

Annual ObservEMHe Report: Orphan Drugs approved by the European Commission and authorized in Spain

Scope of the study

All Orphan Drugs approved by the European Commission until 31 December 2021

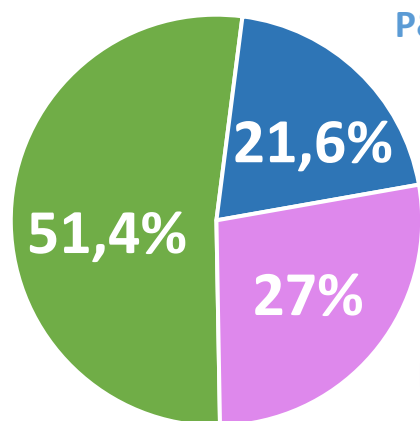


- To describe Pricing & Reimbursement (P&R) situation in Spain of Orphan Drugs (ODs) approved by European Commission
- To analyse administrative timelines and impact of Therapeutic Positioning Report on the P&R process of ODs in Spain
- To describe variables that would be relevant for the P&R process of ODs in Spain

57 ODs had P&R approval

24 ODs were undergoing the P&R process

30 ODs had P&R request rejected

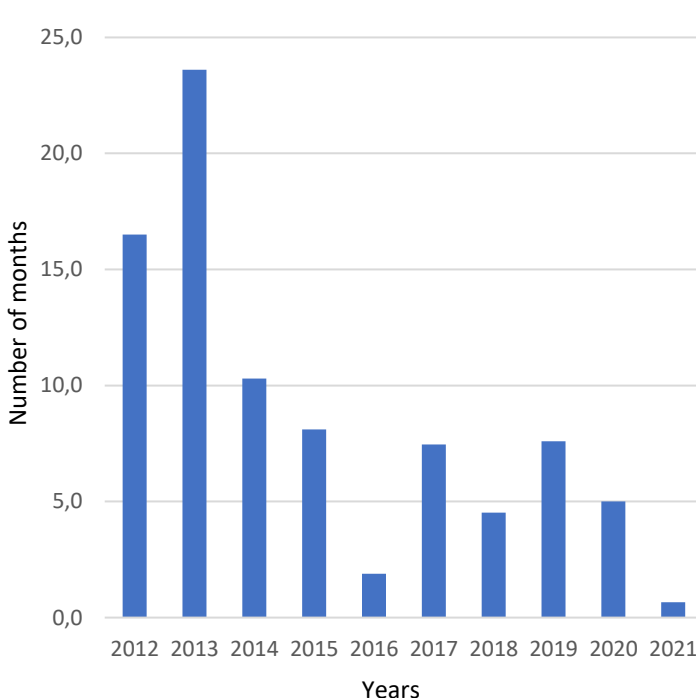


Out of the 128 ODs approved by the European Commission, 111 (86,7%) ODs had been granted marketing authorisation in Spain

P&R evaluation timelines (from the Spanish Marketing Authorisation to P&R decision) before and after the inclusion of the Therapeutic Positioning Report in 2013



The mean time from European Commission approval to Spanish Marketing Authorisation for ODs has shortened over the years



P&R evaluation timelines have been slightly reduced with the inclusion of the Therapeutic Positioning Report by an average of less than 1 month

Clinical and regulatory variables that could be relevant for the P&R process in Spain were assessed

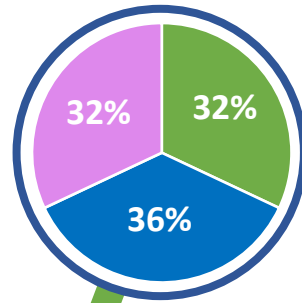
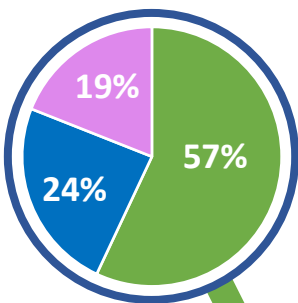
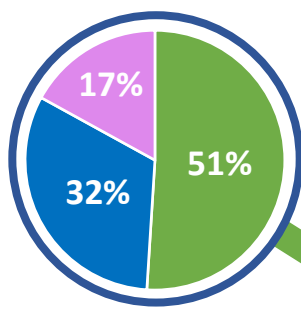
OUTCOMES CLASSIFICATION

ODs with survival-related outcomes

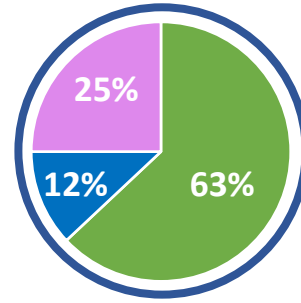
CONDITIONAL APPROVAL

ODs with conditional approval

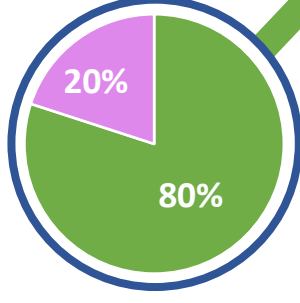
THERAPEUTIC AREA
ODs with an oncology indication



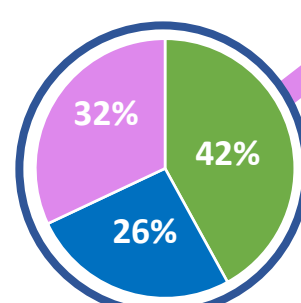
RARITY OF DISEASE
ODs indicated for an ultra-rarity of disease



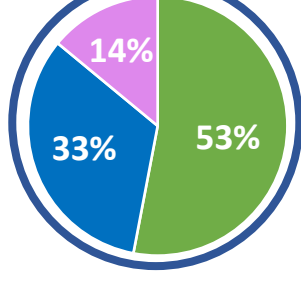
TPR CONCLUSION
Positive TPR conclusion



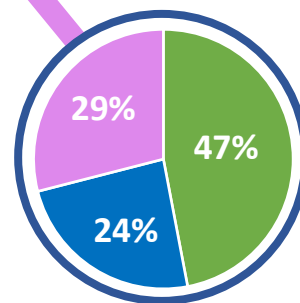
THERAPEUTIC ALTERNATIVE
Absence of therapeutic alternative



SAFETY PROFILE
Obligation to conduct a PASS



TYPE OF POPULATION
ODs indicated for paediatric patients



Variables that would influence P&R decision

Variables that would not influence P&R decision

Reimbursed
Under P&R decision
Reimbursement rejected