CONDITIONS AND RESTRICTIONS ON EARLY ACCESS TO INNOVATIVE MEDICINES IN PORTUGAL

HTA234

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OBJECTIVES

Patients living with critical diseases may require access to medicines that are unavailable to them for various reasons. In 2014, the National Health Technology Assessment System (SiNATS) facilitated access to innovative medicines through early access programs (EAPs). Those are meant for accelerated pathways making new medicines available to patients before formal Health Technology Assessment (HTA) in Portugal[1]. The aim of this study was to identify the type of restrictions on the target population of innovative medicines under EAPs, and quantify those restrictions according to the therapeutic indication resulting from pricing and reimbursement (P&R) process after formal HTA.

METHODS

All the EAPs approved and published on the INFARMED - National Authority of Medicines and Health Products website[2] from January 2020 until December 2023 with a P&R decision[3] regarding the HTA process, were reviewed.

Table 1. EAP main characteristics

EAP Characteristics	N=54
Therapeutic area (ICD10[4]), n (%)	
Neoplasms (II)	31 (57)
Endocrine, nutritional and metabolic diseases (IV)	7 (13)
Diseases of the skin and subcutaneous tissue (XII)	4 (7.4)
Diseases of the blood and blood-forming organs (III)	3 (5.6)
Other*	
Orphan drug, n (%)	16 (30)
Maximum patients that can be included, mean (range)	12 (1 – 50)

* Includes *Diseases of the nervous system* (n=2), *Diseases of the eye and adnexa* (n=2), *Diseases of the respiratory system* (n=2), *Diseases of the circulatory system* (n=1), *Diseases of the digestive system* (n=1) and *Certain infectious and parasitic diseases* (n=1). EAP: Early access program.

The marketing authorisation (MA) dates, and public data from P&R decisions were merged with data from EAP.

Table 2. P&R decision main characteristics

P&R decision characteristics	N=54
P&R positive decision, n (%)	48 (89)
Added therapeutic value, n (%)	39 (81)
Equivalence, n (%)	9 (19)
P&R negative decision, n (%)	6 (11)

Restrictions to drug utilisation under EAP relative to therapeutic indication resulting from MA to EAP and from EAP to P&R decision, were classified according to patient population demographics, disease characteristics, treatment history, efficacy and safety variables.

RESULTS

A set of 7 restriction criteria were agreed upon: age, previous treatments, lack of efficacy or safety, resistance/refractory disease, contraindication/ intolerance/ ineligibility, disease-associated scores (e.g., ECOG), and disease characteristics/ genetic profile/mutations.

Of 54 EAPs reviewed, 53.7% restricted their target population due to contraindication/intolerance/ineligibility criteria versus the MA approved indication and 48.1% versus the P&R approved indication. Of the EAPs that were for orphan drugs, the most prevalent restrictions on early access were related to the disease's physiopathology and contraindications.

RESULTS (cont.)

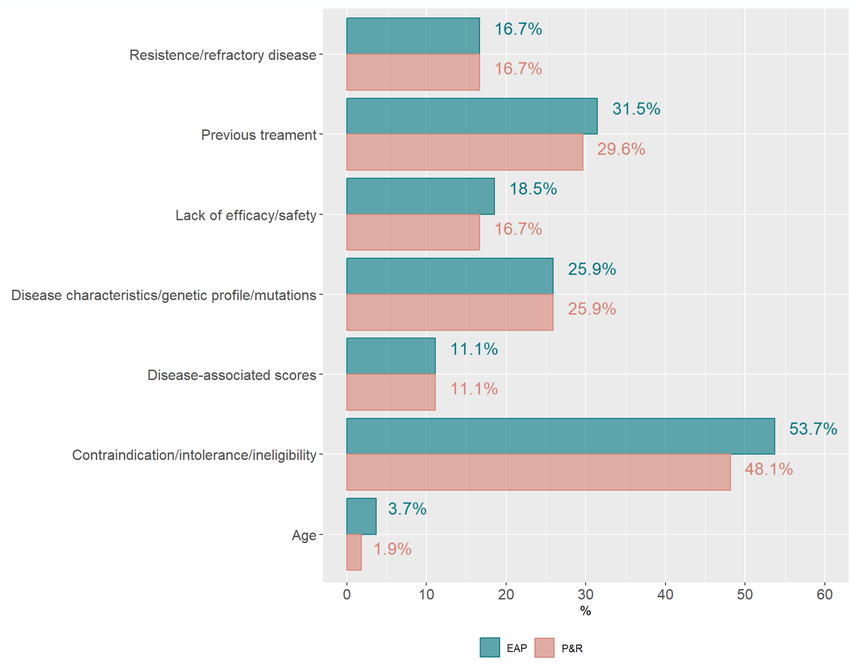

Figure 1. Distribution of the type of restriction in the EAPs indication versus MA approved indication and in the P&R approved indication versus EAP indication
EAP: Early access program; P&R: Pricing and reimbursement.

When the EAP had no restrictions, the indication considered in the P&R process also did not have restrictions. Of the 77.8% (n/N=42/54) of EAPs with restrictions, 66.7% (n/N=28/42) had no restrictions in the P&R approved indication.

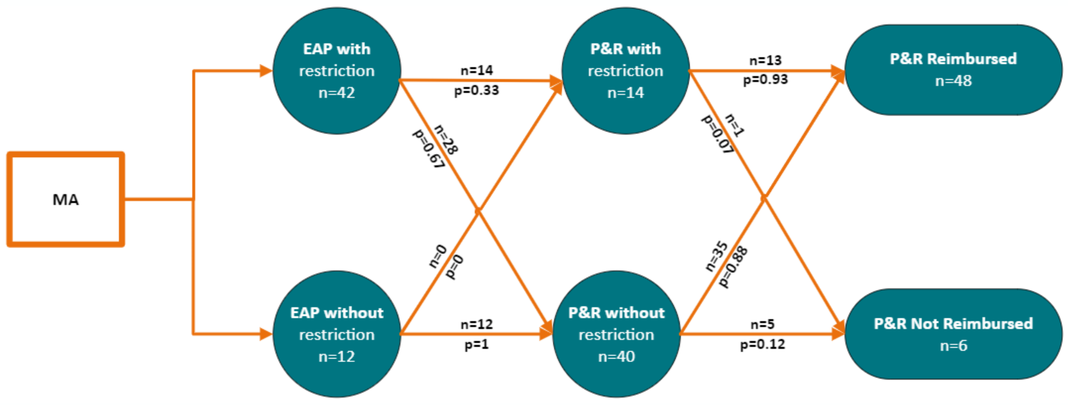

Figure 2. Flowchart since the marketing authorisation of drugs with EAP approval to their P&R decision
EAP: Early access program; MA: Marketing authorisation; P&R: Pricing and reimbursement.
n=number of EAPs; p=transition probability

The chance of restrictions in P&R approved indication compared to MA indication for those with restricted EAP indication is OR=12.7 (95%CI=[0.7, 230.4]) more times than those with unrestricted EAP. In addition, the Kaplan-Meier estimate suggests that the decision probability curve do not depend on the EAP restriction.

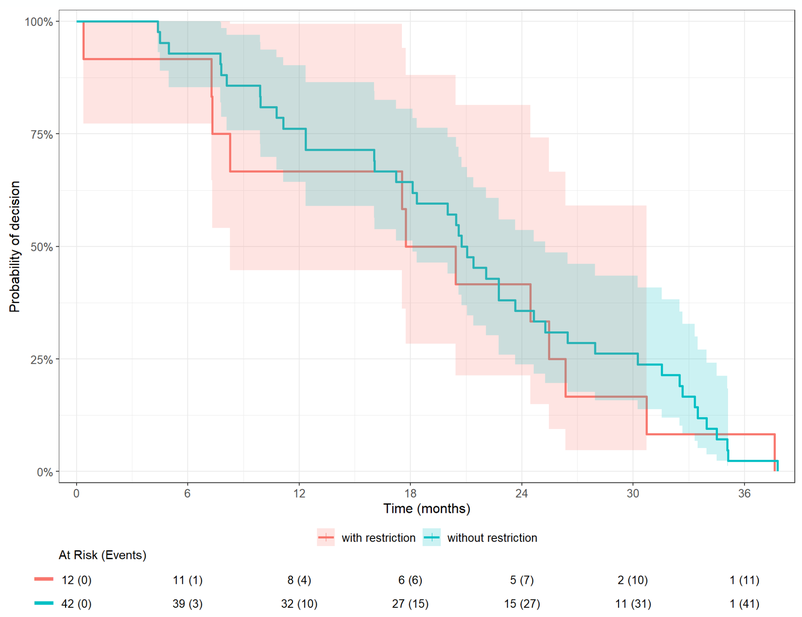

Figure 3. Time to P&R decision from EAP approval, with (red line) or without restrictions (blue line) on the EAP, using the Kaplan-Meier estimator

CONCLUSION

The most prevalent restrictions on early access to innovative medicines in Portugal are aligned with the legal requirement regarding the absence of therapeutic alternative for a specific target population in which patients are at immediate risk of life or of suffering serious complications.

REFERENCES: 1. INFARMED, Deliberação n.º 80/CD/2017: Programa de acesso precoce a medicamentos (PAP) para uso humano sem Autorização de Introdução no Mercado (AIM) em Portugal. 2017. 2. INFARMED. Programas de acesso precoce a medicamentos. 2024. [01/04/2024]; Available from: https://www.infarmed.pt/web/infarmed/avaliacao-terapeutica-e-economica/programa-de-acesso-precoce-a-medicamentos. 3. INFARMED. Lista de novas DCI/ indicações terapêuticas com financiamento público. 2024 [01/04/2024]; Available from: https://www.infarmed.pt/web/infarmed/relatorios-de-avaliacao-de-financiamento-publico. 4. World Health Organization (WHO). International Statistical Classification of Diseases and Related Health Problems 10th Revision. 2019 23/10/2024].