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Comparison of Alignment Between EMA Indications and National Reimbursement Decisions in Italy for Orphan Drugs

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Autors: Elena Chaiter[1], Alessandro d'Audino[1], Federico Tartarelli[1], Olivia Ramdin[1], Valeria Viola[1]
1 - Pharma Value srl, Rome, Italy

Objectives

Orphan drugs are developed for rare diseases, defined in the EU as conditions affecting fewer than 5 in 10,000 individuals. According to Regulation (EC) No 141/2000[1], these drugs respond to unmet medical needs and benefit from special regulatory pathways. However, the clinical development of orphan drugs often involves small patient populations, non-standardized trial designs, and reliance on unvalidated or surrogate endpoints, which may complicate the definition of the target population in the approved indication. These limitations can increase uncertainty in pricing and reimbursement decisions at the national level. This study aims to assess whether the Italian Medicines Agency (AIFA) narrows the EMA-approved therapeutic indications when granting reimbursement status.

Methods

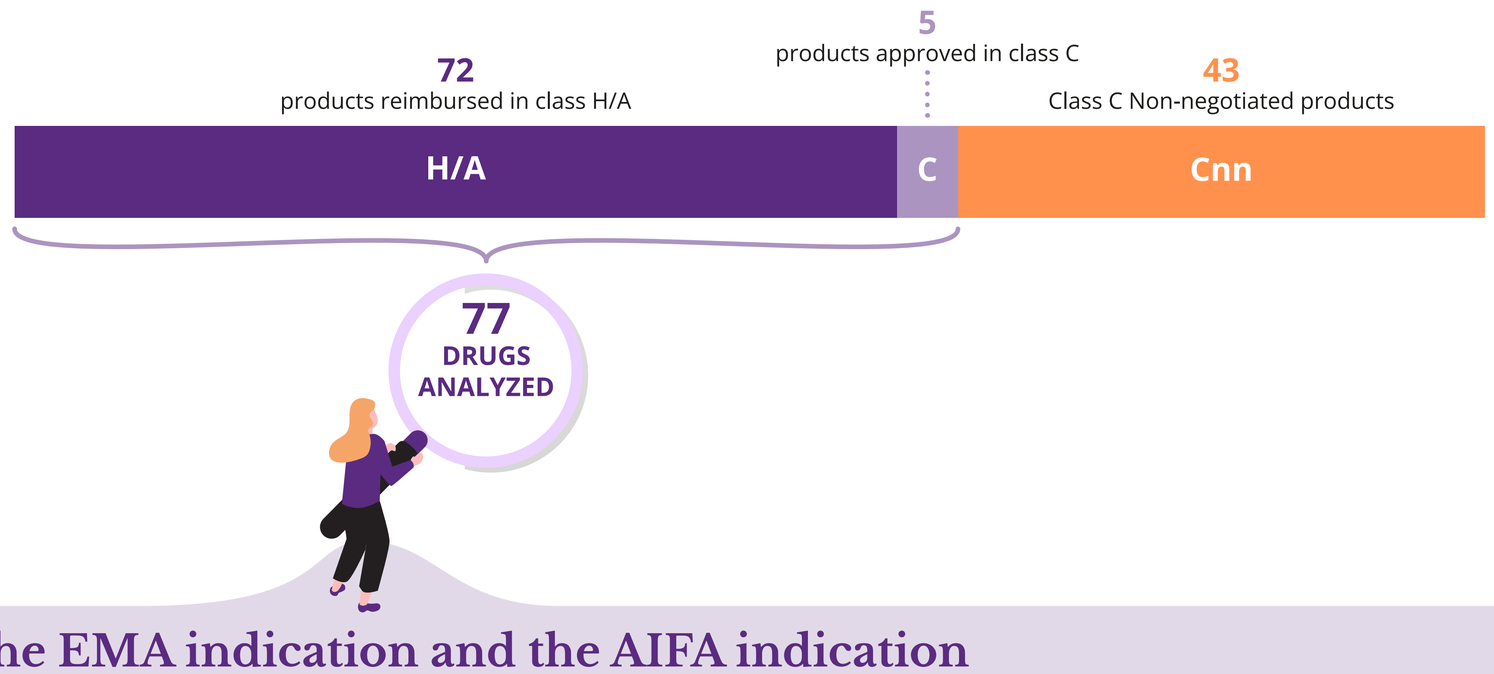
A dataset of 120 orphan-designated drugs approved between 2016 and 2024 was compiled. After excluding products in Cnn Class, 77 drugs remained for analysis. For each drug, we compared the approved indication published in the EMA's European Public Assessment Reports (EPARs) with the indication described in the corresponding Gazzetta Ufficiale publication by AIFA. The comparison focused on the presence of narrowing or restriction in the reimbursed indication.

Does AIFA restrict the therapeutic indications approved by the EMA when granting reimbursement?

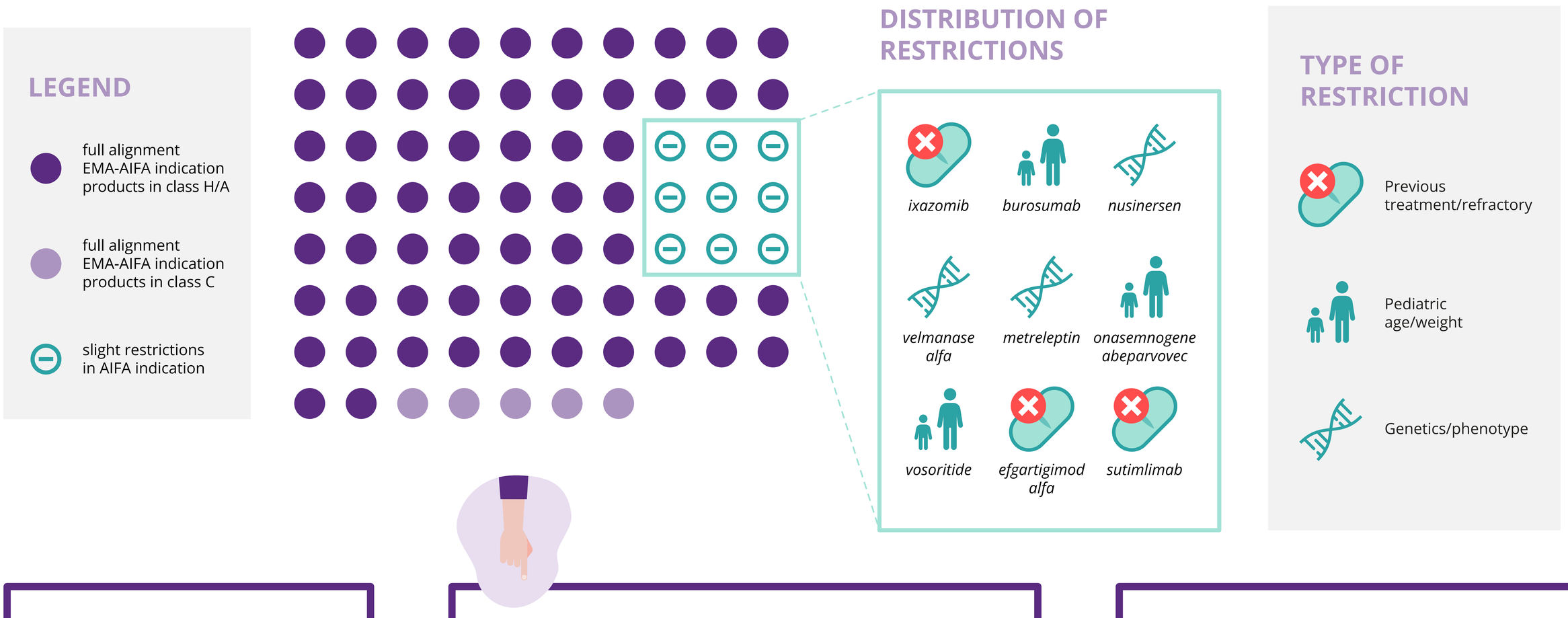
DATASET COMPOSITION

120 N° of orphan-designated drugs

2016 2024 Approval period



Results of the comparison between the EMA indication and the AIFA indication



Results

Among the 77 orphan drugs evaluated, 9 showed slight restrictions in the reimbursed indication compared to the EMA-approved version. These differences primarily concerned the treated population or specific disease subtypes. The remaining 68 drugs maintained full alignment with the EMA indication, with no further narrowing at the national level.

Conclusions

The findings suggest a substantial level of consistency between EMA-approved indications and national reimbursement decisions by AIFA. Although a minority of cases (9 out of 77) showed some restrictions - mainly concerning the eligible population - this practice appears limited. Overall, the observed alignment supports the objective of the EU Orphan Drug Regulation to ensure equitable access to treatments for rare diseases. AIFA's approach generally preserves the therapeutic scope defined at the European level.

References

1. European Commission. (2000). Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products. Official Journal of the European Communities, L 18, 1–5.

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for insights and questions: *valeria.viola@pharmavalue.it*