Early Access Programs and Pricing and Reimbursement agreements of Orphan Medicines in Portugal

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OBJECTIVES:

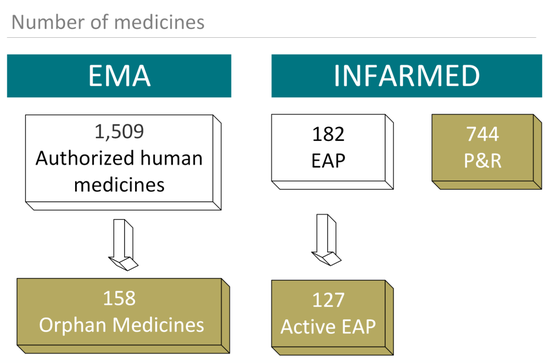
A Marketing Authorization (MA) granted to a medicinal product does not guarantee its availability to patients. In Portugal access to medicines occurs via early access programs (EAP) and pricing and reimbursement (P&R) agreements. This study aimed to characterize access to Orphan Medicines (OM) by the mechanisms of EAP or P&R in Portugal. We compare availability of OM to Rare Disease Action Plan goal for 2025.

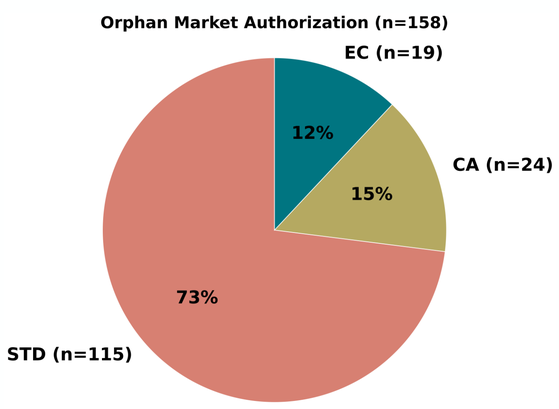
METHODS:

Orphan Medicines MA was extracted from the European Medicines Agency (EMA) database [1] on the 17th October of 2025. Additional information on country level registration, commercialization (marketed) and availability was gathered from the Portuguese Medicines database (INFOMED/INFARMED) [2] and INFARMED P&R [3] and EAP [4] public decisions. Data sources were merged by the name of medicine regardless the number of approved therapeutic indications. Counts and proportions were used to describe the characteristics of interest.

RESULTS:

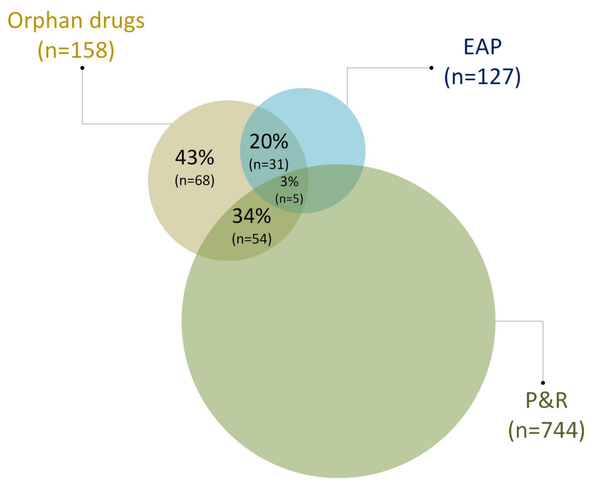
At the data cut-off there were 1,509 medicines authorized by EMA from which 10% (n=158) were OM. In Portugal there were 744 medicines with P&R and 182 EAP with 69.8% (n=127) being accessible (active).





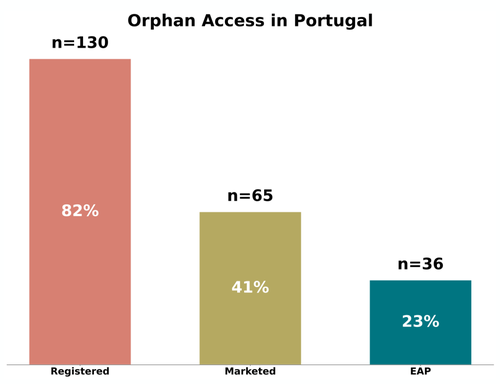
The type of MA granted to OM were Standard (STD) authorizations in 73% of the cases, 12% were granted under Exceptional circumstances (EC) and 15% of the MA were granted under Conditional approval (CA).

From all the MA granted to OM, 66% were associated with the need of additional monitoring, 17% were considered priority medicine (PRIME), 9.5% were advanced therapy medicinal products and 8.9% were associated with accelerated assessment at EMA level.



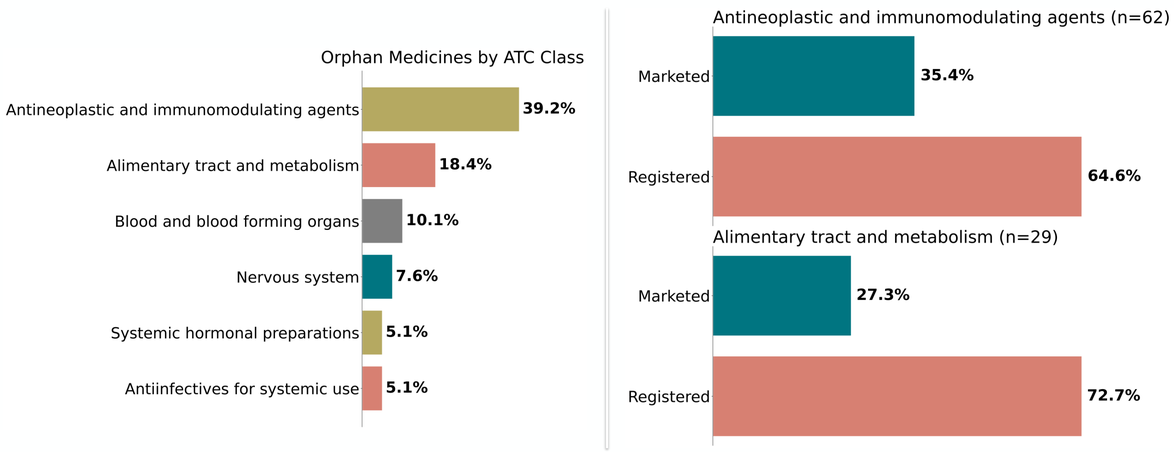
Among the 158 orphan-designated medicines, 37% (n=59) are included in P&R processes, while 23% (n=36) are available through EAP. A small fraction (3.2%, n=5) overlaps across all three categories, indicating that only a limited number of orphan medicines are simultaneously reimbursed, and accessible via EAP due to different therapeutical indications.

From the OM authorized by EMA, 130 (82%) are registered in Portugal and 65 (41%) are marketed. Non-marketed with EAP are imported medicines.
The majority (69%) of the OM having P&R in Portugal were associated with the need of additional monitoring. This was also verified for most (59%) of the OM with currently active EAP.

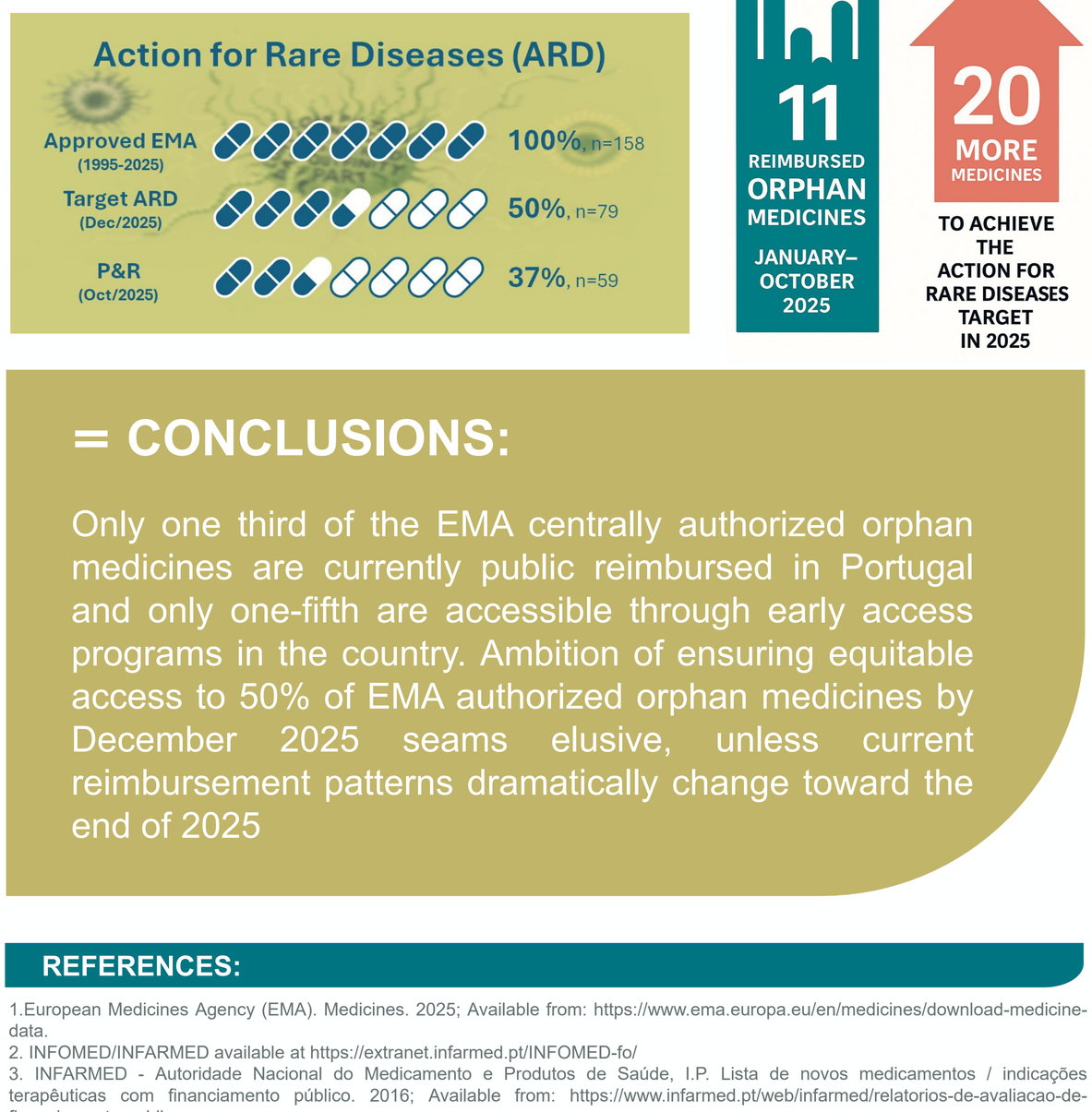


RESULTS (CONTINUATION):

Most orphan medicines belong to two main therapeutic area: Antineoplastic and immunomodulating agents (39.2%) and Alimentary tract and metabolism (18.4%). Together, these categories account for more than half of all orphan medicines. Additionally, 14.6% correspond to therapeutic areas with individual representation below 5% (not in the plot).
In Portugal, most orphan medicines within the Antineoplastic and immunomodulating agents (64.6%) and Alimentary tract and metabolism (72.7%) classes are registered; however, only a smaller proportion are effectively marketed (35.4% and 27.3%, respectively). These findings highlight a persistent gap between regulatory registration and real market access, indicating that patients may still face challenges in obtaining these therapies despite formal approval.



In 2025 Rare Disease Action Plan has been implemented to ensure equitable access to medicines, with the objective of having 50% of EMA-authorized orphan medicines available in the Portuguese market by December 2025 [5]. As of October 17, 2025, 59 orphan medicines had been reimbursed in Portugal. Therefore, to achieve the established target, an additional 20 orphan medicines must be reimbursed by December 2025.



= CONCLUSIONS:

Only one third of the EMA centrally authorized orphan medicines are currently public reimbursed in Portugal and only one-fifth are accessible through early access programs in the country. Ambition of ensuring equitable access to 50% of EMA authorized orphan medicines by December 2025 seams elusive, unless current reimbursement patterns dramatically change toward the end of 2025

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