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Compassionate use of medicinal products: a multicenter study

Compassionate Use (CU) is a way of making available to patients with an unmet medical need a promising medicine which has not been authorised for their condition. The main objective of this study was to describe the magnitude and profile of the CU in a sample of Andalusian public hospitals. As a secondary objective we wanted to analyze the evolution of these new treatment options under development.

Methods: A cross-sectional study including all applications of CU in 2010. Setting: 7 hospitals, 6 different provinces. Variables: center, clinical area, medicinal product, ATC classification, therapeutic indications, and approval by the EMA or AEMPS during the four following years.

Results: A total of 194 applications regarding CU of drugs were submitted to the respective Pharmacy Committees. Among them we identified 27 different types of CU. Reina Sofía Hospital (Córdoba) was the most active center, both in number of applications (24%) and variety of them (15 of the 27 types). Drugs such as dalfampridine, decitabine, defibrotide and figitumumab only were requested from this center. In all, the most frequent CU were: bendamustine for lymphoma (12% of applications) and 5-aminolevulinic acid for surgery for malignant glioma (12%), followed by treprostinil (10%), everolimus (8.2%) and idebenone (8%). The ATC L Group (antineoplastic and immunomodulating) accounted for almost 60% of all CU. Stem cell therapy for advanced chronic lower limb ischaemia, diacetylmorphine for heroin substitution and norcholesterol (radio drug) were also included as CU. After 4 years, 60% of drugs has achieved marketing authorisation. The remaining 40% have not yet managed license or have been rejected by the EMA (eg gentuzumab and idebenone).

Conclusion: CU showed a variable profile between the different hospitals. Early access to certain investigational drugs has been supported by the high percentage of marketing authorisations. However, there are uses with some uncertainty.