Introduction

In Italy, access to treatment for patients suffering from a rare disease is guaranteed through various legislative instruments. The centralized procedure represents the standard access route, whenever an orphan drug has no marketing authorization, patient access is ensured through different early access schemes. One of these early access tools is the Law 648/96.

Objective

This study aimed to assess AIFA’s approach on the inclusion of ODs in the 648 list. Approvals, rejections and methods followed by AIFA for its decisions were reviewed.

Methods

The drugs’ panel was built by systematically reviewing the CTS meetings decision (office “Area pre-autorizzazione”) from January 2013 to September 2015, checking the Scientific-Technical Committee (CTS). The list of orphan drugs (ODs) included in the 648 list was downloaded from AIFA’s website (https://www.aifa.governo.it/legge-648-96).

The inclusion of a medicinal product in the 648 list is performed by AIFA on the basis of a documented request from patients’ associations, scientific societies, health facilities, universities or following recommendations of AIFA’s Scientific-Technical Committee (CTS). The list of orphan drugs (ODs) included in the 648 list, and analysing each single decision taken by the CTS.

For all of them, we systematically checked the issuing date of the reimbursement authorisation valid throughout the year, the year of the date of inclusion in the 648 List, the date of exclusion from the 648 List, the date of the price publication in the Italian Official Journal (I.O.J.), the launch date in the reimbursed class.

The related dates of reimbursement were collected via the official websites of: Agenzia Italiana del Farmaco (AIFA), The European Medicines Agency (EMA), I.O.J., Community Register of Medicinal Products.

The differences between the time of EU approval and the inclusion in the 648 List, the permanence time in the 648 List, and where applicable, the time to market have been evaluated (mean and median).

Results

AIFA evaluated 45 requests for orphan drugs’ inclusion in the 648 List during the considered time-frame of this analysis:

- 13 requests were approved (29%);
- 32 requests were not approved (71%).

There were several requests for some drugs and for this reason the total number of the evaluated drugs was equal to 37.

Conclusions

The Law 648/06 is one of the early access schemes in Italy, which allows the supply of specific drugs - not yet available in Italy or undergoing clinical trials or off-label - that are reimbursed by the NHS. These drugs are inserted in the so-called 648 List and supplied to patients in order to respond to pathological conditions with no alternative therapeutic options (when therapeutic alternatives are available the drug’s inclusion in the 648 List is subject to parameters of affordability and appropriateness and its use in the considered indication, different from the authorized one, shall be recognized by the medical-scientific community).

Between Jan 2013 and September 2015, 45 requests on inclusion in the 648 List (i.e. 37 ODs) were evaluated and 29% of them were approved. Of these, 11/13 drugs (85%) were still in the 648 List and only 2/13 (15%) were excluded after obtaining reimbursement status for the 648 indication (158.5 median time). The most frequent ATC was the L (antineoplastic and immunomodulating agents) (59%).

Our data showed that the 648 List is a powerful early access tool to allow early access to ODs for Italian patients.

REFERENCES:

AIFA: http://www.aifa.governo.it/index?Fattura=8667
AIFA Law: http://www.aifa.governo.it/legge-648-96
AIFA Twitter: @AIFA_Italy; @aifa_news; @AIFA_Italia