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COMPASSIONATE USE PROGRAMS THROUGHOUT THE EU: A CRITICAL APPRAISAL OF MEMBER STATES IMPLEMENTING RULES

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Riassunto

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The European pharmaceutical legislation aims at ruling all aspects related to quality, efficacy, and safety profiles of medicinal products through the grant of marketing authorizations (MA), thus protecting public health and favoring greater access to medicines throughout the Union territory. However, the regulatory timing required for granting a MA can be inadequate for patients affected by seriously debilitating or life-threatening diseases for which there are currently no suitable therapeutic options. Therefore, the regulatory frameworks at both European and national levels include provisions for allowing the compassionate use of medicinal products to a group of patients affected by above-mentioned diseases and who cannot be treated satisfactorily by an authorized medicinal product or be enrolled in a clinical trial. For medicinal products eligible for a centralized procedure, art. 83 of Regulation EC no 726/2004 establishes the so-called “Compassionate use programs” (CUPs) [1]. Despite the obligation for Member States (MSs) to notify to the EMA the start of a CUP, there is plenty of room for MSs in implementing provisions of Article 83 in their national regulatory frameworks on compassionate use. There is therefore a heterogeneous situation among MSs, also related to the definition of a CUP, and this leads to unavoidable significant differences in patients’ access to medicines potentially life-saving, from country to country.
The study aims to analyze both the CUPs established for rare diseases in the MSs from 2014 to 2023 and the different national regulatory frameworks of reference.
Institutional portals of MSs and their NCAs were reviewed to select those that have adopted specific provisions on compassionate use into their law. The NCAs of such MSs were interviewed to collect detailed data on the CUPs activated in the period of analysis and eligibility criteria for medicinal products.
Nineteen out of 27 MSs have legislated on CUPs but for only 11 MSs data on CUPs were collected. Between 2014 and 2023 571 CUPs were started. The MSs which most frequently have activated CUPs are: France (114), Italy (107), Germany (84), Belgium (68), and Romania (59). Among them, the 53.9% of all CUPs refers to medicines for which an orphan designation has been granted by EMA and are used to treat blood and lymphatic system disorders (32,24%), congenital, familial and genetic disorders (22,80%), neoplasms benign, malignant and unspecified (18,89%) and nervous system disorders (9,77%). In absence of EU-harmonized provisions, the national criteria for medicines eligible for CUPs vary among MSs. For an example, Estonia and Hungary require at least a phase II trial completed to consider a drug product as eligible. On the contrary, in Italy, CUPs are allowed to products with a completed/ongoing phase III trial, exception for rare diseases or rare tumors. Moreover, the Italian list does not distinguish between CUPs and other early access programs (i.e., extended access programs).
In conclusion, the application of Article 83 of EC Regulation no 726/2004 is heterogeneous among the MSs and, consequently, poses doubts on the equity of the patients’ access to medicines throughout the EU. This suggests a need to strengthen the efforts on harmonizing European and national regulatory frameworks on CUPs and other early access programs; it should pass through a more active role of EMA in defining unique eligibility criteria for products and patients involved in compassionate use.

Reference

1. Regulation (EC) No 726/2004 of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency.

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