The aim of this analysis is to investigate MEAs adopted for new medicines. The prices of reimbursed products or indications linked to an Orphan designation are also added to the Registry. The Managed Entry Agreements (MEAs) were established by AIFA through the use of specific Monitoring Registries, in order to assure access to new medicines for all patients, to maintain the pharmaceutical budget by using these innovative drugs in target disease populations, and to avoid unnecessary expenses to the National Health Service (NHS). Through the AIFA’s Monitoring Registries System, it is possible to evaluate the drug utilisation in clinical practice, to gather epidemiologic data, to get information on safety profile or to collect ex-post evaluation about missing information. Actually there are two kinds of active Registries through the AIFA’s web system: Drug Product Registries (DPR), fully focused on the single drug, its (appropriate) use and impact in terms of cost for the Italian NHS, and Drug Product Therapeutic Area Registries (DP TAR), focused on the drug, but within the therapeutic algorithm. The Registry tracks the eligibility of patients, guaranteeing the appropriate use of medicines according to their approved indications and evaluating the effectiveness of treatment in clinical practice. Epidemiology, safety profile, and post-market data are also added to the Registry. The Managed Entry Agreements between AIFA and Companies are “financial based” (to manage budget impact) or “performance based” (to manage utilisation in real life or to provide evidence regarding uncertain decisions). Some Registries are also aimed to just monitor the appropriateness of drug prescription.

Objectives

The aim of this analysis is to investigate MEAs adopted in Italy on orphan drugs, and the released time elapsed from EMA approval to their availability on the Italian market.

Methods

All the MEAs released by AIFA have been systematically reviewed, integrating different sources such as AIFA Registries, Gazzetta Ufficiale, EMA website, Reimbursement Network, etc. Data has been gathered for a total of 34 (on a total of 125) products or indications linked to an Orphan Designation, under AIFA’s monitoring on April 2016.

Results

Out of 125 AIFA active Monitoring Registries on April 2016, 34 are related to drugs that obtained orphan designation. The most frequent type of MEA under monitoring is linked to a specific therapeutic area (12/34), followed by financial based agreements (12/34). All the performance based agreements except one (Signifor, for Cushing’s Syndrome) are related to oncological drugs. The 34 Monitoring Registries are about 26 drugs: Yondelis, Iclusig, Vidaza, Ad cetris, Thalidomide having 2 Registries differing for specific indication, and Lenalidomide has 4.

Conclusions

A variety of MEAs, including orphan drugs, are used in Italy in order to manage budget impact and uncertainty around clinical and cost-effectiveness. The use of MEAs represents a significant tool for the management of a sustainable pharmaceutical expenditure and also for the generation of further clinical evidences by Monitoring Registries’ adoption. They allow orphan drugs - and especially new and expensive therapies - to be available for the Italian patients, managing the difficult balance among sustainability, safety and effectiveness of treatments.

References


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