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EARLY ACCESS PROGRAMS: AN ANALYSIS SINCE THEIR ESTABLISHMENT IN ITALY

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Introduction

Italy has a broad range of Early Access Programs (EAPs) that allow the access of medicinal products to market before their authorization at National level. The Italian **Law 648/96** allows the use of three types of medicinal products at National Healthcare System (NHS) charge: innovative drugs for which the sale is not yet authorized in Italy; drugs without authorization, but have undergone clinical trials; and drugs to be used for a therapeutic indication different from the authorized one (*off-label*). The **AIFA 5% fund**, according to Article 48 of Law 326/2003, requires all pharma companies operating in Italy to pay 5% of their promotional expenses to an independent research fund, designed to promote orphan diseases research and to make available to rare diseases' patients medicines awaiting market entry. **Compassionate Use** (CU), or expanded access, is the therapeutic use of investigational drugs outside clinical trials. In Italy, the CU is regulated by the Decree of 8 May 2003 "Therapeutic use of medicinal products subjected to clinical trials", according to which a drug can be requested for use outside clinical trial "when there is no valid therapeutic alternative to the treatment of serious illnesses, or rare diseases, or disease conditions that put the patient's life at risk".

The objective of the present work was to analyse the success rate of EAPs requests since their implementation in Italy and understand which factors influenced AIFA's decisions and change over time. In our research, we investigated the trend of the three EAPs in place in Italy: drugs under 648/96 law, AIFA 5% fund and compassionate use programs (CUPs).

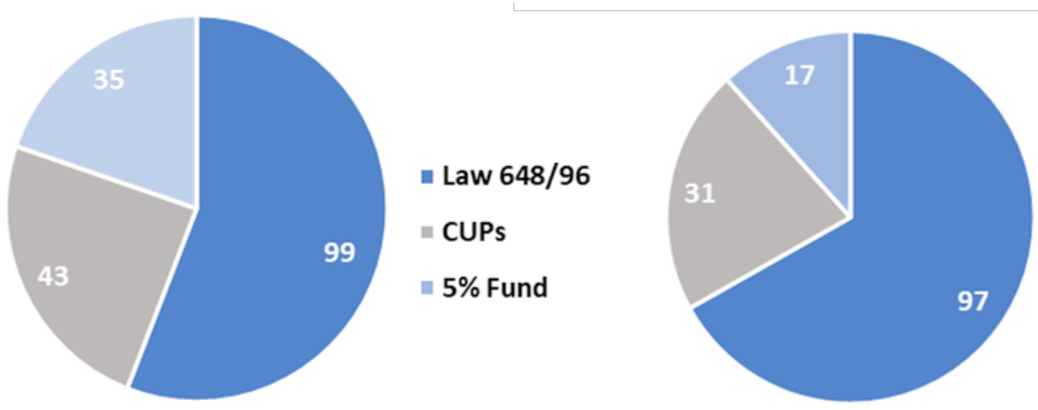
Methods

An Excel database was created to track EAPs since their implementation in Italy up to July 2018. Data were retrieved from different published sources: drugs included in the law 648/96 list and CUs were found on the AIFA website[1,2]; drugs in the 5% fund list were gathered from AIFA official reports on drug use (OSMED)[3]. Sensitivity analyses were conducted to test parameters mostly influencing AIFA's decisions.

Results

Of 99 drugs entered in the 648/96 list since 1997, only two are not anymore under this class. The CU list included 43 drugs since 2011, 19 of which added since 2017; at July 2018, the total number of drugs included in the list was 31. Since 2011, 35 drugs were included in the 5% fund; of those, only 17 drugs have remained in this list (Figure 1).

Figure 1. Number of drugs entered (left) and remained in 2018 (right) in EAPs

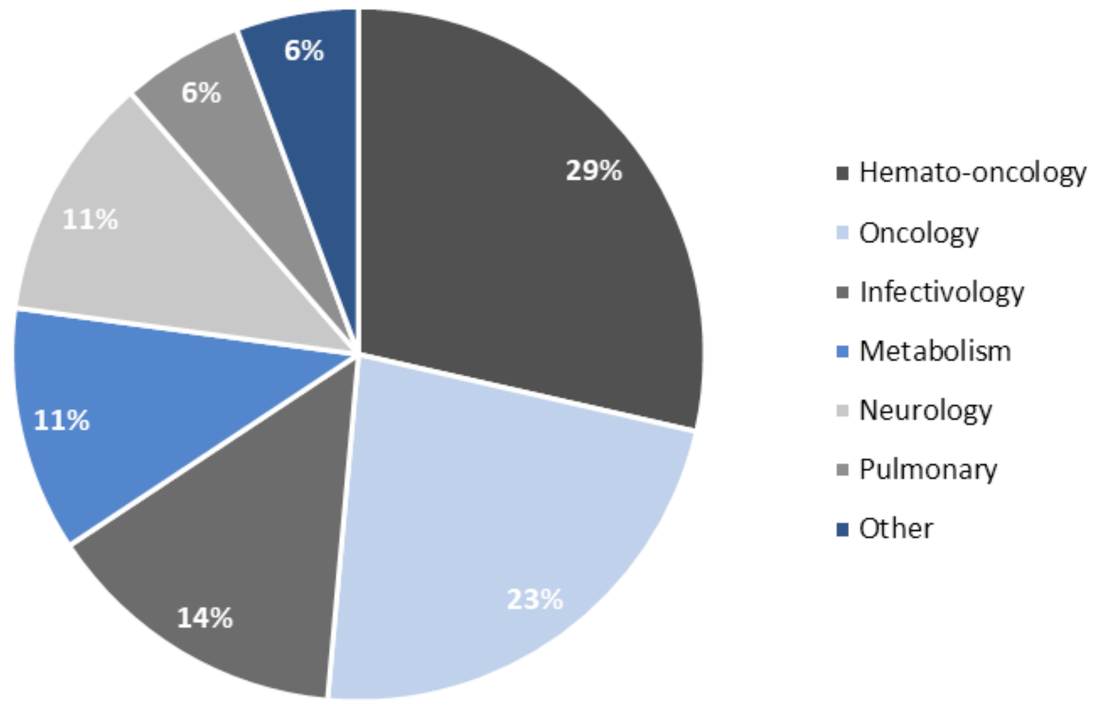


The 648/96 list included mostly drugs for rare diseases (68%), covering different therapeutic areas. Of the 124 examined applications, 43 received a negative evaluation. Main reasons for such refusal were: lack of scientific data, presence of a valid therapeutic alternative, official approval in the meantime or suggestion to access the 5% AIFA fund.

Regarding the CU program, cancer drugs represented the majority (around 60%) of approved drugs, followed by immunomodulators and CNS (11%) drugs, while drugs for respiratory and gastrointestinal areas were less than 3%.

The 5% fund list, included 35 drugs in the analyzed timeframe, most of which were for onco-hematological and oncological diseases (Figure 2). Sensitivity analyses showed that in general drugs were refused an EAP for lack of scientific data or for the presence of valid therapeutic alternatives.

Figure 1. Type of drugs included in the 5% AIFA fund list



Conclusions

Law 648/96 guarantees early access to drugs that, despite being still under clinical investigation and awaiting for EMA approval or local registration, have demonstrated a clear benefit in a cohort of patients.

Law 326/2003 together with the Ministerial Decree of 8 May 2003 are also a valuable resource for rare disease patients needing for treatment.

Our survey shows that, except from cases where there was clear lack of scientific data or adequate eligibility criteria, all patients in need had access to the necessary treatment.

Our study demonstrates the strong AIFA commitment to allow early access to drugs for patients in high need.

This is crucial especially in oncology and other life-threatening diseases, where there is poor market knowledge and precise data on epidemiology and place in therapy are often not available.

References

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