Access to orphan medicines in Portugal

HPR234

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OBJECTIVES

Centralised procedure (CP) is compulsory for orphan medicines and, after the marketing authorisation (MA) by the European Commission, the medicine can be marketed throughout the EU. However, accessibility is dependent on pharmaceutical and economic policy in each EU Member State, which is determinant not only for patients access but also to pharmaceutical companies' market strategy. The aim of this study is to evaluate the access to orphan drugs in Portugal.

METHODS

Orphan drug records were extracted from EMA database[1]. Variables collected included medicine name, MA status and date, ATC code, conditional (CA) and exceptional circumstances (EC) approvals. Only those with a valid MA were included. Therapeutic areas were categorized according to the first level of ATC code. National access data regarding registration number, marketing status and early access programs (EAP) were retrieved from INFARMED - National Authority of Medicines and Health Products database

RESULTS

Up to June 2024, 175 orphan drugs applications have been assessed under CP: 18 have been refused and one have been withdrawn, leaving a total of 156 orphan drugs with valid MA (Figure 1).
Of all the orphan drugs included, the most frequent type of approval was the standard (STD) (n = 110, 70.5%), followed by conditional approval (n=26, 16.7%) and approval under exceptional circumstances (n=20, 12.8%) (Figure 2).

Figure 1. Flowchart for database creation and definition of the analysis sample

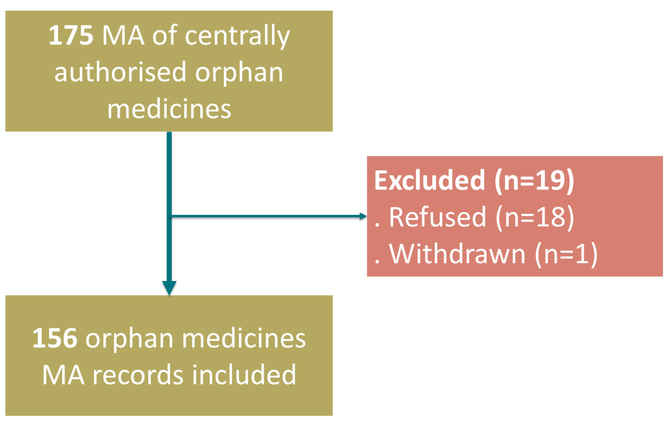
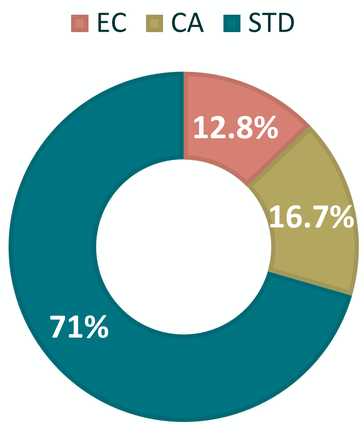
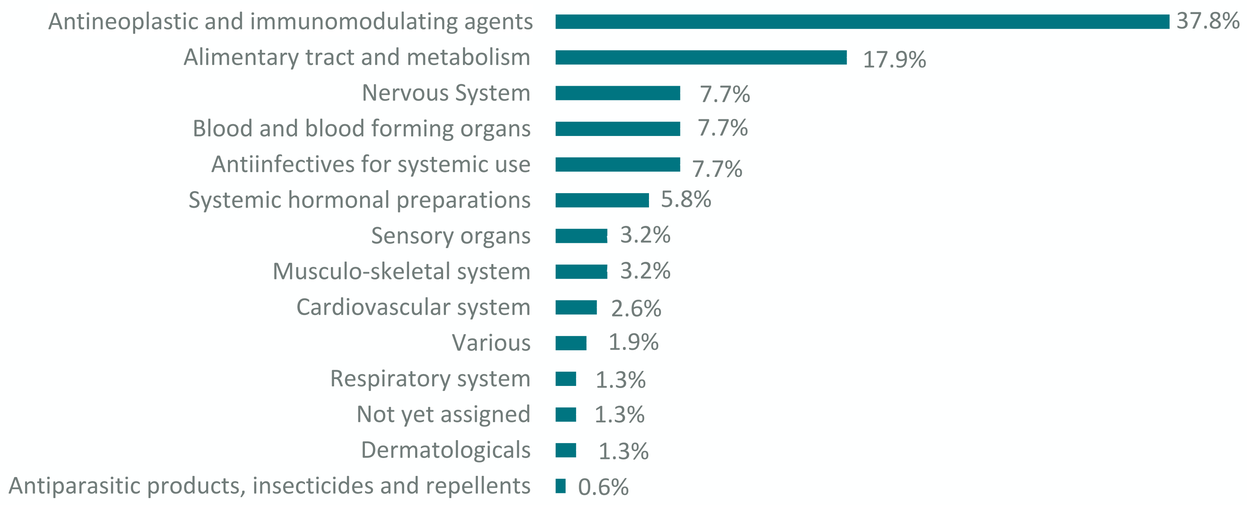


Figure 2. Orphan drug distribution by type of marketing authorization approval



Antineoplastic and immunomodulating agents (AIA) (37.8%) and alimentary and tract metabolism drugs (ATM) (17.9%) represent the most frequent therapeutic areas of orphan drugs with MA (Figure 3).

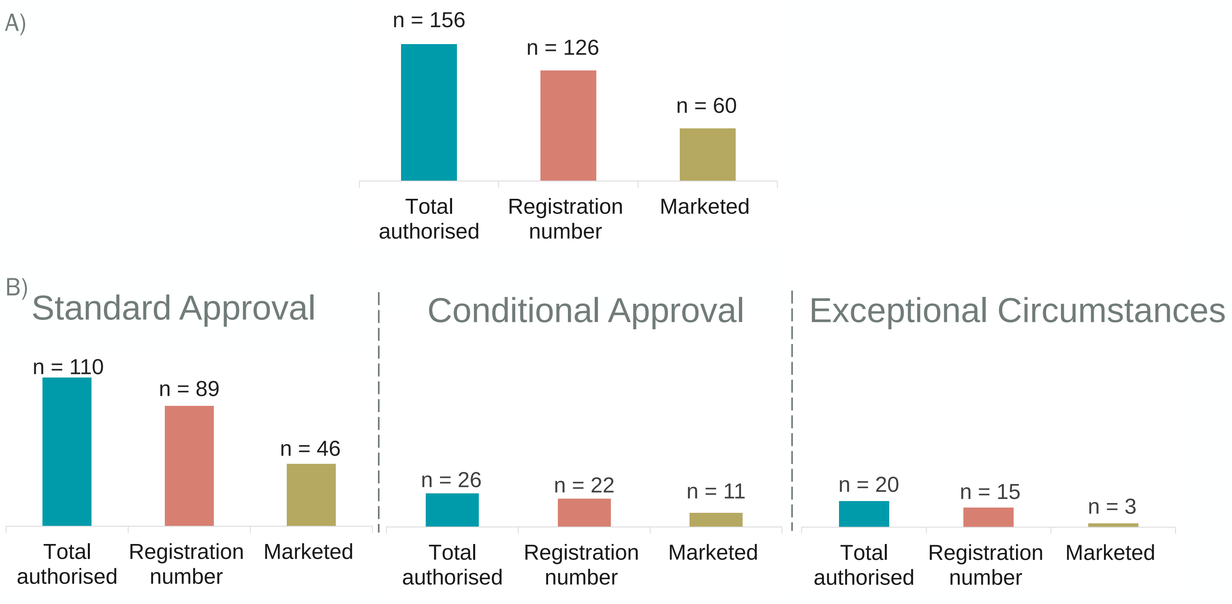
Figure 3. Orphan drugs distribution by therapeutic area



RESULTS

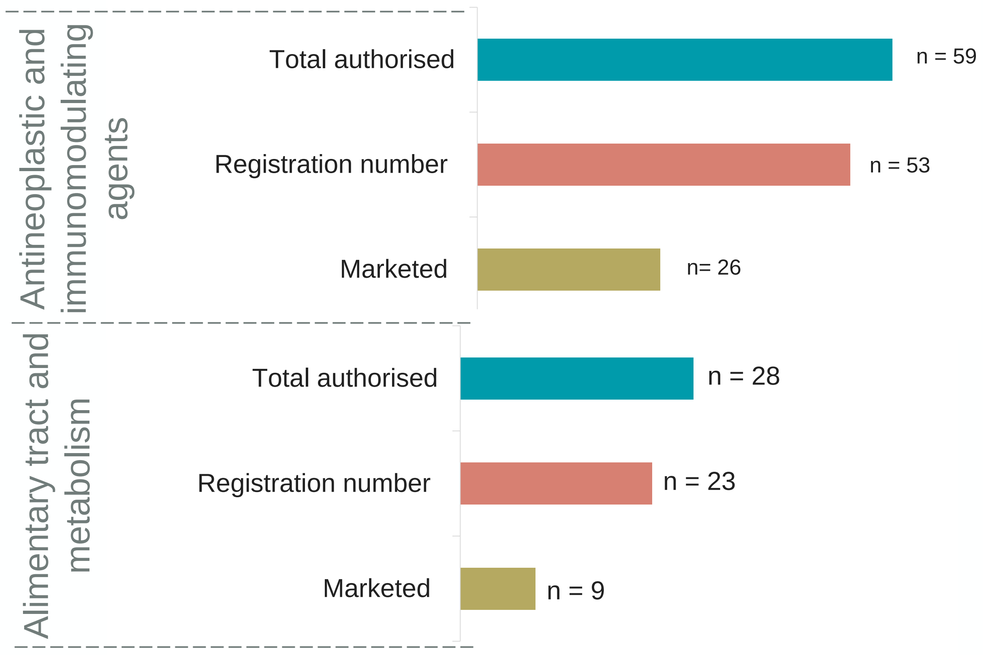
The proportion of authorised orphan drugs registered in Portugal is 80.8% (n=126) and only 38.5% (n=60) are currently being marketed. For orphan drugs approved under CA and EC, only 84.6% (n=22) and 75.0% (n=15) have at least one registration number in Portugal, respectively. Access is being substantially conditioned for both CA and EC, for whom only 42.3% (n=11) and 15.0% (n=3) of the approved applications are being marketed (Figure 4)

Figure 4. Accessibility and access for: A) total orphan drug records and B) by type of MA approval



Considering the therapeutic areas with most orphan drugs approved, accessibility to antineoplastic and immunomodulating agents or to medicines for alimentary tract metabolism is being impaired, as more than 50% of orphan drugs approved by EMA are not marketed in Portugal (Figure 5)

Figure 5. Access to orphan drugs for the most frequent therapeutic area



Of the 156 orphan drugs with valid MA, only 38 (24.4%) are currently being accessed through a national early access program.

= CONCLUSION

A gap has been observed between European authorisations and national accessibility to orphan drugs, revealing barriers on the access to innovation.

[1] EMA – European Medicines Agency, Medicines Data Table, https://www.ema.europa.eu/en/medicines/download-medicine-data, Accessed at 2024, May 31st