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TIMELY ACCESS TO THERAPIES FOR SEVERE DISEASES WITH UNMET MEDICAL NEED

Enrico Bosone

SIAR President

Timeline of Authorization and reimbursement for oncology drugs in Italy

- Prada_2017.pdf

- AIFA average evaluation time from
  - 264 days between 2013-2014

  TO
  - 219 days between 2015-2016

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RESEARCH @ POINT OF CARE

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Timeline of authorization and reimbursement for oncology drugs in Italy in the last 3 years

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ABSTRACT

Introduction: The main purpose of this analysis was to quantify the time elapsed between the validation date of European Medicines Agency (EMA) centralized procedure and the first purchase of a product by at least 1 Italian health care structure, evaluating different variables that affect the process, the number of products approved by the Committee for Medicinal Products for Human Use (CHMP) that are available on the Italian market (July 2016), and the impact of the Cnn class for oncology drugs in Italy.

Methods: A panel of oncology products has been defined, which considered drugs approved by the EMA between January 2013 and December 2015, and authorized for the treatment of oncology diseases, excluding generics. Data were obtained via the EMA website by the Agenzia Italiana del Farmaco (AIFA; the Italian Medicine Agency) meeting reports, by official administrative acts of marketing authorization, and the date of the first purchase (first day of the first handling month).

Results: The mean time of EMA evaluation for the considered panel of medicines was about 441 days (standard deviation (SD) 108; range 266-770); the average approval time for AIFA was about 248 days (SD 131; range 85-688). Interestingly, the mean AIFA evaluation time decreased significantly from 264 days for products submitted to AIFA assessment in 2013-2014 to 219 days for products evaluated in 2015-2016. Focusing on the regional access, both the timing and the number of drugs available for patients were widely different from region to region.

Discussion: A reduction in the approval time in the last 2 years has been observed in Italy. However, several variables influence the efficiency of the process and need to be addressed to make the access to drugs timely and efficient.

Keywords: Market access, Patient access, Reimbursement, Time to market

Introduction

New oncology drugs in Europe are assessed by the European Medicines Agency (EMA) and are authorized with a centralized procedure, which came in force in 1995 and allows applicants to obtain a marketing authorization that is valid throughout the entire European Union (EU) (1). However, even if a European marketing authorization has been granted, this does not imply that the product will be immediately available to patients everywhere in the EU (2).

The Italian model is characterized by a centralized National Health Service (NHS), responsible for drugs assessment, pricing and reimbursement (P&R). The regions are the budget holders and, therefore, are responsible for the local provision of healthcare (3), and they represent a second real hurdle for the market access of new treatments (4).

Once authorized by the EMA, Italy, as a member state of the European Community, automatically accepts drug marketing authorizations by defining the supply regimen and starting with the pricing and reimbursement process.

In Italy, the price setting of medicines reimbursed by the NHS is regulated at the central level by the Agenzia Italiana del Farmaco (AIFA), the Italian national regulatory authority (5). AIFA provides, with its scientific authority and autonomy, the clinical, scientific, and economic evaluation regarding medicines. AIFA activities are supported by 2 technical scientific committees consisting of experts of well-established experience.

- Technical Scientific Committee (CTS, Commissione Tecnico Scientifica): it assesses the national and European marketing authorization applications, and delivers a consultative opinion on them and provides classification for reimbursement; it takes over the tasks formerly attributed to the National Drug Evaluation Board at the Ministry of Health.

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- Three hurdles:
  - Regulatory Assessment by EMA
  - HTA Bodies
  - Payers
- Result : relevant delay to real access for Patients

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What patient wants

Sylvie Menard : *"the most important thing for a patient with cancer is having **immediately** the best therapy"*

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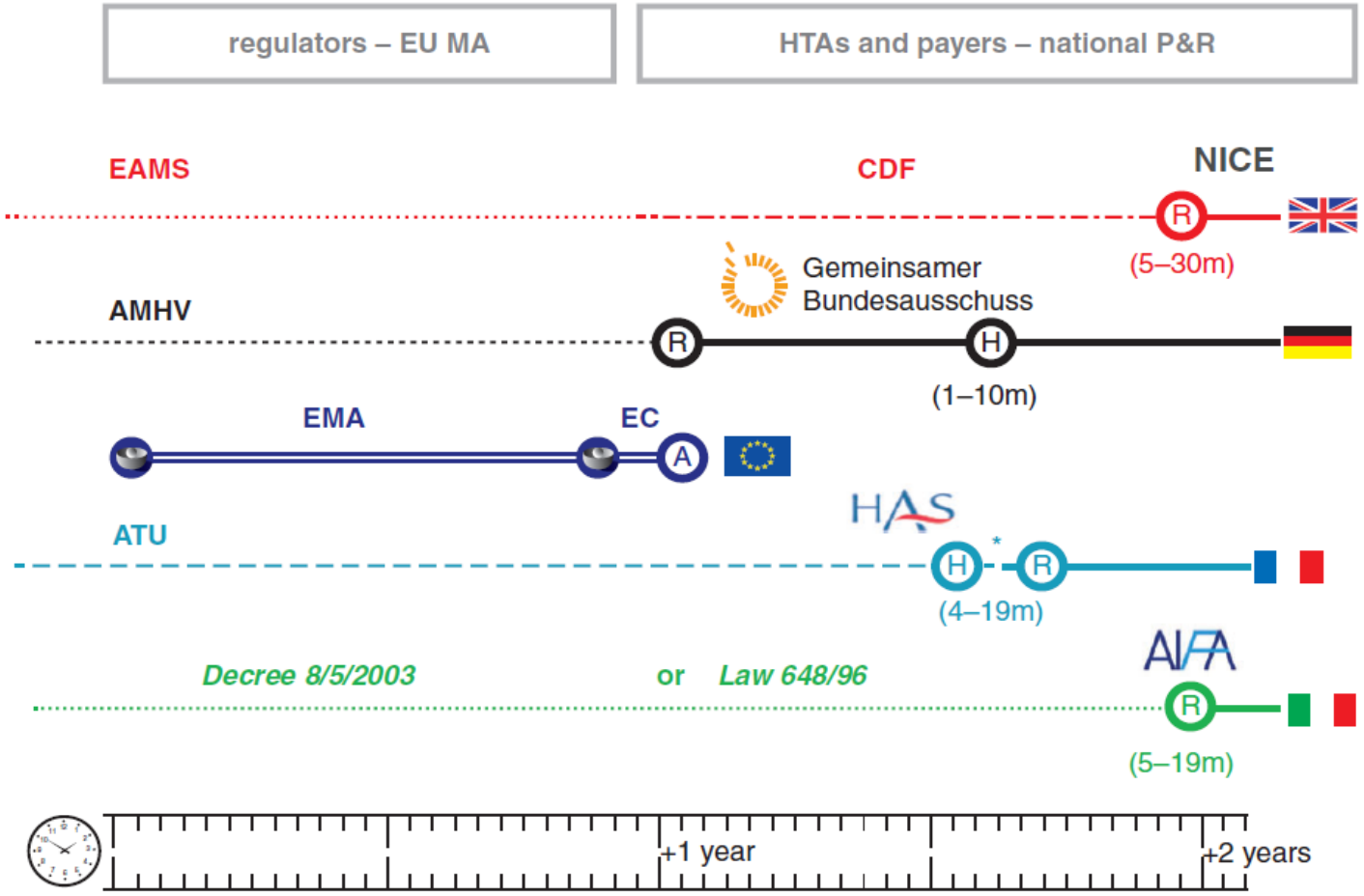
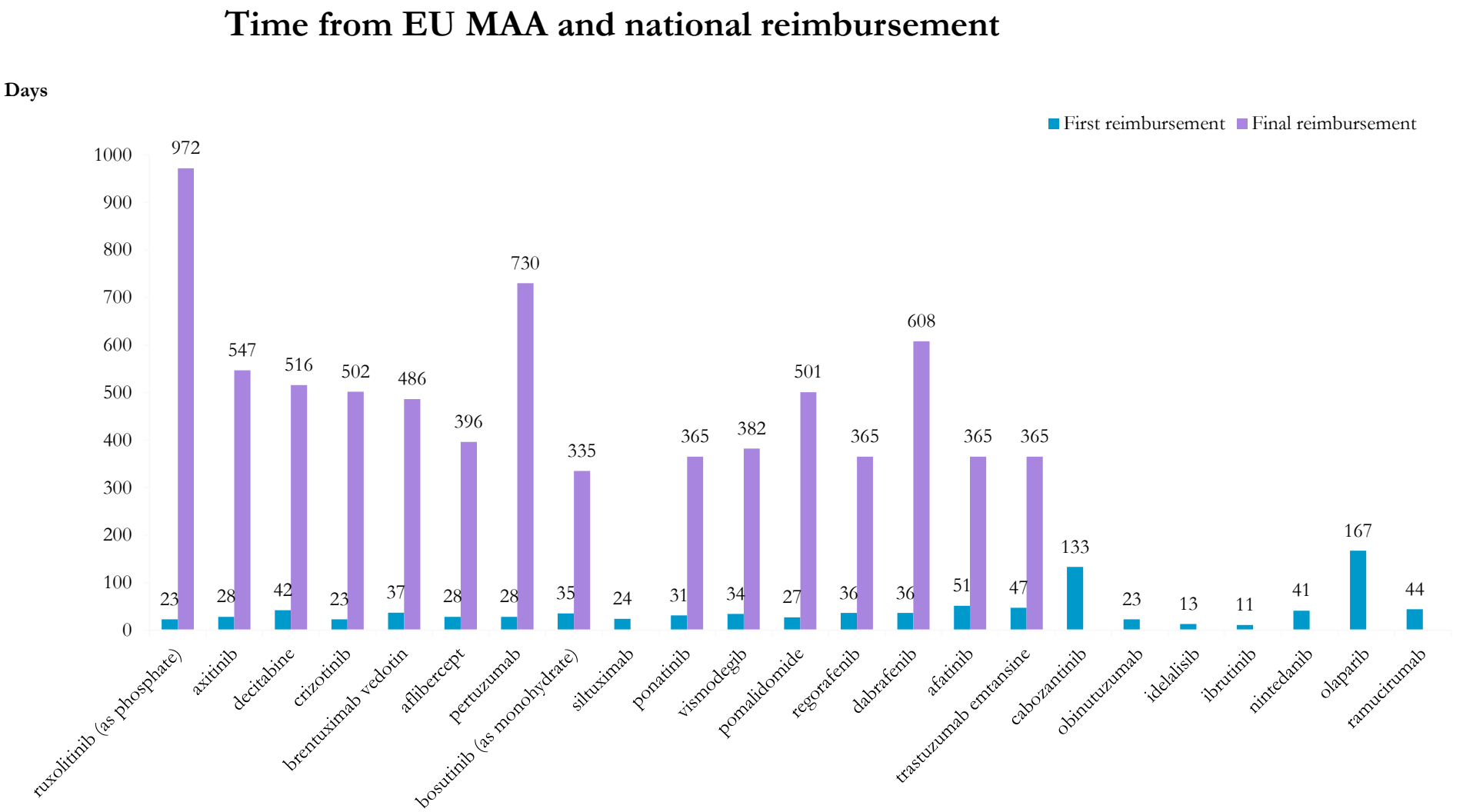
Current situation: delays and inhomogeneities



Early market access of cancer drugs in the EU

J. Martinalbo[1], D. Bowen[1], J. Camarero[2], M. Chapelin[1], P. Démolis[3], P. Foggi[4], B. Jonsson[5], J. Llinares[1], A. Moreau[3], D. O'Connor[6], J. Oliveira[7], S. Vamvakas[1] & F. Pignatti[1]*

Current real availability in Germany: examples 2012 – 2014 (SIAR NEWS 69)



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When the real availability is urgent

The timely **REAL** availability of a new treatment is very urgent when:

- It is for a **severe disease**
- It is for an **unmet medical need (**no available treatments or when the new one has a significant benefit or a major contribution to patient care)

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European incentives for medicines when the real availability is urgent

Many incentives at European level for medicines addressing an unmet need in severe diseases :

- Orphan Medicinal Products: Regulation 141/2000
- Extension of indication with one additional year of protection: Article 14(11) Regulation 726/2004
- Conditional MA: Regulation 507/2006
- Accelerated procedures: Regulation 726/2004
- PRIME procedure

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OMP: Regulation 141/2000

1. A medicinal product shall be designated as an orphan medicinal product if its sponsor can establish:

(a) that it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10 thousand persons in

that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorised in the Community or, if such method exists, that the medicinal product will be of significant benefit to those affected by that condition.

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Significat benefit for OMPs (Regulation 847/2000 Art 3)

2. For the purposes of the implementation of Article 3 of Regulation (EC) No 141/2000 on orphan medicinal products, the following definition shall apply:

— 'significant benefit' means a clinically relevant advantage or a major contribution to patient care.

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European Assessment
about severity and unmet need during MA evaluation

- For OMPs : by COMP, updated at the MA
- For extension of indication: by CHMP at the time of the approval of the new indication
- For Conditional MA: by CHMP
- For accelerated procedure: by SAWP/CHMP
- For PRIME: by SAWP/CHMP

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Status at 31 Dec 2015

Total European MAs	878
•OMPs	92 (10.5%)
•Extensions with SB	17 (1.9%)
•Conditional MAs no OMP	6 (0.7%)
•Accelerated no OMP/CMA	18 (2.1%)
Total “priorities”	133 (15.2%)

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SIAR proposal for timely access to therapies for severe diseases with unmet medical need (1)

- Additional administrative timely national procedures for therapies recently (less than 6 months) evaluated by CHMP/COMP, being for severe diseases without alternatives (or with a clinically relevant advantage or a major contribution to patient care)
- MA by consensus
- Immediate reimbursement by the NHS at the European MA time
- Initial price decided by the sponsor equal to the lowest price in EU
- In addition to the usual national negotiation

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SIAR proposal for timely access to therapies for severe diseases with unmet medical need (2)

- Possible 100% pay back of the difference between the temporary and the final price
- National Register if requested
- Maximum turnover in the first year: 0.5% of expenditure for all the reimbursed medicines ?
- Country by country rules in case of failure (for example class C in Italy with a partial pay back)
- Possible synergy with the “first HTA” by EMA - EUnetHTA

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How to **contribute** to the European debate

Show and discuss the proposal with :

- Patient Associations (Cittadinanzattiva, Active Citizenship Network, Other Patient Associations in Europe)
- Scientific Associations (Benzi Foundation,SSFA)
- Institutions (MinSal, European Parliament)
- Other stakeholders

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Thank for your attention