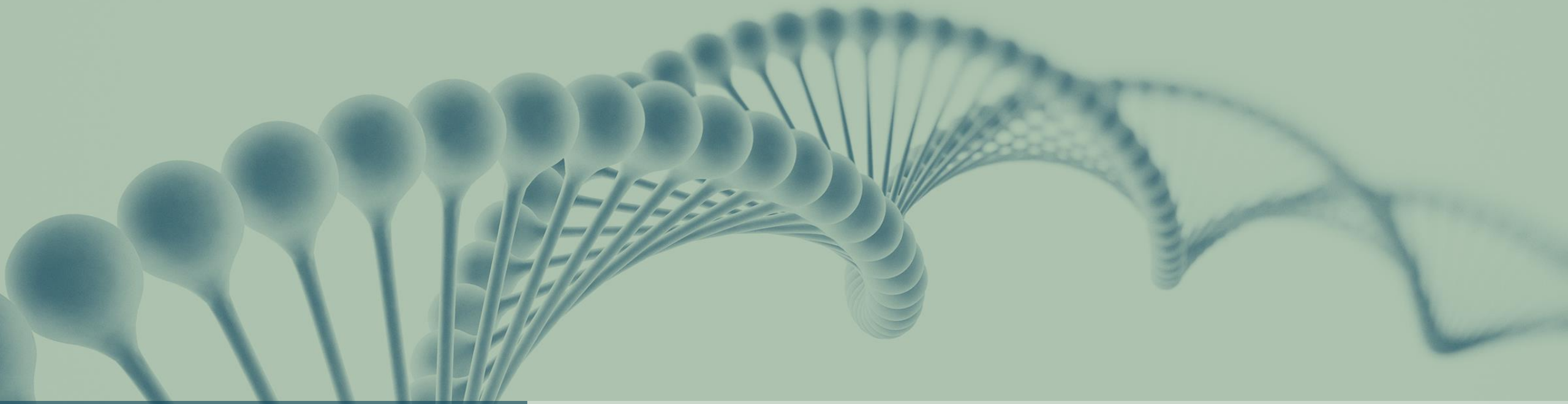


"Registry Studies and Health Technology Assessment (HTA)"

Thursday, June 23, 2022

AIFA registries: past and current & upcoming challenges

Entela Xoxi

Disclosure

- EUnetHTA & IMPACT HTA member on behalf of Catholic University of Rome, School of management & Economics
- IMI European Health Data & Evidence Network (EHDEN) Data Source Prioritisation Committee Member
- Expert Advisory Group Member of ROADMAP IMI BD4BO
- Former Italian Medicine Agency (AIFA): Co-ordinator of AIFA registries

Consultancy, participation in international & national Advisory boards, receives honoraria for courses, seminars, workshops.

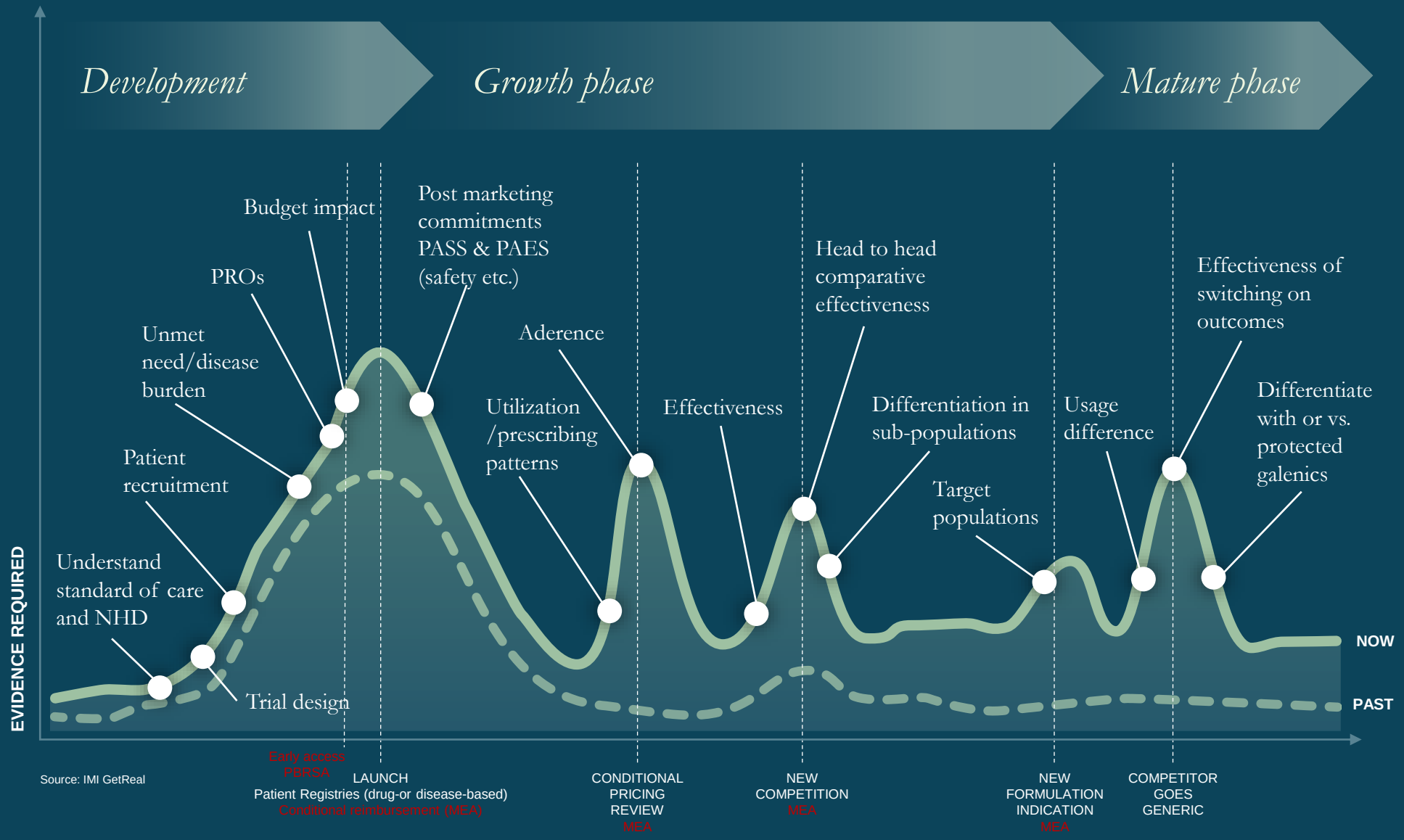
Italian Medicine Agency

Since 2004, prices of all medicines reimbursed by the Italian NHS are set through Negotiation procedure between AIFA & Pharmaceutical companies.

1. AIFA Technical-Scientific Commission (CTS)
2. AIFA Pricing and Reimbursement Committee (CPR)

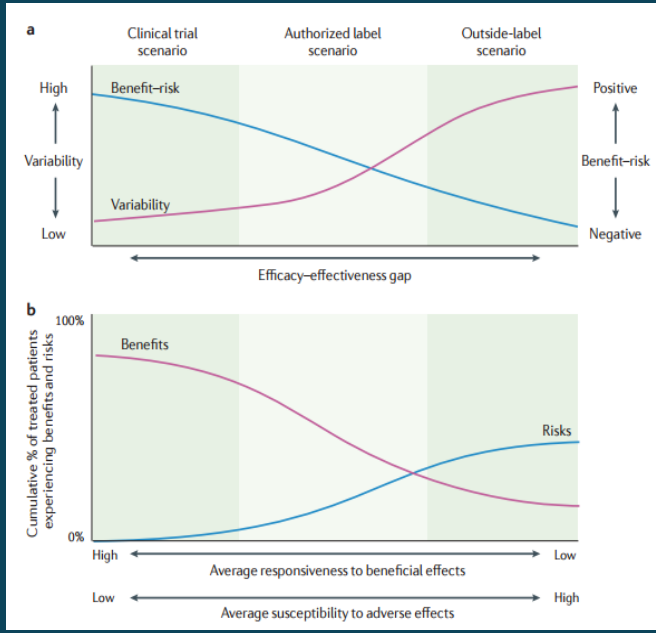
- Early access tools (648/1996 Law)
- **AIFA REGISTRIES** (2005)
- Managed Entry agreements (2005)
- New Innovativeness' recognition (2017)
- New GL on PR negotiation (2020)

Real-World Data



Adapted from IMI GetReal

The regulatory procedures & pathways + early access



Eichler HG et al., Bridging the efficacy-effectiveness gap: a regulator's perspective on addressing variability of drug response. Nat Rev Drug Discov. 2011 Jul 1;10(7):495-506. doi: 10.1038/nrd3501

❖ Accelerate assessment (**PRIME scheme**)

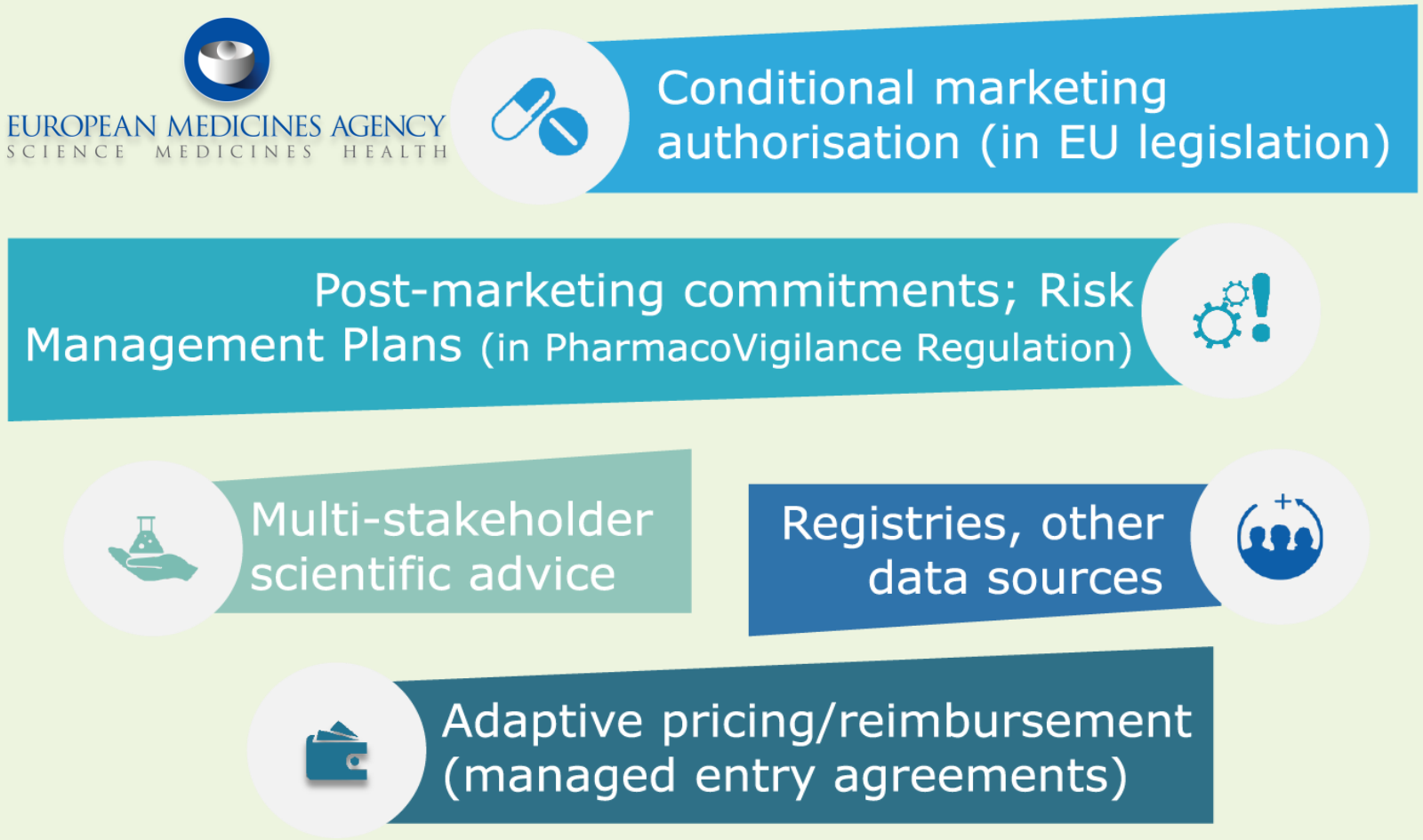
- Conditional Marketing Authorisation (**PRIME scheme, Adaptive pathway**)
  - Under Exceptional circumstances

❑ Orphan designation (OD) (**PRIME scheme**)

✓ Post-authorisation Effectiveness or Safety Studies (**PAES or PASS**)

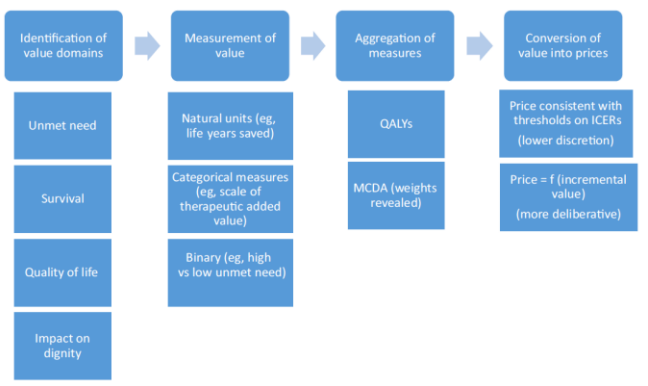
- Compassionate use/ Expanded Access Program (**OD**)
- Special schemes (**OD**): 648/96 IT Law

Adaptive pathways



EMA workshop on Adaptive pathway, 2016. Available at https://www.ema.europa.eu/en/events/adaptive-pathways-workshop Accessed 6 June 2022

Value-based pricing



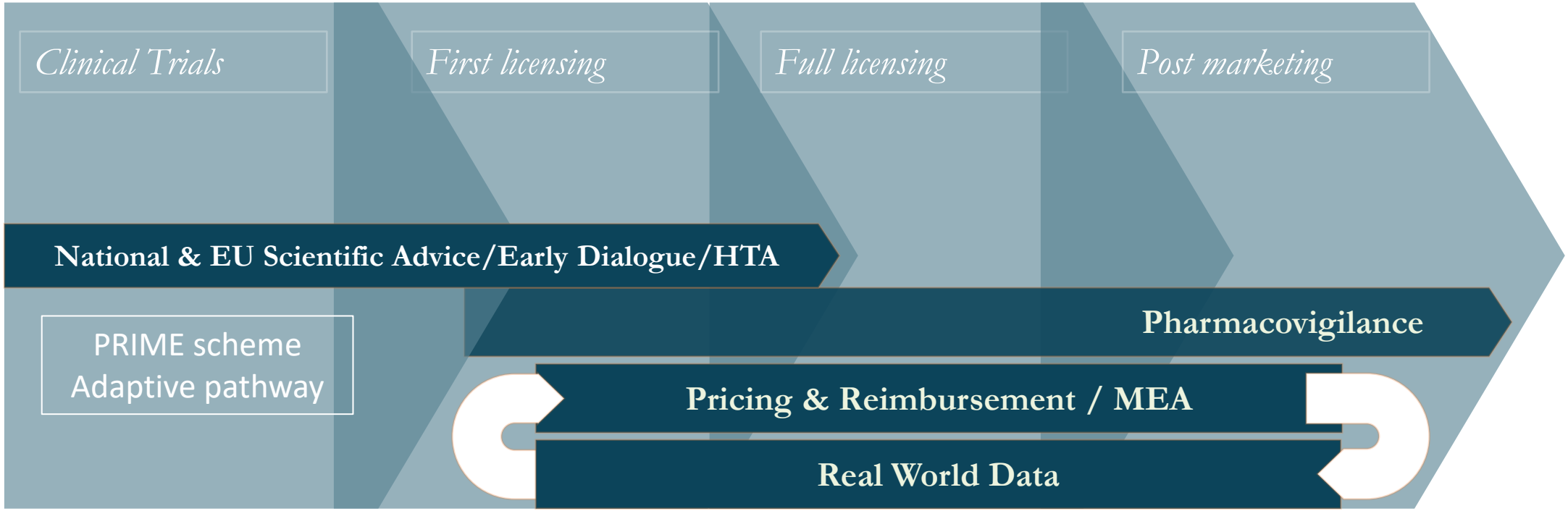
ICER, Incremental cost-effectiveness ratio; MCDA, multi-criteria decision analysis; QALYs , quality-adjusted life years

Country	Negotiating Entity	Value Criteria	MEAs	Role of ICER	Indication-based Pricing
France	CEPS (CEESP for economic evaluation)	Additional clinical value (graded)	Mainly finance-based (price/volume agreements)	For moderate to high additional clinical value/budget impact more than €20 million	No
Germany	SHI (Discount)	Additional therapeutic value (graded)	–	In principle, an efficiency frontier	No
Italy	CPR-AIFA	Additional clinical value	Both finance-based and outcome-based	Suggested for "very innovative drugs" and medicines for orphan diseases	Yes, through MEAs
Spain	CIPM	Additional clinical value	Mainly finance-based	–	No
United Kingdom	DoH (MEAs)	QALYs	Mainly finance-based	Most important criterion	Yes, through MEAs

CEESP = Economic Evaluation and Public Health Committee; CEPS = Health Products Economic Committee; CIPM = Prices and Reimbursement Inter-ministerial Committee; CPR-AIFA = Price/Reimbursement Committee–National Medicines Agency; DoH = Department of Health; MEA = managed entry agreement; QALYs = quality-adjusted life years; SHI = Social Health Insurance.
Elaboration on Jommi and Minghetti,[9] Panteli et al,[10] Theidel and von der Schulenburg,[11] and Toumi et al.[12]

Jommi C, Armeni P, Costa F, Bertolani A, Otto M. Implementation of Value-based Pricing for Medicines. Clin Ther. 2020 Jan;42(1):15-24. doi: 10.1016/j.clinthera.2019.11.006. Epub 2019 Dec 24. PMID: 31882225.

Progressive authorisation lifecycle



Facilitate an "end-to-end" process with de-risked, staggered development costs and better predictability: **Medicines Adaptive Pathways**

List of policy documents, guidelines, and academic publications/ per HTA agency

HTA agency	Policy papers and guidelines	Academic publications	Number of interview participants and transcript reference
TLV	Guide for companies when applying for subsidies and pricing for pharmaceutical products [21] General guidelines for economic evaluations from the Pharmaceutical Benefits Board (LFNAR 2003:2) [28] The Swedish Pharmaceutical Reimbursement System [33]	-	1 participant Transcript reference: a
NICE	Guide to the methods of technology appraisal 2013 [22] NICE DSU technical support document 17: The use of observational data to inform the estimates of treatment effectiveness in technology appraisal: Methods for comparative individual patient data [26]	Evidence requirements for reimbursements of pharmaceuticals across Europe [12] Methodological challenges in evaluating the value of registries [18] Evidence informed decision making: The use of "colloquial evidence" at NICE [17] How RWD can compensate for scarce evidence in HTA [19] How to improve the quality of evidence when new treatments are funded conditional on collecting evidence of effectiveness and safety [20]	3 participants Transcript reference: b
IQWiG	Allgemeine Methoden version 4.2 [23]	-	1 participant Transcript reference: c
HAS	General method for assessing health technologies [24] Choices in methods for economic evaluation [29] Les etudes post-inscription sur les technologies de santé (médicaments, dispositifs médicaux et actes) [31]	-	2 participants Transcript reference: d
AIFA	-	Evidence requirements for reimbursements of pharmaceuticals across Europe [12] New perspectives and new challenges in clinical trial regulation in Italy [13] Feasibility and challenges of independent research on drugs: The Italian Medicines Agency (AIFA) experience [15] The Italian post-marketing registries [14] The nationwide Osmed Health-Db database: A tool to support healthcare decision-making and real-world evidence generation [16]	2 participants Transcript reference: e
ZIN	Beoordeling stand van de wetenschap en praktijk [25] Richtlijn voor het uitvoeren van economische evaluaties in de gezondheidszorg [30] Leiderad voor Uitkomstenonderzoek [27] Procedure voorwaardelijke toelating geneeskundige zorg 2015 [32]	Evidence requirements for reimbursements of pharmaceuticals across Europe [12]	2 participants Transcript reference: f

AIFA, Italian Medicines Agency; HAS, High Authority for Health; HTA, health technology assessment; IQWiG, Institute for Quality and Efficiency in Healthcare; NICE, National Institute for Health and Care Excellence; RWD, real-world data; TLV, Dental and Pharmaceutical Benefits Agency; ZIN, National Healthcare Institute.

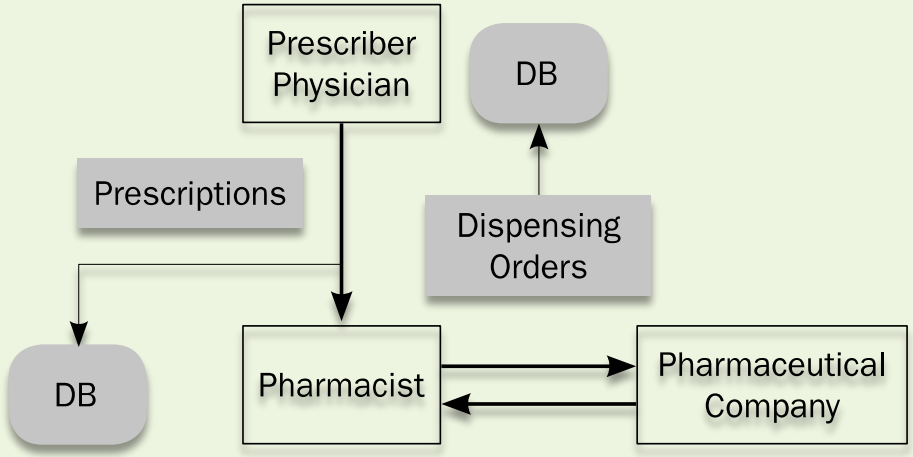
Makady A, Ham RT, de Boer A, Hillege H, Klungel O, Goettsch W; GetReal Workpackage 1. Policies for Use of Real-World Data in Health Technology Assessment (HTA): A Comparative Study of Six HTA Agencies. Value Health. 2017 Apr;20(4):520-532

AIFA drug – based registries[1,2]

1. Longitudinal administrative data collection to verify the Appropriateness (avoid the off-label use)
2. Apply Managed Entry Agreements (risk-sharing scheme between AIFA & industry) at patient/population level
3. Govern the public drug expenditure

Italian regulation

- 2012/135 Law on ITS in NHS
- 2015/125 Law on MEA assessment
- 2017/2015 Law on IMPs and RWD and patient journey
- 2018 Ministry of Health document on pharmaceutical governance
- 2019 AIFA triennial plan



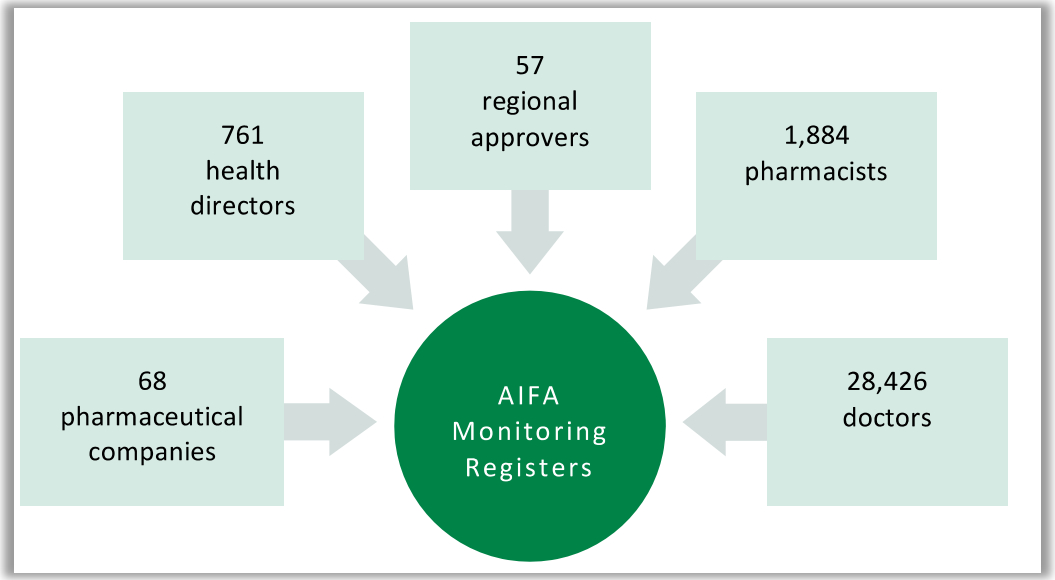
1. Xoxi E et al., The Italian post-marketing registries 2012 Pharmaceutical Programming Vol. 5 N° 1&2
2. Montilla S, Xoxi E et al., International Journal of Technology Assessment in Health Care, 31:4 (2015), 210–213

Data

	N.			Δ %
	2018	2019	2020	20-19
Registers*	141	166	166	0.0
web-based TPs*	12	14	13	-7.1
Treatments	2,177,819	2,730,119	3,209,838	17.6
Patients	1,858,603	2,288,704	2,655,909	16.0

*Registers intended as single active IT entities are counted (therefore all previous and inactive versions of a Register that have occurred over time are excluded from the calculation)

Age class	Men		Wome	
	No. of patients	Inc. %	No. of patients	Inc. %
<40	22,779	4.2	19,443	4.0
40-49	47,795	8.9	38,506	8.0
50-59	100,033	18.6	79,575	16.4
60-69	132,087	24.6	111,818	23.1
70-79	160,186	29.9	147,323	30.4
≥80	73,709	13.7	87,301	18.0
Total	536,589	100.0	483,966	100.0



Examples

Figure 4.1.2. Treatments initiated with anti-PCSK9 medicines in December 2
trend)

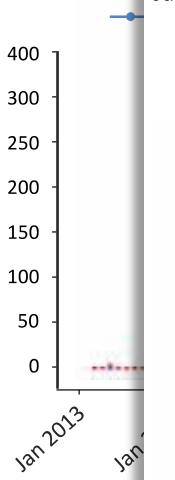


Figure 4.1.3. Monthly trend of treatments initiated wi
January 2013 to 2020 in Italy

*The t

Figure 4.1.6. Trend of treatments initiated

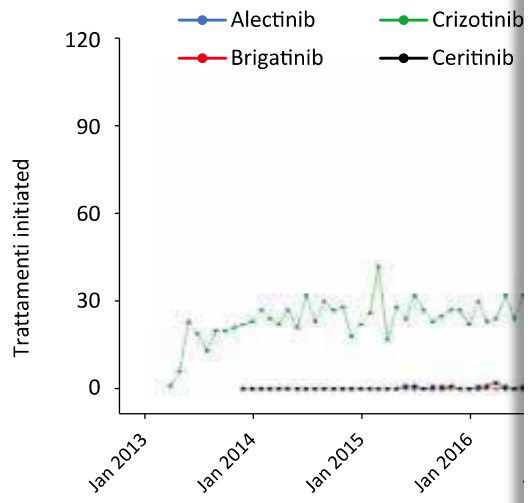
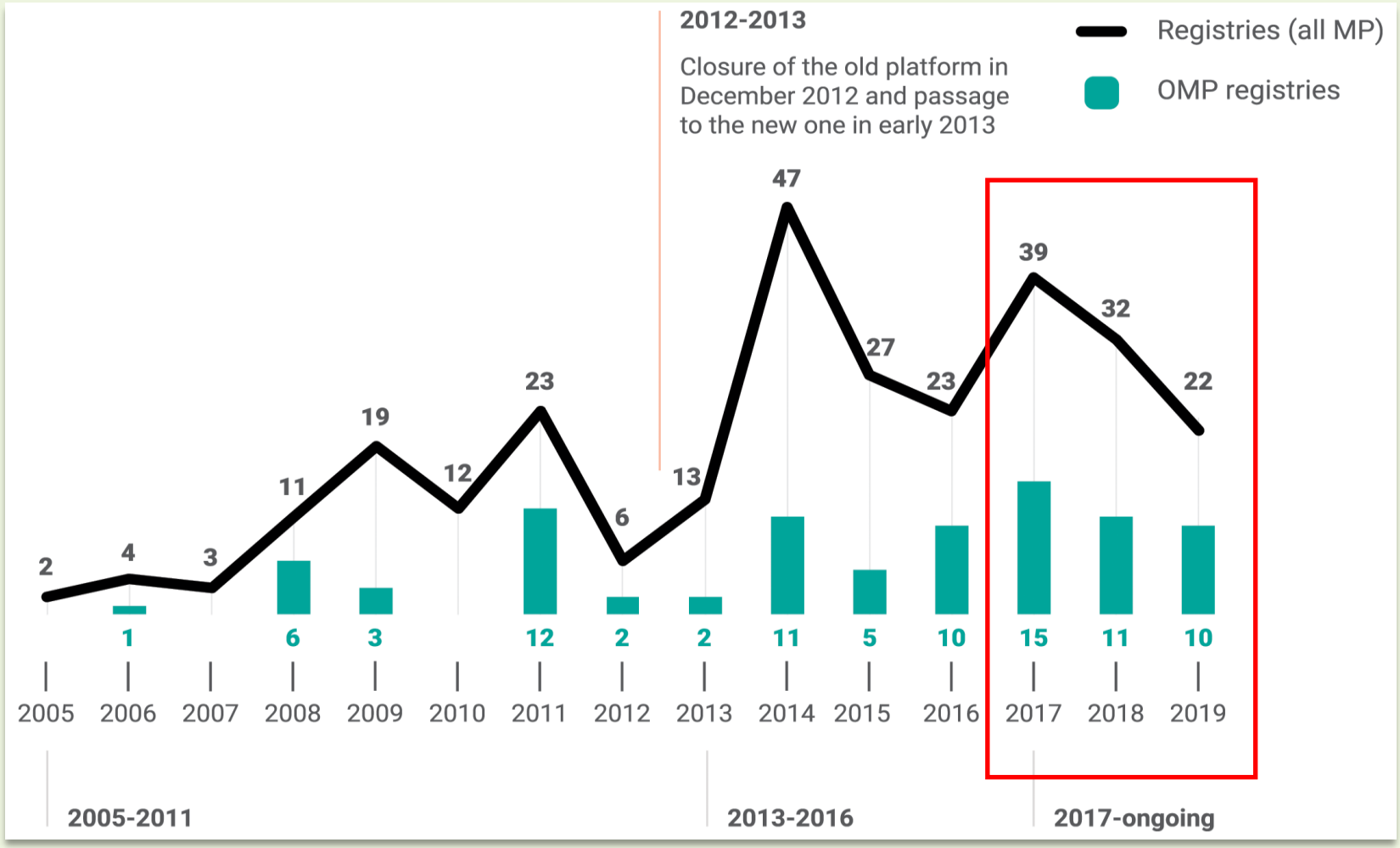


Table 4.1.16. Baseline characteristics for adult patients with diffuse large B cell lymphoma

Characteristics at baseline	Axicabtagen ciloleucel N (%)	Tisagenle-cleuce N. (%)	Total N. (%)
Eligible patients	67 (100.0)	97 (100.0)	164 (100.0)
Gender*			
F	24 (36.4)	38 (42.2)	62 (39.7)
M	42 (63.6)	52 (57.8)	94 (60.3)
Age at register entry (median years - range)	49.4 (19,8-70,2)	57.5 (29,7-70,6)	54.8 (19,8-70,6)
Time since first diagnosis (median years - IQR)	1.2 (0,8-2,2)	1.6 (1,0-3,8)	1.4 (0,9-3,1)
IPI score			
0	4 (6.0)	8 (8.2)	12 (7.3)
1	15 (22.4)	15 (15.5)	30 (18.3)
2	21 (31.3)	30 (30.9)	51 (31.1)
3	17 (25.4)	30 (30.9)	47 (28.7)
4	10 (14.9)	11 (11.3)	21 (12.8)
5	0 (0.0)	3 (3.1)	3 (1.8)
Days from insertion to infusion (median days - range)	**61.5 (40,0-120,0)**	64 (33-131)	63 (33-131)
Number of previously administered systemic lines of therapy	(including rituximab and anthracyclines)		
2	38 (56.7)	57 (58.8)	95 (57.9)
3	19 (28.4)	29 (29.9)	48 (29.3)
≥4	10 (14.9)	11 (11.3)	21 (12.8)
Patient candidate for ASCT			
No	66 (98.5)	95 (97.9)	161 (98.2)
Yes	1 (1.5)	2 (2.1)	3 (1.8)
Performance status (ECOG)			
0	53 (79.1)	67 (69.1)	120 (73.2)
1	14 (20.9)	30 (30.9)	44 (26.8)
Previous anti-CD19 therapy			
No	66 (98.5)	97 (100.0)	163 (99.4)
Yes	1 (1.5)	0 (0.0)	1 (0.6)
Relapse after ASCT			
No	44 (65.7)	67 (69.1)	111 (67.7)
Yes	23 (34.3)	30 (30.9)	53 (32.3)
Patient not eligible for ASCT			
Other (including transplant already performed)	13 (19.4)	21 (21.6)	34 (20.7)
Age/Comorbidity	0 (0.0)	1 (1.0)	1 (0.6)
Age/Comorbidity+failure to respond to rescue therapy	1 (1.5)	4 (4.1)	5 (3.0)
Failure to respond to rescue therapy	53 (79.1)	71 (73.2)	124 (75.6)
Stadium (Lugano mod. Ann Arbor criteria)			
I	0 (0)	1 (1.0)	1 (0.6)
I E	0 (0)	1 (1.0)	1 (0.6)
II	5 (7.5)	11 (11.3)	16 (9.8)
II bulky	8 (11.9)	4 (4.1)	12 (7.3)
II E	6 (9.0)	1 (1.0)	7 (4.3)
III	9 (13.4)	20 (20.6)	29 (17.7)
IV	39 (58.2)	59 (60.8)	98 (59.8)

AIFA registries



June 2022

- 160 Only **Appropriateness** registries
- 14 Appropriateness with **Financial-based**
- 4 Appropriateness with **Payment AT Result**
- 10 Appropriateness with **Payment by result**

Xoxi E, Facey KM, Cicchetti A. The Evolution of AIFA Registries to Support Managed Entry Agreements for Orphan Medicinal Products in Italy. Front Pharmacol. 2021 Aug 10;12:699466. doi: 10.3389/fphar.2021.699466
AIFA web page: https://www.aifa.gov.it/web/guest/registri-e-piani-terapeutici1 Accessed June 23 2022

Criteria on Innovativeness' recognition (therapeutic indication-based)

https://www.aifa.gov.it/en/web/guest/farmaci-innovativi
AIFA Innovativeness' recognition – 519/2017 Deliberation
Galeone C, et al. BMJ Open 2021;11:e041259. doi:10.1136/bmjopen-2020-041259

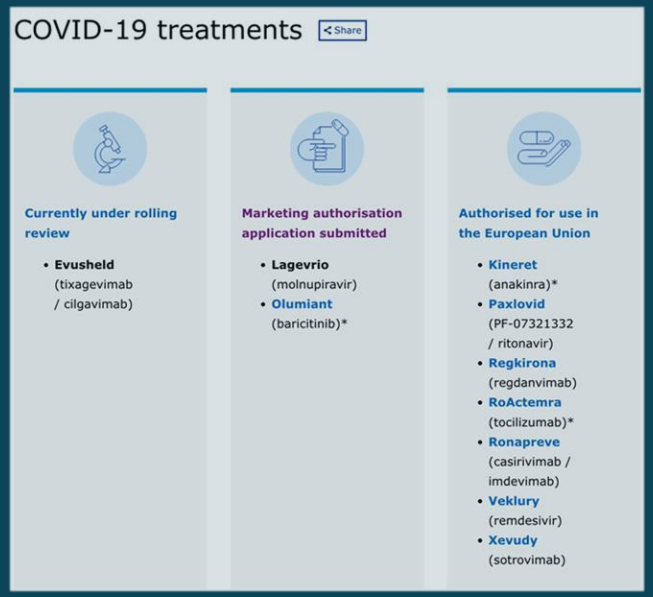
AIFA Innovative appraisals (04/2017 – 01/2020)

	Fully innovative		Conditionally innovative		Non-innovative†		*p*-value*
	n = 37		*n* = 29		*n* = 43		
Oncological drug	24	64.9	20	69.0	23	53.5	0.363
Orphan drug	16	43.2	11	37.9	14	32.6	0.616
Oncological and orphan drug	10	27.0	6	20.7	8	18.6	0.645
Non-oncological and non-orphan drug	7	18.9	4	13.8	14	32.6	0.155
Therapeutic need							
Maximum	5	13.5	4	13.8	4	9.3	0.081
Important	17	45.9	7	24.1	12	27.9	
Moderate	15	40.5	18	62.1	22	51.2	
Poor	0	0.0	0	0.0	5	11.6	
Absent	0	0.0	0	0.0	0	0.0	
Added therapeutic value							
Maximum	1	2.7	0	0.0	0	0.0	<0.001
Important	31	83.8	0	0.0	1	2.6	
Moderate	5	13.5	29	100.0	5	13.2	
Poor	0	0.0	0	0.0	29	76.3	
Absent	0	0.0	0	0.0	3	7.9	
Quality of clinical evidence							
High	10	27.0	3	10.3	5	11.6	0.451
Moderate	19	51.4	18	62.1	24	55.8	
Low	7	18.9	6	20.7	9	20.9	
Very low	1	2.7	2	6.9	5	11.6	

*Data were summarized as numbers (n) and frequencies (%). *Chi-square test, when the conditions were respected, or Fisher's exact test was applied to evaluate the association between categorical variables. †For five observations the added therapeutic value was "Untestable" and therefore classified as NA.*

Fortinguerra F, Perna S, Marini R, Dell'Utri A, Trapanese M, Trotta F; Scientific & Technical Committee (Commissione Tecnico-Scientifica, CTS) of Italian Medicines Agency-AIFA. The Assessment of the Innovativeness of a New Medicine in Italy. Front Med (Lausanne). 2021 Dec 8;8:793640

AIFA registries & COVID-19



- Oral Antiviral: molnupiravir - PF 07321332 ritonavir
- Remdesivir
- Monoclonal antibodies: bamlanivimab e etesevimab/ casirivimab e imdevimab/ sotrovimab

EMA's governance during COVID-19 pandemic webpage: https://www.ema.europa.eu/en/human-regulatory/overview/public-health-threats/coronavirus-disease-covid-19/emas-governance-during-covid-19-pandemic Access 8 March 2022

viruses

MDPI

Article

Mortality in SARS-CoV-2 Hospitalized Patients Treated with Remdesivir: A Nationwide, Registry-Based Study in Italy

Pierluigi Russo [1,*], Evelina Tacconelli [2], Pier Paolo Olimpieri [1], Simone Celant [1], Antonietta Colatrella [1], Luca Tomassini [1] and Giorgio Palù [1,*]

Crude 15-day and 29-day mortality were 7.1% (95% CI, 6.7–7.5%) and 11.7% (95% CI, 11.2–12.2%), respectively. Being treated within two days of admission reduced the risk of death by about 40% (HR 1.4, 95% CI, 1.2–1.6). Results from the largest cohort of remdesivir-treated patients suggests that mortality in SARS-CoV-2 hospitalized patients is substantially influenced by the days between SARS-CoV-2 diagnosis and drug prescription.

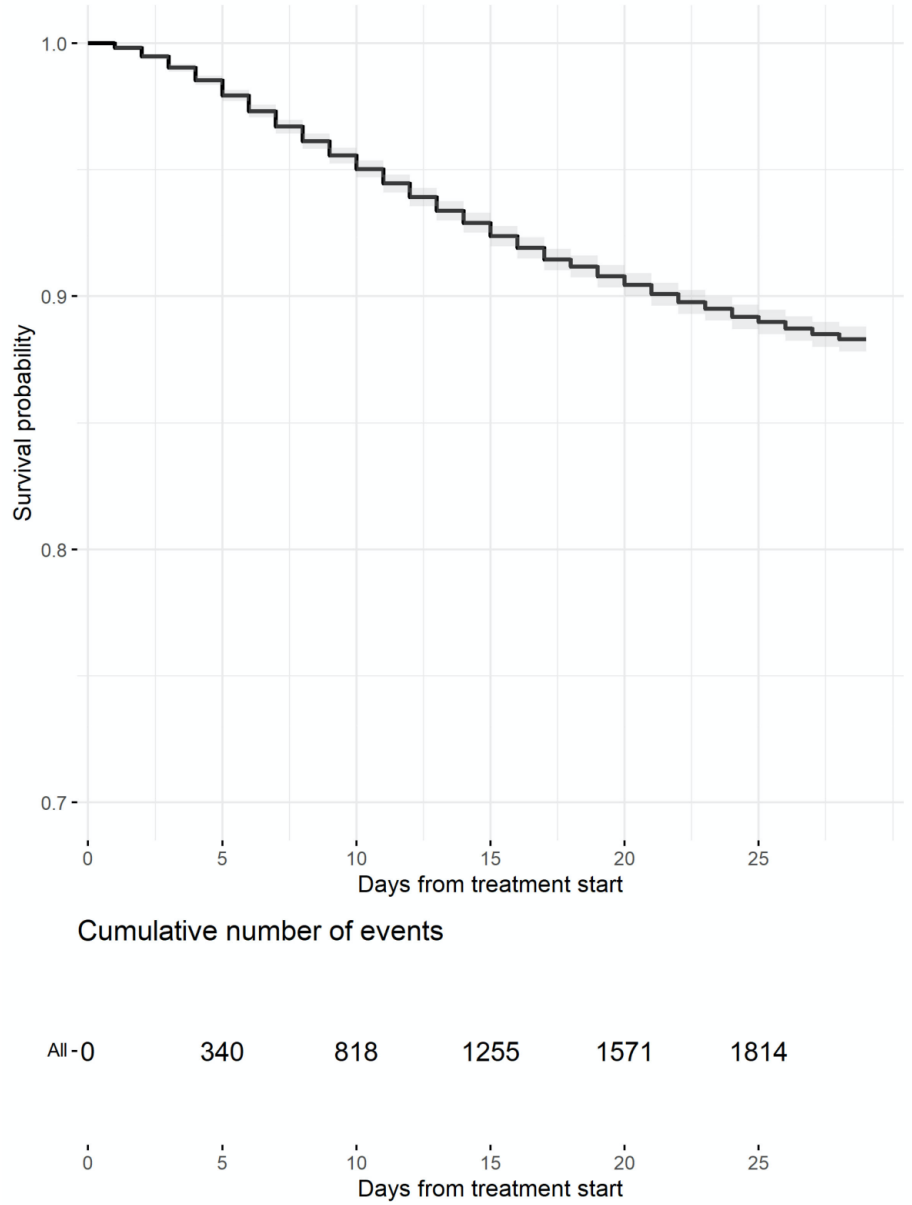


Figure 1. Kaplan–Meier Estimates of survival probability up to day 29 (events are censored at day 29). The gray area represents the 95% confidence band. Table of cumulative events (deaths) is reported below the curve.

COVID-19 Monoclonal antibodies monitoring within AIFA registries

AGENZIA ITALIANA DEL FARMACO

Report n. 53

Monitoraggio Anticorpi Monoclonali per Covid-19

Ufficio Registri di Monitoraggio AIFA

Dati relativi alla settimana 02 – 08 giugno 2022

(estrazione dati 09 giugno 2022)

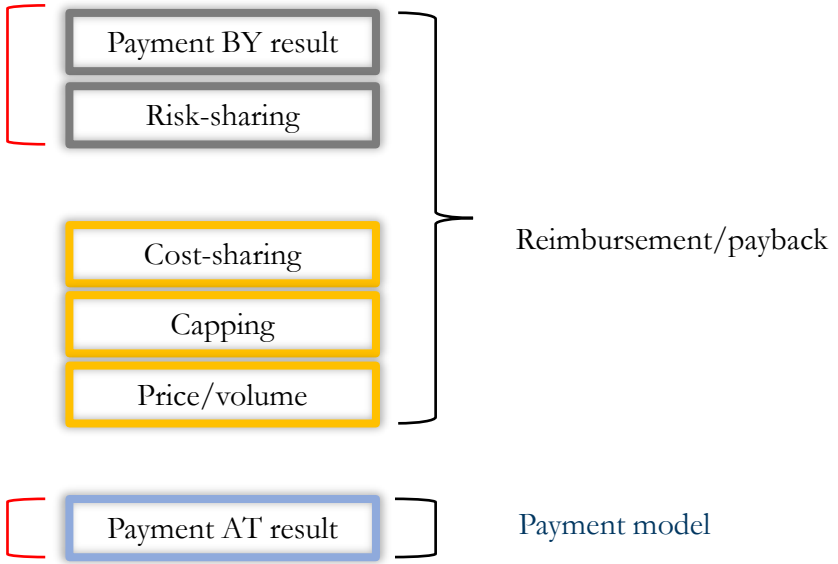
Registro AIFA anticorpi monoclonali per Covid-19
Dettagli monitoraggio

Principio attivo	Autorizzazione	Giorni di monitoraggio
bamlanivimab (Eli-Lilly)	Determina AIFA n.274 nella GU n.58 del 09.03.2021	59
	Determina AIFA di revoca n.557 nella GU n. 108 del 07.05.2021	
bamlanivimab e etesevimab (Eli-Lilly)	Determina AIFA n.318 nella GU n.66 del 17.03.2021	448
	Determina AIFA n.697 nella GU n.142 del 16.06.2021	
casirivimab e imdevimab (Regeneron/Roche)	Determina AIFA n.340 nella GU n.71 del 23.03.2021	442
	Determina AIFA n.912 nella GU n.187 del 06.08.2021	
	Determina AIFA n.978 nella GU n. 209 del 01.09.2021	
	Determina AIFA n. 1414 nella GU n. 282 del 26.11.2021 (L. 648/1996)	
casirivimab e imdevimab (Ronapreve/Roche)	Determina AIFA n. 155 nella GU n. 282 del 26.11.2021	194
sotrovimab (GlaxoSmithKline)	Determina AIFA n.911 nella GU n.187 del 06.08.2021	306
tixagevimab e cilgavimab (AstraZeneca)	Determina AIFA n.87 nella GU n.42 del 19.02.2022	109
	Determina AIFA n.53 nella GU n.88 del 14-04-2022	

Totale pazienti inseriti con almeno 1 DF*: **61.854**
Totale strutture prescriventi: 284
Totale regioni prescriventi: 21

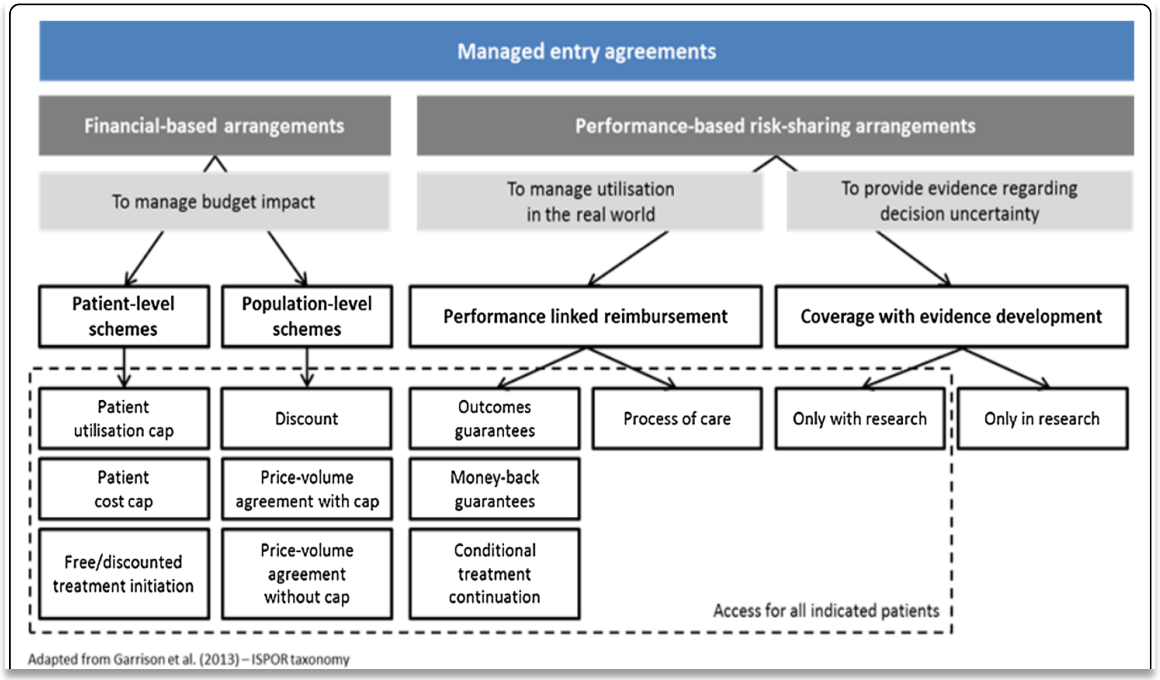
AIFA web page: https://www.aifa.gov.it/uso-degli-anticorpi-monoclonali Accessed June 23 2022

Managed Entry Agreements' implementation



Based on outcome

All registries capture clinical data



Morel T, Arickx F, Befrits G, Siviero P, van der Meijden C, Xoxi E and Simoens S. *Orphanet Journal of Rare Diseases* 2013, 8:198

MEAs' impact on pharmaceutical expenditure

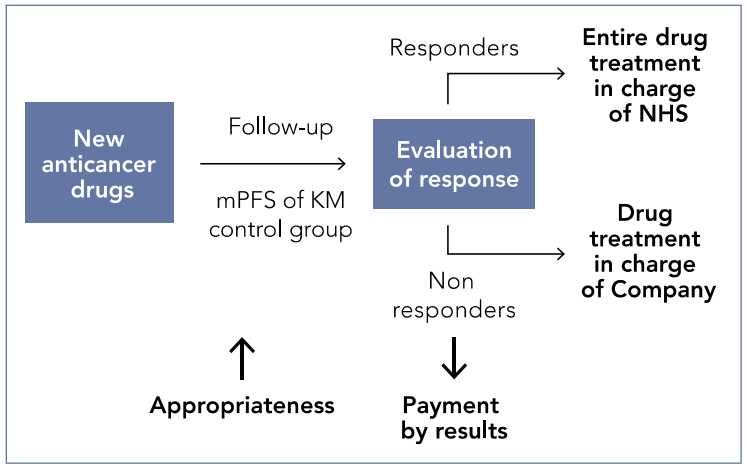
Impact of MEAs (implemented in the AIFA registries) on NHS pharmaceutical expenditure = 0.5%

	2020
Rimborsi MEA gestiti dai Registri	114.835.024
Rimborsi MEA gestiti tramite flussi di monitoraggio	228.820.009
Rimborsi MEA gestiti tramite flussi di monitoraggio convenzionata	43.251.664
Rimborsi MEA gestiti tramite flussi di monitoraggio acquisti diretti	185.568.345
Totale rimborsi	**343.655.033**
Impatto MEA gestiti dai Registri sulla spesa SSN (%)	0,5
Impatto MEA gestiti dai Registri sulla spesa acquisti diretti (%)	0,8
Impatto MEA gestiti tramite flussi di monitoraggio sulla spesa SSN (%)	1,0
Impatto MEA gestiti tramite flussi di monitoraggio su spesa convenzionata (%)	0,4
Impatto MEA gestiti tramite flussi di monitoraggio su acquisti diretti (%)	1,4
Impatto totale MEA (gestiti tramite Registri e tramite flussi di monitoraggio) sulla spesa SSN (%)	1,5
Impatto totale MEA (gestiti tramite Registri e tramite flussi di monitoraggio) su acquisti diretti (%)*	2,2
Inc. % su sfondamento	11,1

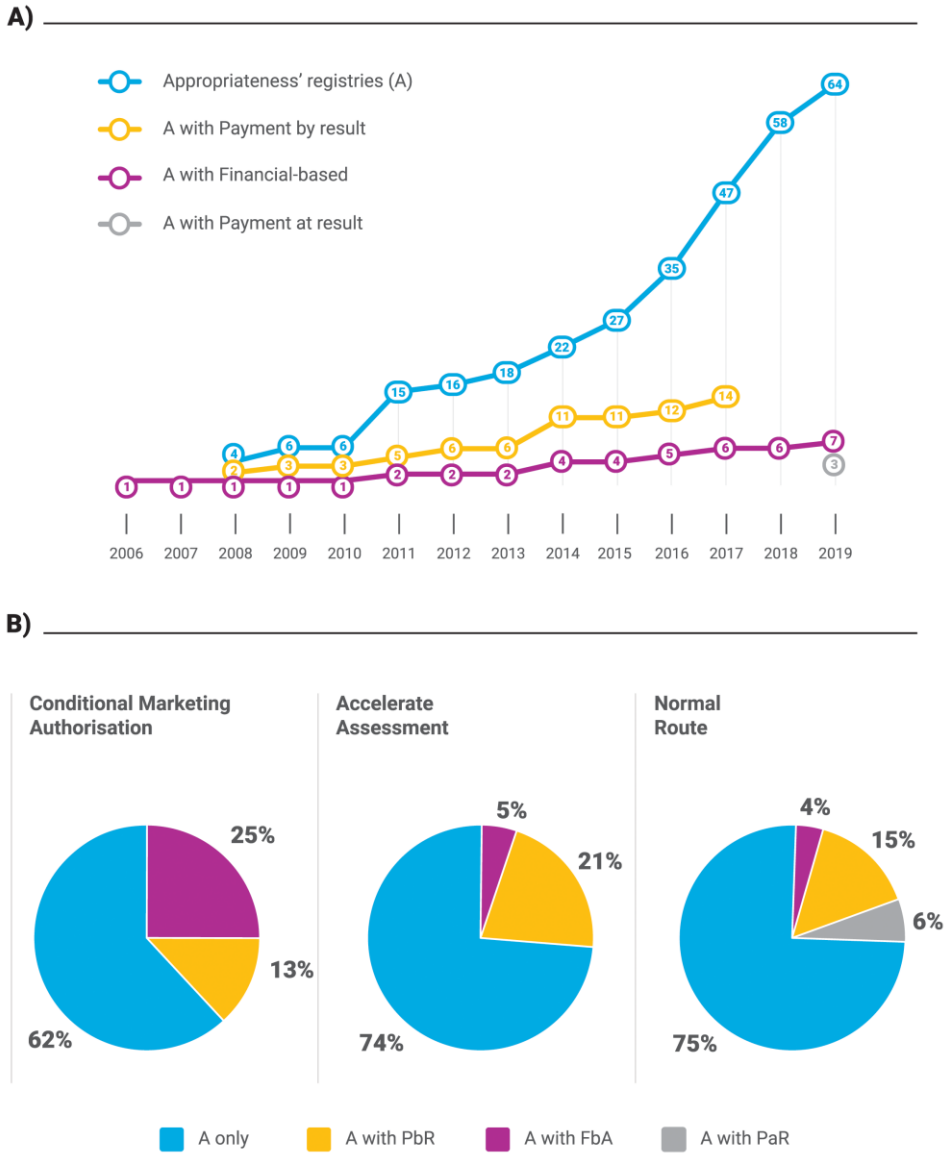
*Esclusi i rimborsi che vengono erogati in assistenza convenzionata

2021 IT National Drug Utilisation (OsMed)

AIFA registries and entry agreements: from Payment BY result to a new trend

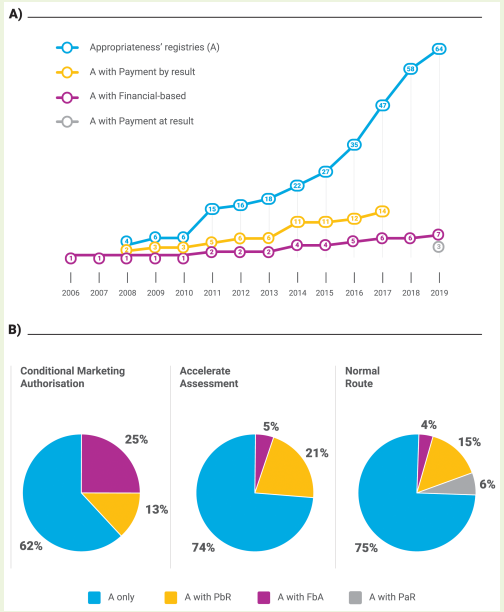


mPFS of KM: tempo di follow-up calcolato sulla mediana della PFS della curva di Kaplan-Meier nel gruppo di controllo



Martini et al., 2015 Un nuovo modello di governance per il market access dei nuovi farmaci in oncologia; Xoxi E, Facey KM, Cicchetti A. The Evolution of AIFA Registries to Support Managed Entry Agreements for Orphan Medicinal Products in Italy. Front Pharmacol. 2021 Aug 10;12:699466. doi: 10.3389/fphar.2021.699466

New trend on MEAs: payment AT result with instalment



Xoxi E, Facey KM, Cicchetti A. The Evolution of AIFA Registries to Support Managed Entry Agreements for Orphan Medicinal Products in Italy. Front Pharmacol. 2021 Aug 10;12:699466

Principio attivo	Tisagenlecleucel
ATC V livello	L01XX
Specialità	Kymriah®
Confezione	1 sacca
Prezzo al pubblico	€528.128
Prezzo ex-factory al lordo delle riduzioni di legge, IVA esclusa	€320.000
Dose raccomandata in RCP	1 infusione *una tantum*
Numero confezioni per la durata del trattamento	1 confezione
Costo per la durata del trattamento per paziente a carico del SSN, IVA esclusa (€)	€320.000
Condizioni negoziali	Sconto confidenziale alle strutture del SSN per l'indicazione DLBCL e meccanismo di pagamento condizionato (Payment at results) all'infusione, a 6 e a 12 mesi per entrambe le indicazioni.

Figura 3. Stima di Kaplan Meier della sopravvivenza per l'indicazione DLBCL (dati di registro AIFA)

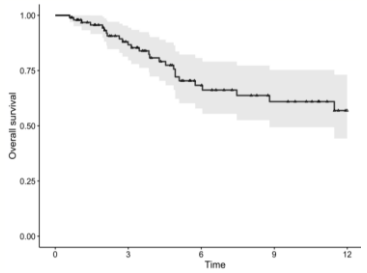
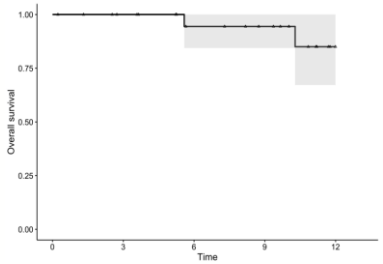


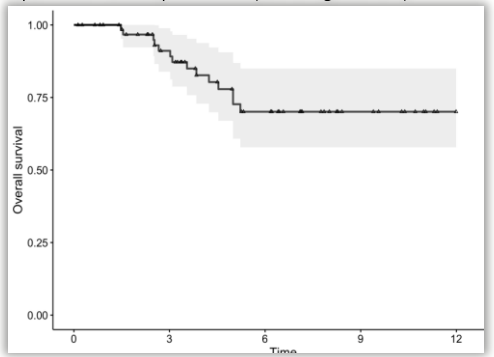
Figura 4. Stima di Kaplan Meier della sopravvivenza per l'indicazione LLA (dati di registro AIFA)



Median survival for DLBCL was not reached, while the 6-month survival probability was 68.3% with a 95% confidence interval (CI) of 57.8% -80.7%. Also for the ALL indication, the median survival was not reached and the 6-month survival probability was estimated to be 94.4% with a 95% CI: 84.4% - 100.0%.

Principio attivo	Axicabtagene ciloleucel
ATC V livello	L01XX
Specialità	Yescarta™
Confezione	1 sacca
Prezzo al pubblico	€539.680,80
Prezzo ex-factory al lordo delle riduzioni di legge, IVA esclusa	€327.000
Dose raccomandata in RCP	1 infusione *una tantum*
Numero confezioni per la durata del trattamento	1 confezione
Costo per la durata del trattamento per paziente a carico del SSN, IVA esclusa (€)	€327.000
Condizioni negoziali	Sconto confidenziale alle strutture del SSN e meccanismo di pagamento condizionato (Payment at results) a 180, 270 e 365 giorni

Figura 3. Stima di Kaplan Meier della sopravvivenza (dati di registro AIFA)



The six-month survival probability, equal to 70.1% with a 95% confidence interval (CI) of 57.8% - 84.9%, was estimated on the basis of the survival curve sec. KM which for day 180 returns the values reported in the report (with relative 95% confidence intervals). The short median follow-up and the limited number of patients account for the breadth of the confidence interval and the still preliminary nature of this analysis.

AIFA Technical-scientific reports for Kymriah (20/09/2021) e Yescarta (28/07/2021)

Other Technical docs incoming:

In this section, the in-depth information relating to the data collected through the registry will be published when requested by the AIFA Commissions, or provided for as a result of price renegotiation and / or reimbursement procedures.

Registri di monitoraggio

In questa sezione saranno pubblicati gli approfondimenti relativi ai dati raccolti attraverso il registro di monitoraggio di Libmeldy® nel momento in cui saranno richiesti dalle Commissioni AIFA, o previsti ad esito di procedure di rinegoziazione del prezzo e/o della rimborsabilità.

AGENZIA ITALIANA DEL FARMACO

REPORT TECNICO

Zolgensma®
(onasemnogene abeparvovec)

Agenzia Italiana del Farmaco
12 luglio 2021

AGENZIA ITALIANA DEL FARMACO

REPORT TECNICO

Libmeldy®
(atidarsagene autotemcel)

Agenzia Italiana del Farmaco
20 Maggio 2022

Does Italy need a new MEA Value-based MEA pathway?

frontiers | Frontiers in Medical Technology

POLICY AND PRACTICE REVIEWS
published: 16 June 2022
doi: 10.3389/fmedt.2022.888404

A Proposal for Value-Based Managed Entry Agreements in an Environment of Technological Change and Economic Challenge for Publicly Funded Healthcare Systems

Entela Xoxi[1], Filippo Rumi[1], Panos Kanavos[2], Hans-Peter Dauben[3], Iñaki Gutierrez-Ibarluzea[4], Olivier Wong[5], Guido Rasi[6] and Americo Cicchetti[1]*

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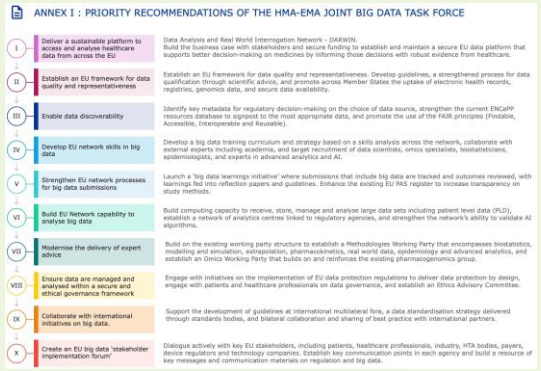
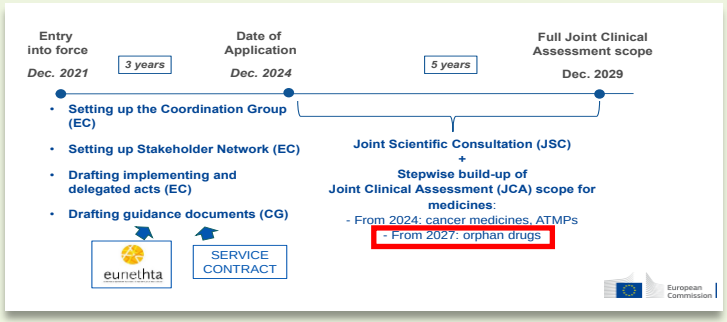
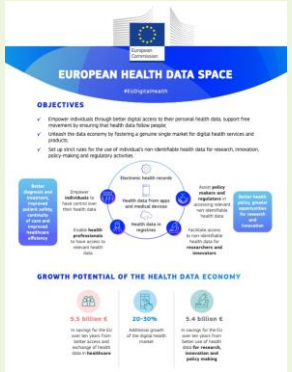
- Data quality
- Transparency & Return on Scientific Evidence
- Administrative vs observational studies → (studies)
- Individual- vs. population-level → (epidemiological purposes)
- Avoid duplication of data collection → (interoperability)
- Digital health, big data, AI (unavoidable) → (technology)

Challenges







- DARWIN EU project
- Internationally regulatory initiatives: ATMP (cluster, genome editing) & Orphans (cluster)
- Drug development (see PRIME figures)
- The complexity of innovation: therapeutic agnostic (platform trials), ATMP, platform technologies (mRNA)
- New HTA EU Regulation: joint scientific consultations (EUnetHTA 2021)
- Revision of EU Regulation on Orphans & paediatrics
- The proposal of EC on European Health Data Space

Thank you

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