Expectations from the revision of the System

27 October 2017

Enrico Bosone
SIAR President

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SOCIETÀ ITALIANA ATTIVITÀ REGOLATORIE

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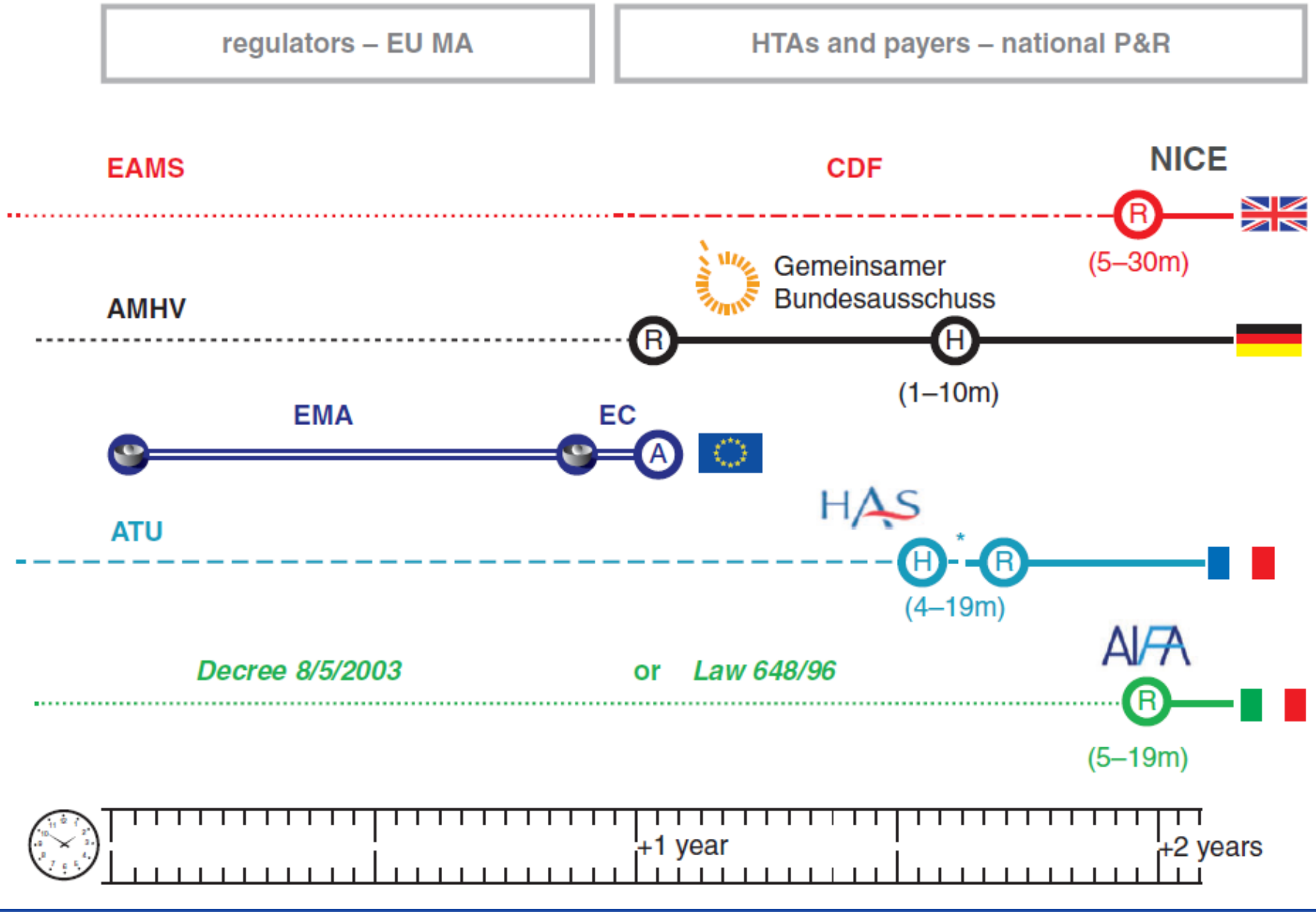
Medicines in Europe: delays and inhomogeneities



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]*

When the democratic availability is really urgent ?

- The timely **REAL** availability of a new treatment is really 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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EuMA 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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EuMA status at 31 Dec 2015

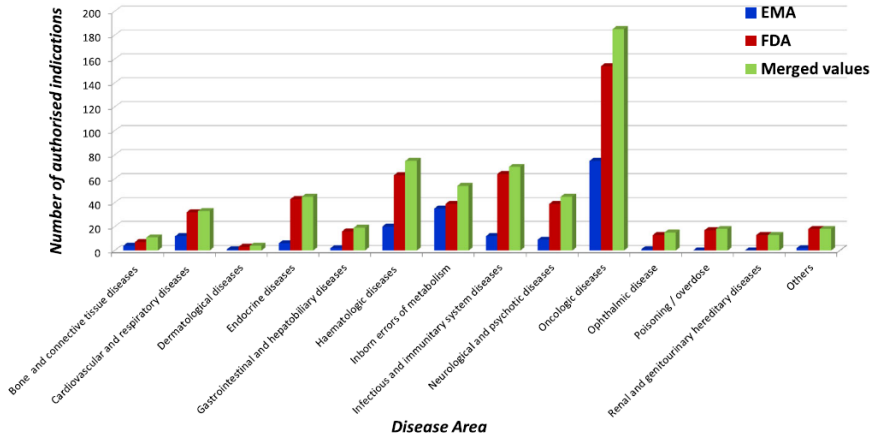

Fig. 6 Distribution of authorised indications for rare conditions per disease area

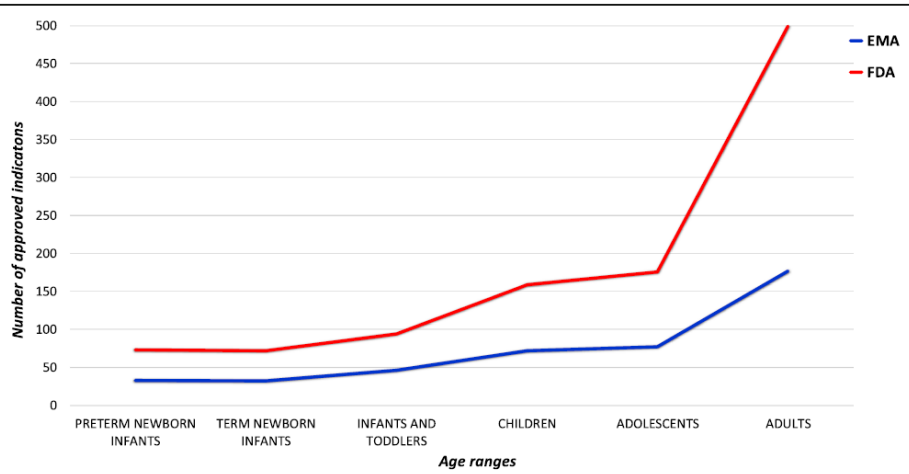

Fig. 5 Distribution of indications in the EU and US per age groups. Legend: label not available for 7 ODDs

Orphanet 2017; BMJ Open 2017

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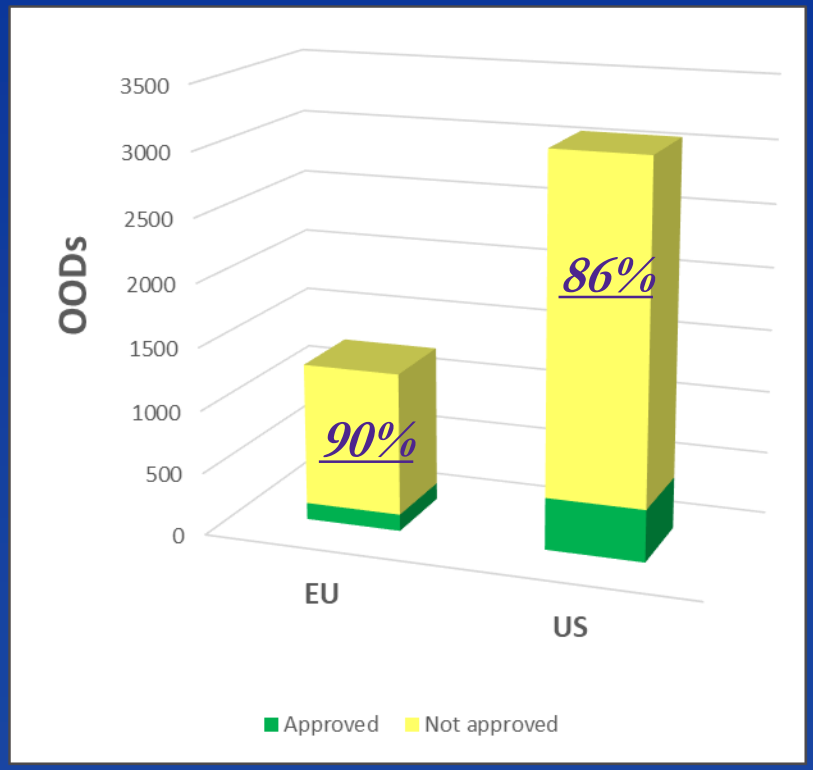
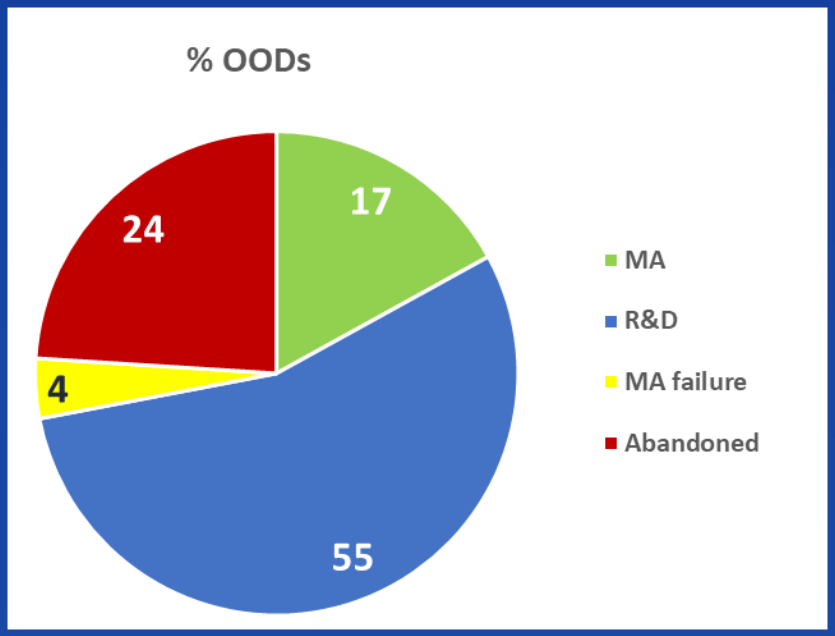
EuMA status at 31 Dec 2015

A large percentage does not reach marketing approval



27.8% failures



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BUT

- The European Marketing Authorization is not sufficient for the **TIMELY** real universal availability, also for «primary» Medicines

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Update OMPs 2016-2017

- 2016 : 14 OMP MAs *versus* 81 CHMP positive opinions
- 2017 : 14 OMP MAs *versus* 92 CHMP positive opinions
- *2018 : 14 OMP MAs up to 17 October 2018*
- Trend : fron 8-10 OMPs per year to about 15
- Total number of OMP MAs 142 (end of 2017)

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Timely availability in Italy for OMPs

Molecule	Disease	MA	Delay (months)	Note
daratumumab	MM	20/05/2016	13	
ixazomib	MM	21/11/2016	07	Class C
cells (Strimvelis)	Severe immunodef.	26/05/2016	02	
allogenic T cells	GVH disease	18/08/2016	17	
migalastat	Fabry disease	26/05/2016	09	
eftrenonacog	Hemophilia B	12/05/2016	09	
venetoclax	CL Leukemia	05/12/2016	08	
obeticholic acid	Liver cirrhosis	12/12/2016	07	
pitolisant	Narcolepsy	31/03/2016	11	Class C
albutrepenonacog	Hemophilia B	11/05/2016	08	
olaratumab	Sarcoma	09/11/2016	08	

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Timely availability (2)

Molecule	Disease	MA	Delay	Note
irinotecan	Pancreatic neoplasm	14/10/2016	>18	Under evaluation
human coagulation factor X	Factor X deficiency	16/03/2016	03	C nn
avelumab	Neuroendocrine tumors	18/09/2017	04	
nusinersen	Muscolar spinal atrophy	30/05/2017	04	
dinutuximab	Neuroblastoma	08/05/2017	14	
midastaurin	AMM Leukemia	18/09/2017	10	
niraparib	Fallopian tube neoplasm	16/11/2017	09	
inotuzumab	LL Lymphoma	29/06/2017	11	
chlormethine	Mycosis Fungoides	31/03/2017	07	C nn
cenegermin	Keratitis	06/07/2017	06	

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Delay 2016-2017 OMPs availability

- The average delay between the centralized MA and the classification for reimbursement in Italy for OMPs has been :
  - 9,6 months (2-18)

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Expectations from the revision of the OMP Regulation

- Clear and detailed explanation of the «major contribution to patient care (MCPC) or the clinical relevant advantage (CRA)» in the EPAR and during the meetings with Payers / HTA bodies

- Faster national reimbursement in the States of the European Union

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Thank for your attention

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