Horizon Europe Project Ascertain: Supporting a Sustainable and Transparent Legal EU HTA Framework, Accessibility of Innovative Technologies and Health Equity

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ASCERTAIN aims to

1. Improve affordability and accessibility of innovative medicines and medical devices
2. Enhance methods of pricing, cost-effectiveness, and reimbursement
3. Reward innovation
4. Increase transparency and accountability in methods and the decision-making process
5. Contribute to the sustainability of healthcare systems
6. Include varying perspectives of stakeholders such as patients, industry, policy-makers
7. Consider the variation in healthcare systems and regions across Europe

ASCERTAIN

The Consortium

Erasmus University Rotterdam

Gesundheit Österreich GmbH

UNIVERSITY OF OSLO

COMENIUS UNIVERSITY BRATISLAVA

MPe Myeloma Patients Europe

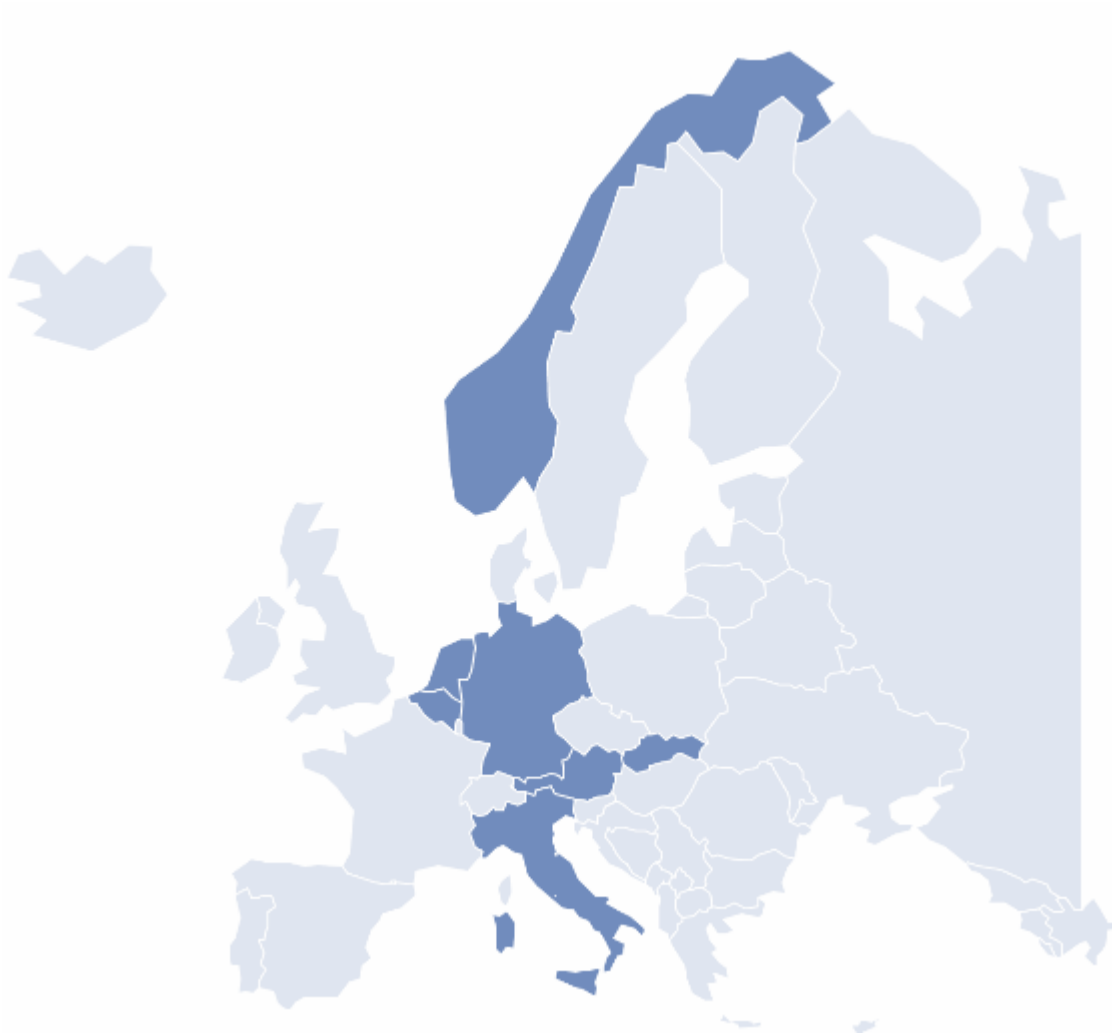
EHA EUROPEAN HEMATOLOGY ASSOCIATION

AIM Healthcare and social benefits for all

CHINO.IO

OptiMedis

NUROMEDIA



10 partners: 3 SMEs, 3 Universities, 4 Non-profit organisations. The consortium covers the value chains from industry to payers. Coordinated by Erasmus University Rotterdam.

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Website and video

- https://www.youtube.com/watch?v=sbPnTmX8Uxs
- www.access2meds.eu

Agenda

- EU Regulation on Health Technology Assessment (HTAR)
  - Mirjana Huić, MD, PhD
- ASCERTAIN project: Issues from legal and sustainability standpoints
  - Nicolas Xander, MSc
- Challenges in Supporting a Sustainable and Transparent Legal EU
  - Isabelle Durand- Zaleski, MD, PhD
- Interactive discussion

EU Regulation on Health Technology Assessment (HTAR)

Sustainable and transparent legal framework for EU cooperation on HTA

Mirjana Huić, MD, PhD

HTA/EBM Center, Zagreb

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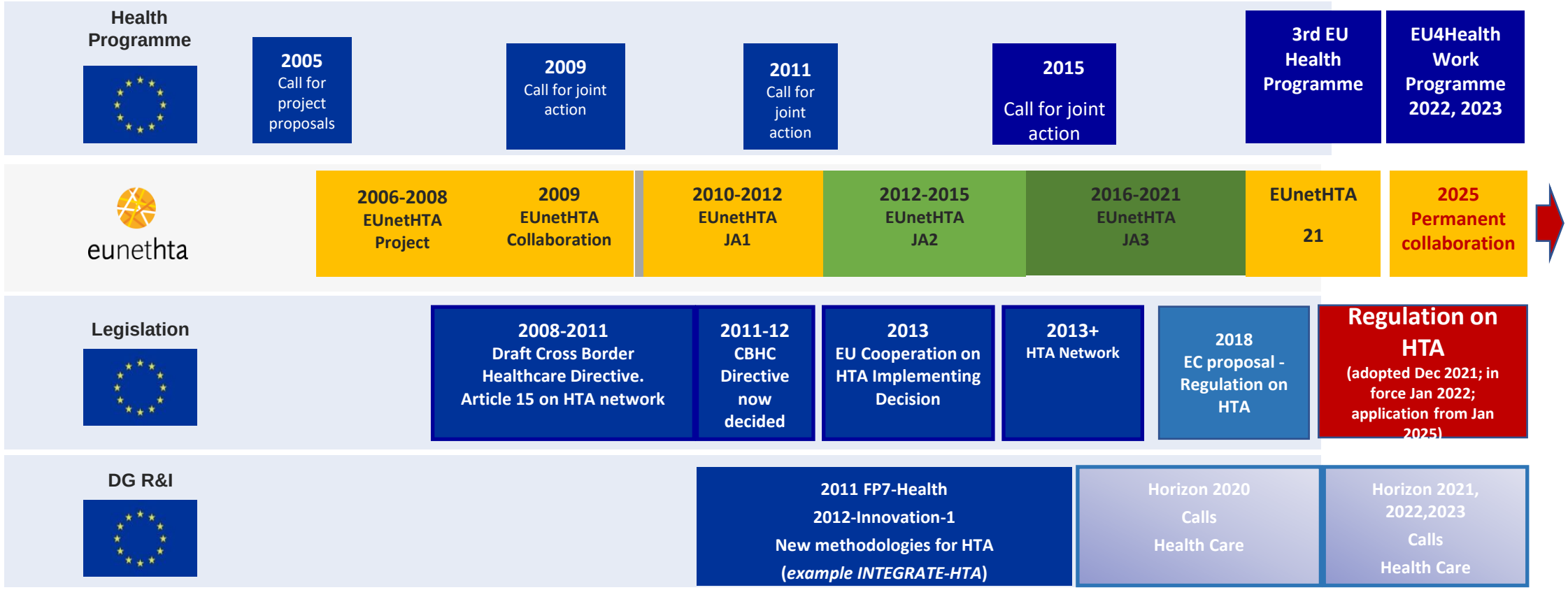
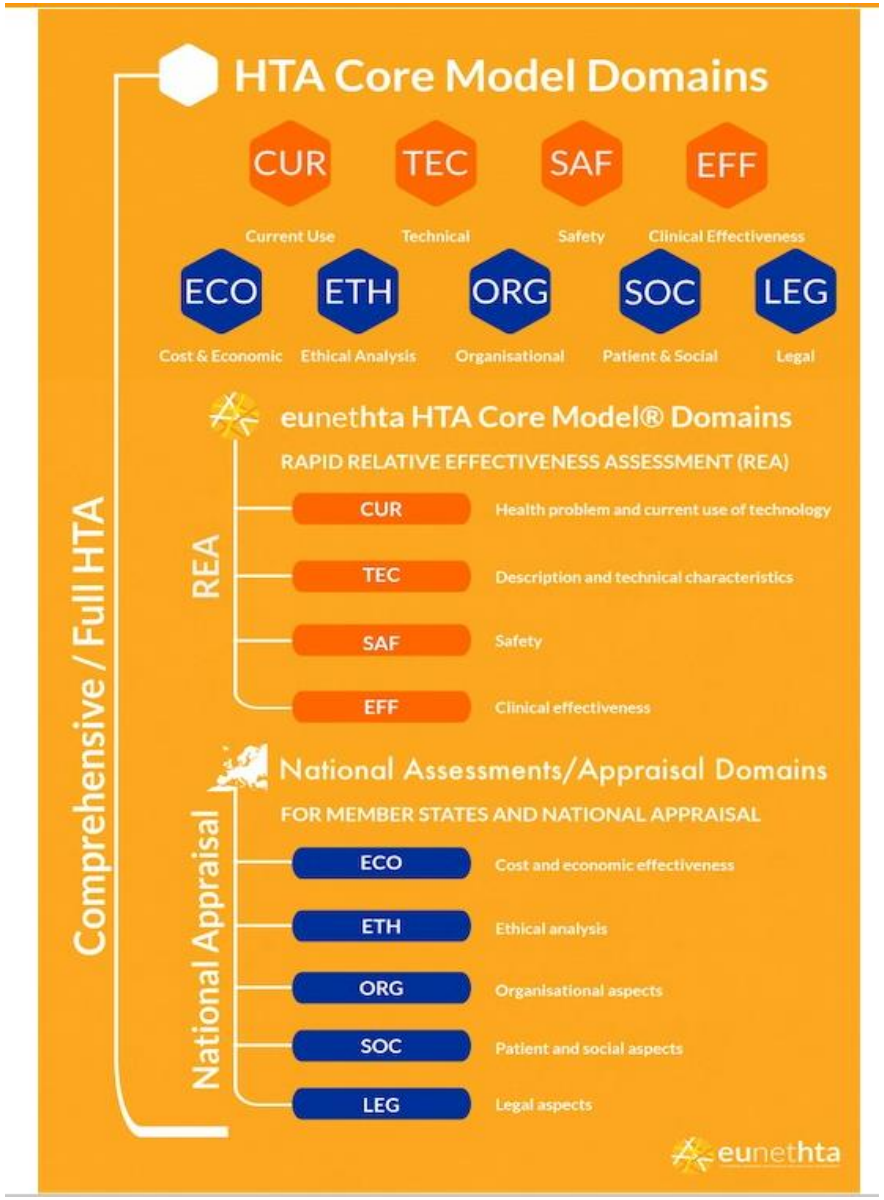
EU framework: The timeline of reaching a sustainable and permanent HTA cooperation in EU



From Full HTA Model to Joint clinical assessment (JCA)

The **Domains of the HTA Core Model®**



Full HTA
9 Domains

Rapid REA
4 Domains

EUnetHTA COVID-19 response
Rolling and **Rapid Collaborative Reviews**

EU HTA Regulation
Joint Clinical Assessment - 4 Domains

HTA Regulation - Key principles

Health problem and current use of technology (CUR); Description and technical characteristics of technology (TEC); **Relative clinical effectiveness** (EFF); **Relative safety** (SAF)

- **Joint work** on common **scientific**, **clinical** **aspects** of HTA
- **Driven** by **Member State HTA bodies**
- Ensure **high quality**, **timelines and transparency**
- Ensure **use of joint work** in **national HTA processes**
- **Member States** remain **responsible** for:
  - Drawing **conclusions on added value** for their health system
  - Taking **decisions on pricing & reimbursement**
- **Addresses stakeholders' engagement in joint work**
- **Progressive implementation**

- **Applied** in **all member states** from **12 January 2025**
- Regulation shall be **binding** in its **entirety** and **directly applicable** in **all Member States (MSs)**

- **Main goal** - to **improve access** to (**life-saving**) **innovative technologies**

- **Commitment** of **EU MSs** and **all stakeholders** is **key** for **appropriate implementation** of **HTAR** and **sustainable joint work** at **EU level**

Four areas of **joint work**:

Patient and Clinical expert involvement

- **Joint clinical assessment (JCA)**

- **Joint scientific consultations (JSC)**

- **Emerging health technologies**

Stakeholder Network and **Patient and Health professional organisations**

- **Voluntary cooperation**

Assumed as in **EUnetHTA** **Patient and Clinical expert** and **Patient and Health professional organisations**

Governance

MS Coordination group (CG: MDs configuration/Medicinal products configuration)

CG Subgroups (MSs experts)

Joint clinical assessments (JCA)	**Joint scientific consultations (JSC)**	Identification of **emerging health technologies (Horizon scanning)**	Development of **methodological and procedural guidance**
JCA reports and summary reports	JSC reports	Input for annual work programme	Guidance documents

Voluntary cooperation

Stakeholder Network

Patient associations
Consumer organisations
Non-governmental organisations
Health technology developers
Health professionals

EC Secretariat (Administrative, technical and IT support; facilitate the cooperation with EMA/ Medical Device Coordination Group…)

Preparatory phase: January 2022 – December 2024

- **Coordination Group**/HTACG and **Subgroups**

- **Stakeholder Network** (EC) - **44** organisations as **members**, **2** as **observers**

- Drafting **implementing** and **delegated acts** (**EC**):

JCA for medicinal products; JCA for medical devices; JSC for medicinal products; JSC for medical devices; Conflict of interest management; Cooperation by exchange of information with the **European Medicines Agency** (EMA): **by Q4 2024**

- Developing **IT platform (EC)**
- Drafting **guidance documents** (**CG**)

EUnetHTA21

Implementation phase: January 2025 - January 2030

Joint Clinical Assessments (**JCA**) on:

- **Medicines** (from **January 2025**: **oncology medicines** and **ATMP**; from **January 2028**: **+ orphan drugs**; from **January 2030**: **full scope**)

- **Medical devices** **(timeline** for **progressive implementation ?)** - **Selection of high-risk implantable MD classified as class IIb or III** and **IVDs class D** for which relevant expert panels have provided a scientific opinion in framework of clinical evaluation consultation procedure

One or more criteria: *unmet medical needs; 1st in class; potential impact on patients, public health or healthcare system; incorporation of software using artificial intelligence, machine learning technologies or algorithms; significant cross-border dimension; major Union-wide added value*

Joint Scientific Consultations (**JSC**) **HTA bodies only** *or* in **parallel with EMA**

Importance of Scoping process – Joint clinical assessment (JCA)

PICO for **JCA**

P: Patient population

I: Intervention

C: Comparisons

O:Outcomes

Example EUnetHTA21, PICO exercises, Consolidated PICO (3 pharmaceuticals)

Pharmaceutical	No of MSs	No of Consolidated PICO
Lutetium (177Lu) vipivotide tetraxetan (PLUVICTO)	**8**	**6 PICOs** (**P**: 2 in the full licenced population and 4 in subpopulations; **C**: **6** different comparators)
Tabelecleucel (Ebvallo)	**10**	**5 PICOs** (**P**: 1 in the full population and 4 in subpopulations, **C**: **5** different comparators)
Cipaglucosidase alfa (Pombiliti)	**10**	**9 PICOs** (**P**: 1 in the full population and 4 in subpopulations, **C**: **4** different comparators)

Example EUnetHTA21, Consolidated PICO (2 Medical devices)

JCAMD001 Assessment Report –
OPTILUME ® URETHRAL DRUG-COATED BALLOON

Description of PICO elements	PICO 1	PICO 2	PICO 3
Population	According to the intended use: Men aged ≥18 yr with bothersome urinary symptoms associated with recurrent anterior urethral strictures ≤3 cm in length.	The same as for PICO 1	The same as for PICO 1
Intervention	According to the intended use: * The Optilume urethral drug-coated balloon catheter is used as a dilation balloon for a single, tandem or diffuse anterior urethral stricture ≤3 cm in length or used as an adjunctive therapy with other dilation devices and/or procedures.	The same as for PICO 1	The same as for PICO 1
Comparator	Urethrotomy [a]	Dilation	Urethroplasty

JCAMD002 Assessment Report –
EVOKE SPINAL CORD STIMULATION SYSTEM

Description of PICO elements	PICO 1	PICO 2	PICO 3
Population[a]	According to the intended use: adult patients with chronic intractable pain of the trunk and/or limbs	Subpopulation: adult patients with chronic intractable back and leg pain (including radiating pain) associated with persistent spinal pain syndrome, with an insufficient effect from conventional pain management therapies	The same as for PICO 2
Intervention[b]	According to the intended use	The same as for PICO 1	The same as for PICO 1
Comparator	Latest generation of open-loop SCS systems (in addition to other pain management therapies)	The same as for PICO 1	Conventional nonsurgical pain management therapies (including pharmacotherapy with or without physiotherapy and/or psychotherapy, etc.)[c]

Thank you for listening!

New models within the ASCERTAIN project

Issues from legal and sustainability standpoints

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Nicolas Xander, MSc

Erasmus University Rotterdam

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ASCERTAIN: Development of Models

Focus:

Innovative health technologies (pharmaceuticals & med devices)

Aim:

Improvement of affordability and accessibility

Improvement of sustainability

ASCERTAIN: Development of Models

Models:


Pricing


Cost-effectiveness / value assessment / budget impact


Reimbursement / payment

Usability:


Application of models in tools supporting policy-making

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ASCERTAIN: Development of Models

Pricing model

Output: Price
(List price)

Use price output for

CE / value assessment model

Output: CE yes/no?
Sufficient value?
Budget impact?

Use CE/VA/BI output for

Reimbursement / payment scheme models

Supporting tools for policymaking
Use of dashboard

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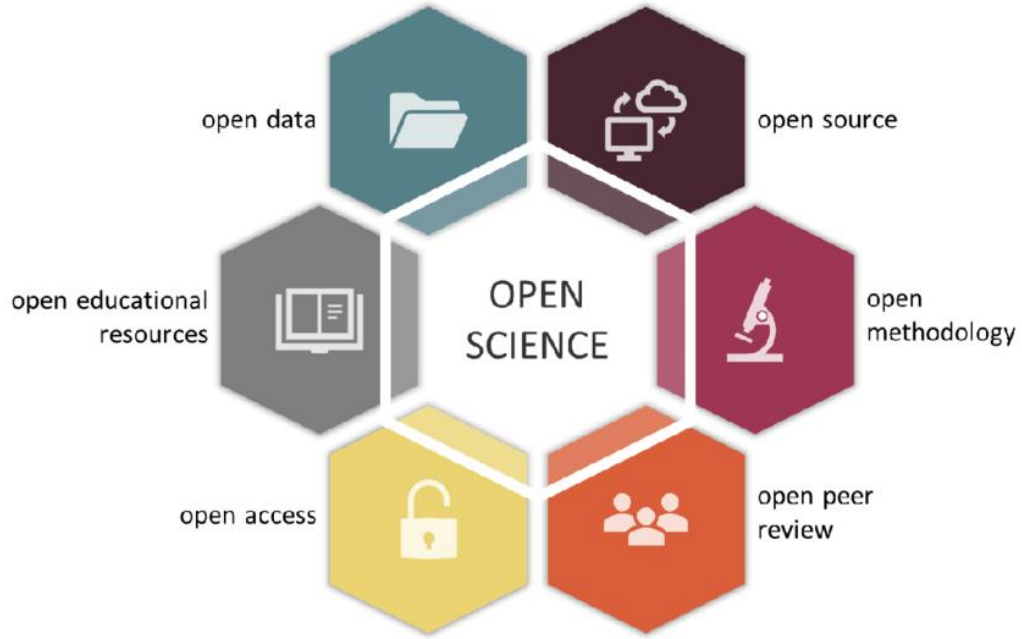
ASCERTAIN: Development of Models



Legal issues

Application in practice

Heterogeneity of policies re. pricing, HTA and reimbursement => *sovereignty of countries*

Assumption of existence of pricing/HTA policies might be misguided

Development of EU policies => *keep in check to keep ASCERTAIN models and tools compliant*

HTA Regulation => discrepancies in practice re. JCA?

Incompatibility with policymaking practice across countries

Application of tools dependent on willingness of policy-makers

ASCERTAIN tools do not resolve heterogeneity in practice:

No uniform approach possible, need to account for decision-making practices across countries

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Issues Regarding Sustainability

Aim:

Inclusion of environmental sustainability aspects in models and tools

Issues:

Which sustainability-related parameters to use (CO_2 consumption, drug wastage)?

How to quantify and measure (within ASCERTAIN)?

How to apply sustainability in the models – as a factor influencing the price? Cost factor in CE/VA? For reimbursement decision-making procedures?

Option: provide countries/decision makers with sustainability-related characteristics

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Supporting a Sustainable and Transparent Legal EU HTA Framework, Accessibility of Innovative Technologies and Health Equity

Isabelle Durand-Zaleski

European Hematology Association

ISPOR Europe,

Copenhagen November 2023

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Theoretical models

Prices = costs and in particular

R&D costs

Prices = value

Prices adjusted to buyers' GDP

"Our results suggest that the price of cancer drugs is independent of novelty.
Our results suggest that current pricing models are not rational but simply reflect what the market will bear."
JAMA Oncol. 2015;1(4):539-540. doi:10.1001/jamaoncol.2015.0373

Problems

Central planning typical of communist economies: productions that are useless

Market based economies, the problem here is the definition of value and value for whom.

Value vs competitors

Discriminating monopoly

Price and reimbursement

Adjustment variables for payers, and problems for patients

- Reimbursement **rates** (OOP or not, for most very severe diseases there is full coverage)
- Reimbursment **yes /no**= thresholds either of price relative to some measure of effectiveness or ICER in cost/QALY
- Or Reimbursement independent from price, based only upon medical benefit
- And negotiation on price

Not true of every country in the EU, limits access

All prices end up at the threshold value

Opaque and could limit access if prices are too low

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Changes introduced by the JCA

- Common metric for clinical effectiveness
- No judgment about added benefit (the comparators may differ between countries)
- Not binding for value-based purchasing

Value based pricing, risk sharing, performance based agreements

- Not too many successes to report
- Outcomes based agreements need:
  - Face validity of the outcomes selected
  - Automated data retrieval
  - To be undisputable in courts

NEJM Catalyst

- Value is a journey, not a destination
- What you measure is what you value
- Partnerships are key

Multiple sclerosis risk sharing scheme: a costly failure
BMJ 2010; 340

Health Economics

The simple economics of risk-sharing agreements between the NHS and the pharmaceutical industry

We argue here that risk-sharing agreements, although attractive due to the principle of paying by results, also entail risks. **Too many patients may be put under treatment. Prices are likely to be adjusted upward**, in anticipation of future risk-sharing agreements between the pharmaceutical company and the third-party payer.

Overall**, the welfare effects of risk-sharing agreements are ambiguous**, and caution is urged regarding their use.

The journey is the destination

Define an engagement process with all stakeholders

Define, agree and prioritize the expectations/ values among the following:

- Encourage innovation
- Cover R&D costs with RoI
- Limit budget impact
- Improve cost effectiveness
- Ensure appropriate drug use
- Ensure access
- Ensure sustainability

- Design a journey (process) that can be considered fair and transparent

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Polling Questions

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Navigate to this session in the meeting app to participate!

Open Question

What are the most important challenges for Joint Clinical Assessments at EU level?

Multiple-Choice Question

A fair price of a drug or device is set in order to:

- cover R&D and production costs
- represent the clinical value
- represent the societal value
- represent the clinical & societal value
- represent a good return on investment
- allow low-income countries to get access to the drug or device.

Open Question

Which factors /elements should be used to reflect environmental sustainability in pricing and/or cost-effectiveness models?

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Single-Choice Question

Which approach could form the basis of an adequate pricing model that captures the important factors for the access of patients to innovative health technologies?

- Cost-based / cost-plus pricing approach
- Value-based pricing approach
- A blend of both approaches
- A completely new approach
- There can be no viable approach