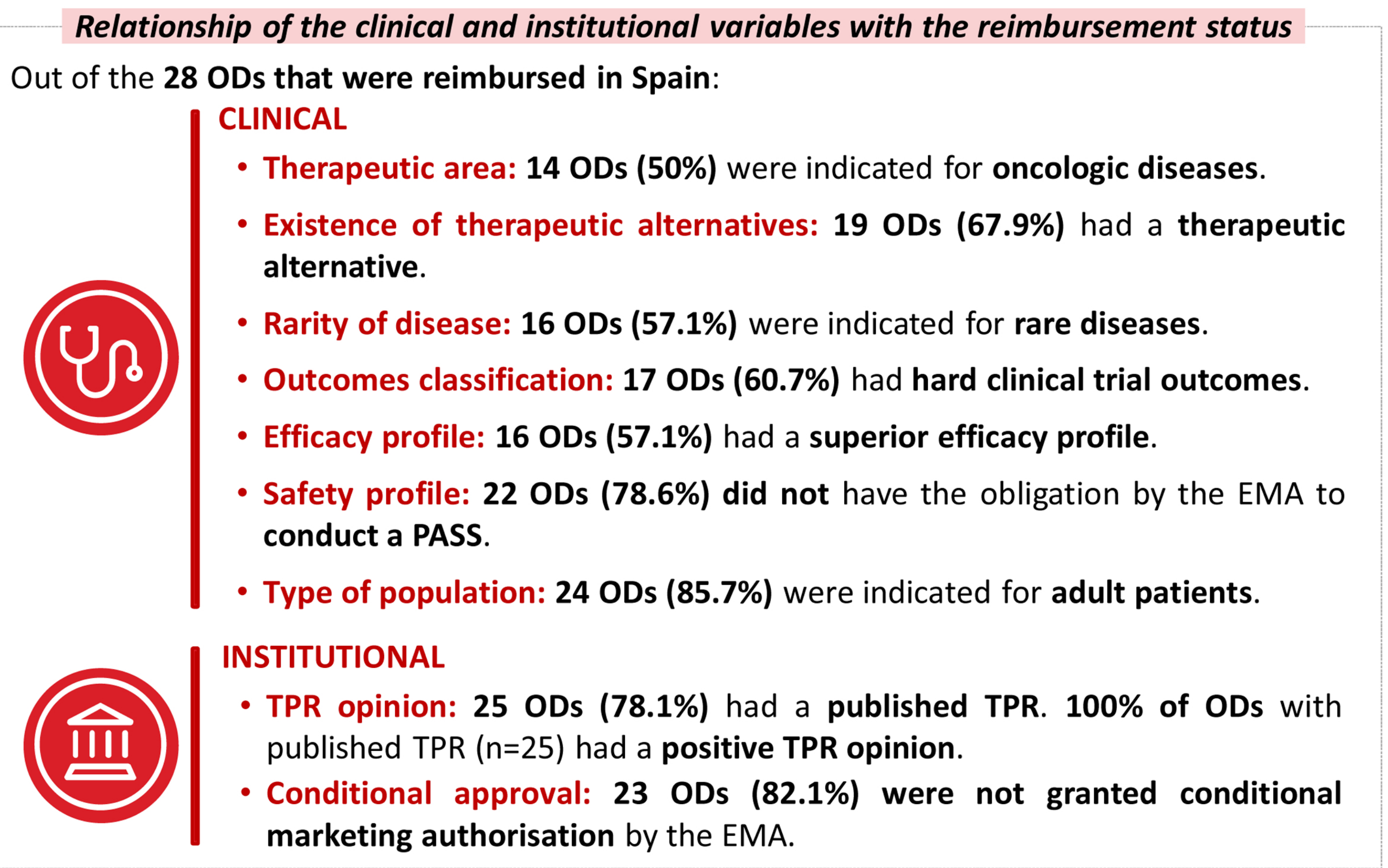
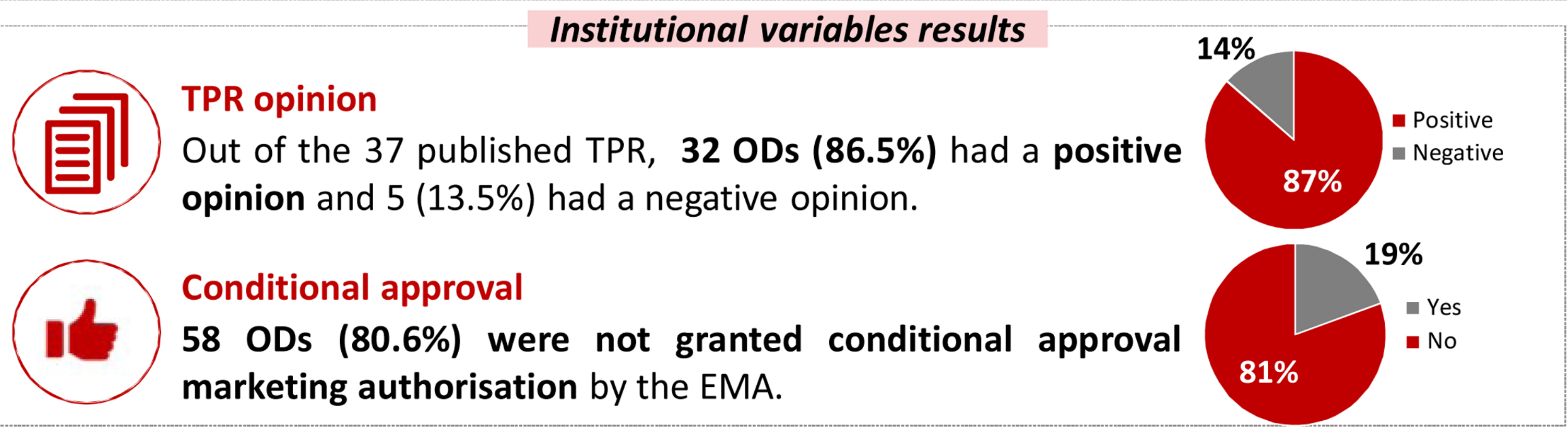
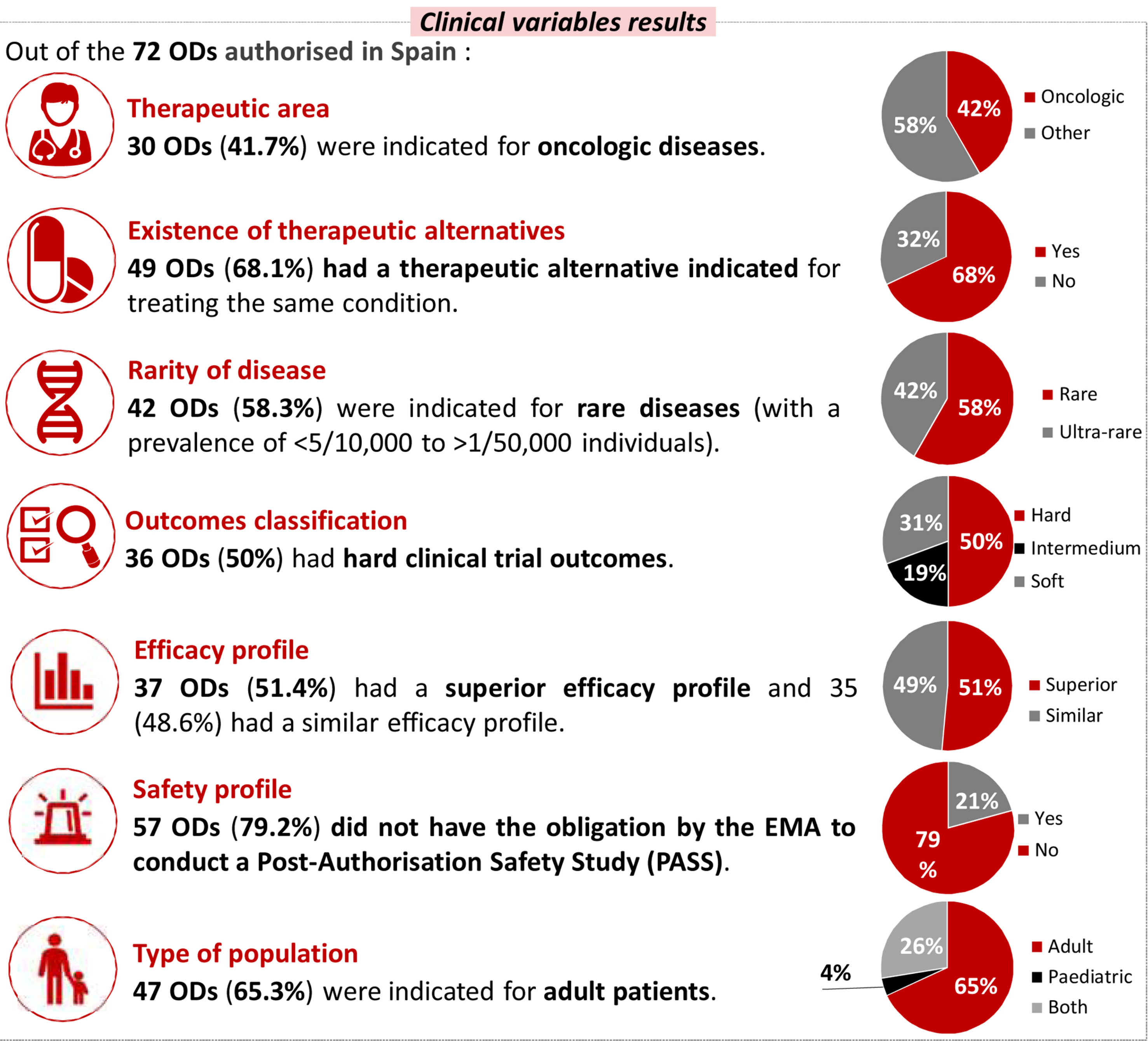
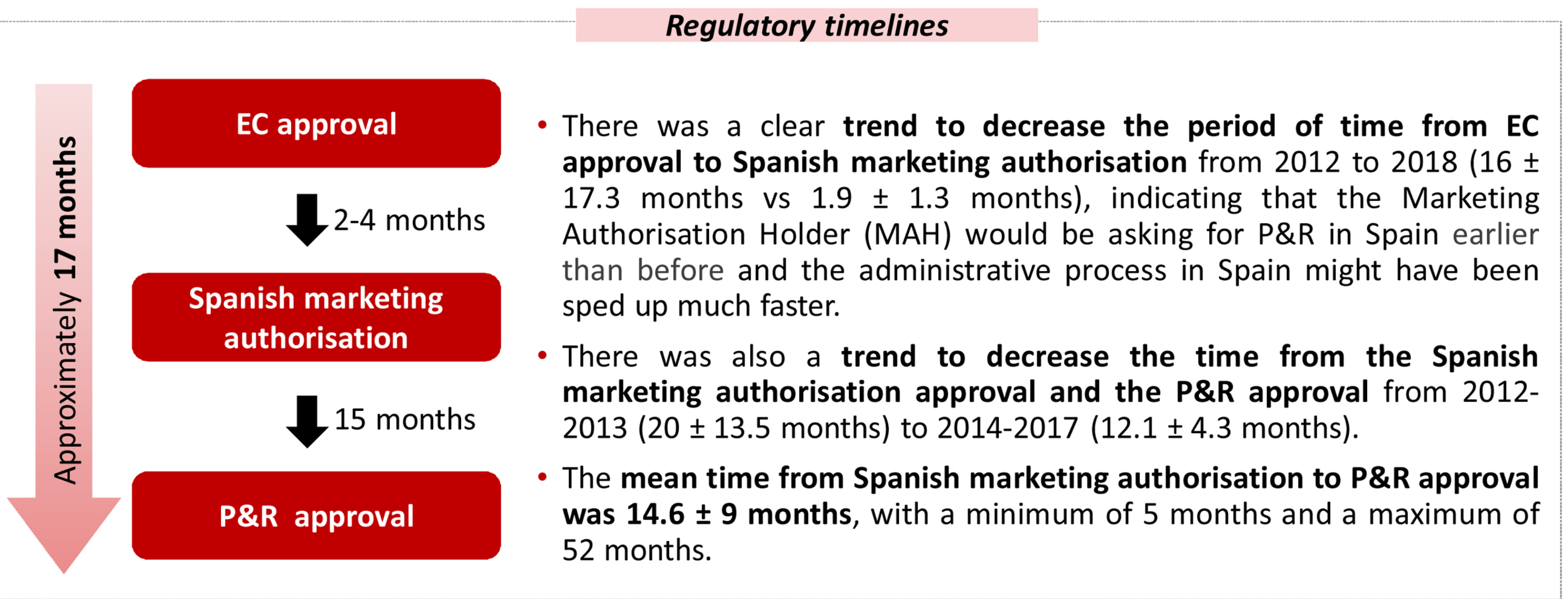
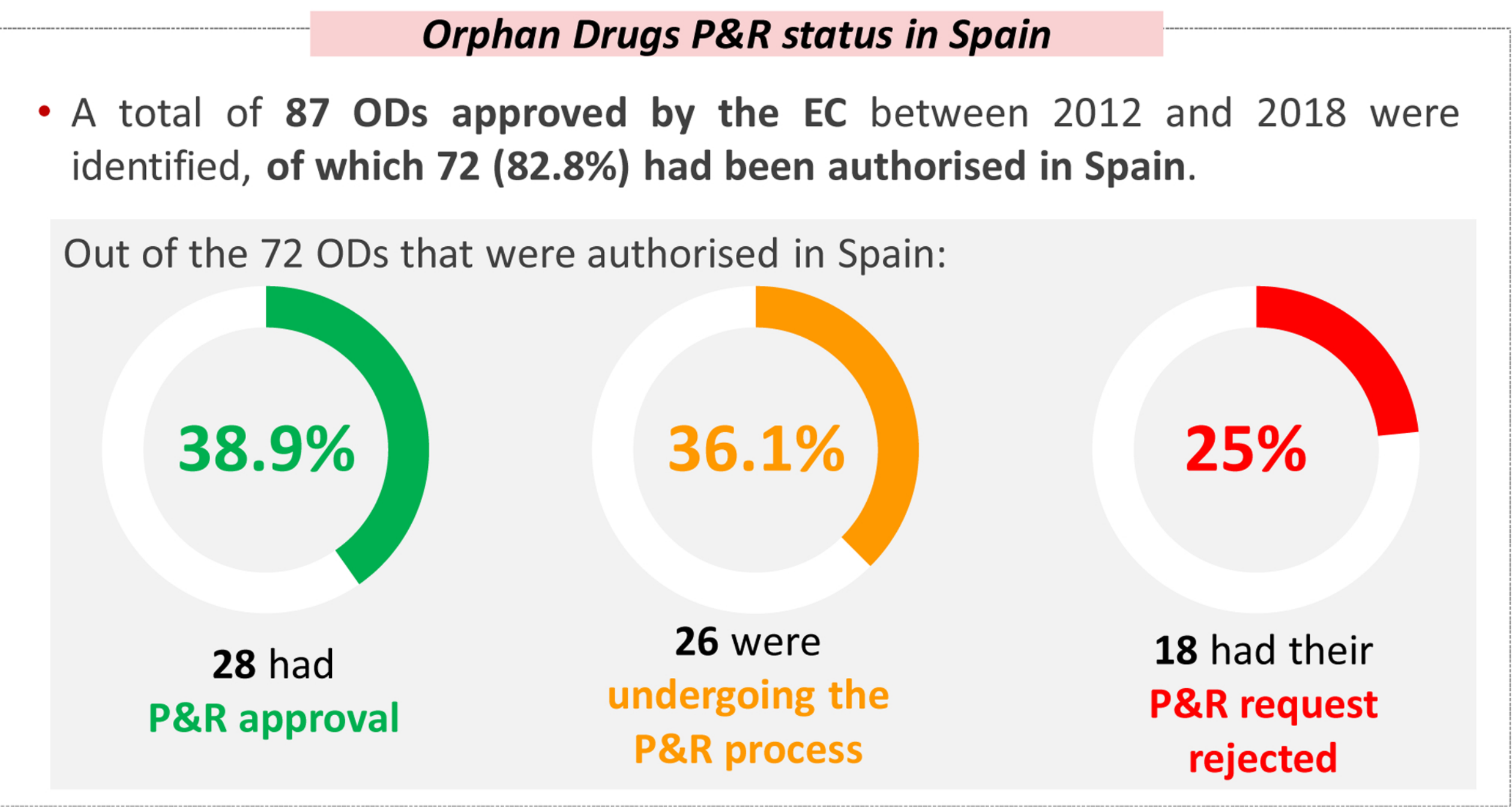
BACKGROUND & OBJECTIVES

- P&R decision-making for orphan drugs (ODs) is faced with additional challenges, as the limited knowledge and heterogeneity of rare diseases make it difficult to demonstrate the added clinical benefit of a treatment¹. Having approval by the European Commission (EC) for an OD does not guarantee access within the Spanish market, which may prevent patients affected by rare diseases equitable access to these treatments². Because of ODs' high prices and limited clinical evidence, P&R evaluators should not only consider cost-effectiveness, but also consider other factors that could influence final decision-making^{3,4,5}. Although P&R evaluation criteria are well defined by law in Spain⁶, factors that might influence on final P&R decisions for ODs in Spain are not clear.
- The objective of this study was to **identify potential criteria that could influence P&R approval for ODs in Spain**.

METHODS

- This study includes ODs approved by the European Commission between 2012 and 2018 and obtained Marketing Authorisation in Spain. ODs were classified according to their P&R status in Spain: approved, undergoing decision and rejected. P&R approval timelines in Spain were analysed.
- Hypotheses on likelihood of ODs reimbursement approval were formulated based on criteria used in the Spanish P&R process: existence of alternative therapies, the availability of hard clinical trial outcomes, a superior comparative efficacy profile, a positive safety profile that raised less uncertainty among evaluators, a positive Therapeutic Positioning Report (TPR) opinion (Authors' subjective interpretation of the conclusions of published TPRs) and the status conditional approval granted by the European Agency of Medicines (EMA).
- Potential variables that might influence reimbursement were classified according to the system described by Zozaya *et. al.* based on criteria for reimbursing ODs⁶: clinical and institutional variables. Economic variables have not been assessed due to lack of publicly-available information.
- The validity of the hypotheses was tested through descriptive statistic analysis and a logistic regression model to predict the potential impact of studied variables on P&R decisions in Spain.

RESULTS



CONCLUSIONS

- From all ODs approved by the EC between 2012 – 2018 and which had obtained Marketing Authorisation in Spain, **28 (38.9%)** were **reimbursed**, **26 (36.1%)** were **undergoing decision** and **18 (25%)** were **rejected**.
- The **mean time from Spanish Marketing Authorisation to P&R approval in Spain was 14.6 ± 9 months**. P&R approval of ODs has sped up an average of 8 months since 2012 in Spain. Therefore, the inclusion of TPRs during P&R negotiations since May 2013 could have accelerated the P&R process in Spain.
- P&R approval for ODs in Spain **might be positively affected** by a **positive TPR opinion** and the **absence of conditional approval status** by the EMA.
- Budget Impact could be an important variable for P&R approval, but economic variables were not assessed in this study due to a lack of publicly available information, as Spain operates a dual pricing system for hospital medicines. Official listed prices in the available databases do not reflect the reimbursement price agreed between the Ministry of health and the MAH. The real price can be reduced between 20-45% and information about sales forecast is not available.

REFERENCES

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