ASSOCIAZIONE ALESSANDRO LIBERATI
NETWORK ITALIANO COCHRANE

Sistemi sanitari e innovazione

Dalla ricerca (utile) alla sostenibilità dei cambiamenti

DALLA RICERCA ALLA SCELTA E INTRODUZIONE DELLE INNOVAZIONI: INIZIATIVE EUROPEE DI HTA

Luciana Ballini

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Sommario

- Determinanti di efficacia dell'HTA
- Iniziative per una cooperazione europea in HTA
  - Normativa per HTA
  - Collaborazione scientifica in HTA
- HTA e ricerca
- Conclusioni

Health Technology Assessment (HTA)

Processo multidisciplinare di sintesi dell'informazione – raccolta in maniera sistematica, trasparente e scientificamente robusta - relativa alle questioni cliniche, sociali economiche ed etiche legate all'utilizzo di una tecnologia sanitaria.

Domini dell'HTA (EUnetHTA)	Informazioni fornite dall'HTA	Criteri per le decisioni
1. Problema di salute	Bisogno clinico assistenziale	Necessità
2. Descrizione e caratteristiche tecniche	Stadio di sviluppo / complessità	Fattibilità
3. Sicurezza	Stima della differenza del rapporto	Valore del beneficio clinico-
4. Efficacia clinica	rischio / beneficio	assistenziale aggiuntivo
5. Costi e valutazione economica	*Value for money* / impatto finanziario	Sostenibilità
6. Aspetti organizzativi	Appropriatezza organizzativa	Implementabilità
7. Analisi etica	Coerenza con i principi e valori	Maggiore rispetto dei principi e valori
8. Ricadute sociali	(Disuguaglianze, diritti, relazioni .....)	etici, sociali e legati
9. Ricadute legali		

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Dalla ricerca alle decisioni

Determinanti di efficacia dell'HTA

Determinanti tecnico-scientifici	Determinanti normativi
Qualità, trasparenza e indipendenza della valutazione	**Mandato istituzionale e ruolo nel processo decisionale**
Tempestività della valutazione	**Capacità di informare le decisioni**
Disponibilità delle informazioni scientifiche	**Trasparenza del processo decisionale**

HTA in Europa – il finanziamento

- **EUR-ASSESS – 1994-1997**
- **HTA Europe - 1997-1998**
- **ECHTA/ECAHI – 1999 -2001**

EUR-ASSESS Project Subgroup Report on Methodology

Methodological Guidance for the Conduct of Health Technology Assessment

by Alessandro Liberati, Trevor A Sheldon, Henry David Banta, Bengt Brorssen, Frederick Fleurette, Torben Jorgensen, Albert Jovell, James P. Kahan, Harri Sintonen, Gabriel ten Velden, Gert-Jan van der Wilt

2004 The European Commission and the Council of Ministers target Health Technology Assessment (HTA) as "a political priority", recognising "(…) an urgent need for establishing a sustainable European network on HTA"

2005 Call for project proposal answered by a group of **35** organisations throughout Europe

2006 EUnetHTA Project (2006-2008)

2009 EUnetHTA Collaboration (2009)

2010 EUnetHTA Joint Action 1 (2010-2012)

2012 EUnetHTA Joint Action 2 (2012-2015)

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> 50 ml €

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HTA in Europa – la normativa

Key issues addressed by the Directive

European Commission

Directive 2011/24/EU of patients' rights in cross-border healthcare

- Right to **choose and be reimbursed** for **healthcare provided** by public or private providers located in the EU
- More **transparency about their rights**, treatment options or , the quality and safety levels of healthcare providers
- Strong focus on **cooperation among Member States**
  - Mutual recognition of prescription
  - eHealth
  - Health Technology Assessment
  - **European Reference Networks**

Article 15

Cooperation on health technology assessment

1. The Union shall support and facilitate cooperation and the exchange of scientific information among Member States within a voluntary network connecting national authorities or bodies responsible for health technology assessment designated by the Member States.

European Commission

EU Objectives in HTA Article 15 Directive 2011/24:

- *Support cooperation between national HTA Authorities*
- *Support MS in the provision of objective, reliable, timely, transparent , comparable and transferable information […] to enable effective exchange of information*
- *Avoid duplication of assessments*

Article 15 Health Technology Assessment Network

Presidenza del Consiglio dei Ministri

CONFERENZA PERMANENTE PER I RAPPORTI
TRA LO STATO, LE REGIONI E LE PROVINCIE AUTONOME
DI TRENTO E DI BOLZANO

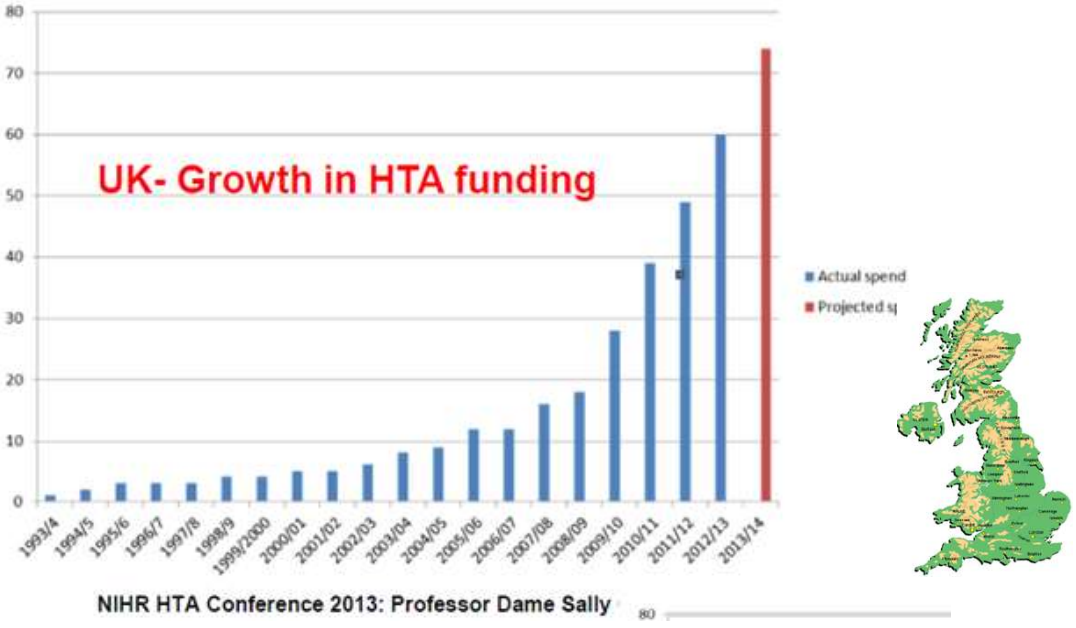
Patto per la Salute per gli anni 2014-2016

Articolo 26

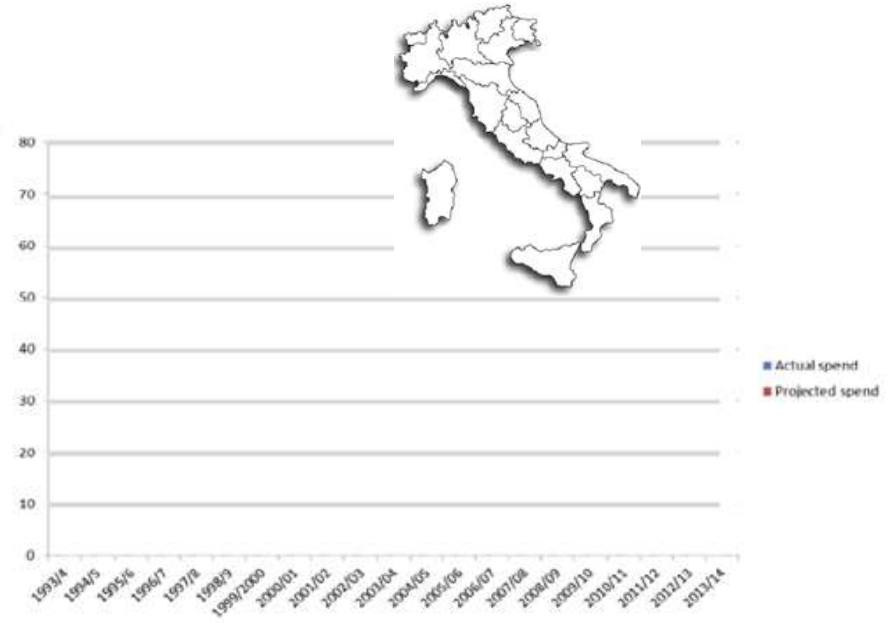
Creazione di un modello istituzionale di HTA dei dispositivi medici

Articolo 27

Valutazione nazionale dei medicinali secondo la metodologia dell'Health Technology Assessment



NIHR HTA Conference 2013: Professor Dame Sally



HTA in Europa – la normativa

EU initiative on HTA

Policy options*

Option 1	Option 2	Option 3	Option 4	Option 5
Status quo – **voluntary cooperation**	**Long-term voluntary cooperation** (beyond 2020)	Cooperation through the **collection, sharing and use of common tools and data**	Cooperation on **production of joint REA (relative effectiveness assessments) reports**	Cooperation on production of **joint Full HTA reports (REA+ economic)**
Non-legislative / *voluntary*		**Legislative / *voluntary + mandatory***		

+ Issues to be addressed

Scope | Funding mechanism | Coordination/ secretariat

*Inception Impact assessment available at: http://ec.europa.eu/smart-regulation/roadmaps/docs/2016_sante_144_health_technology_assessments_en.pdf

EU Cooperation in HTA – where we are

- **HTA Network** (art 15 Directive 2011/24)– **strategic level** (set up October 2013) →MS representatives (mainly MoH); EMA associated
- ***EUnetHTA*** *(Joint Action HP)– **scientific level** – on-going until October 2015 → HTA doers (mainly HTA Agencies)*
- *Two levels in synergy and complementary*
- *Involvement of stakeholders - both @ strategic level and scientific level*

Karolina Hanslik European Commission
DG SANTE - Health Systems

HTA in Europa – la collaborazione scientifica

EUnetHTA Partners and Associates

80 Partner organisations designated by Ministries of Health

Large number of regional agencies and non-for-profit organisations that produce or contribute to HTA

HTA in Europa – la collaborazione scientifica

Gli obiettivi

Efficiency	Quality	Availability of evidence
Timely information	Shared methodology	Communication of evidence needs
Less duplication	Quality Management System	Dealing with research gaps
Re-use and local adaptation	New/emerging methodological challenges	

Availability of Scientific Information

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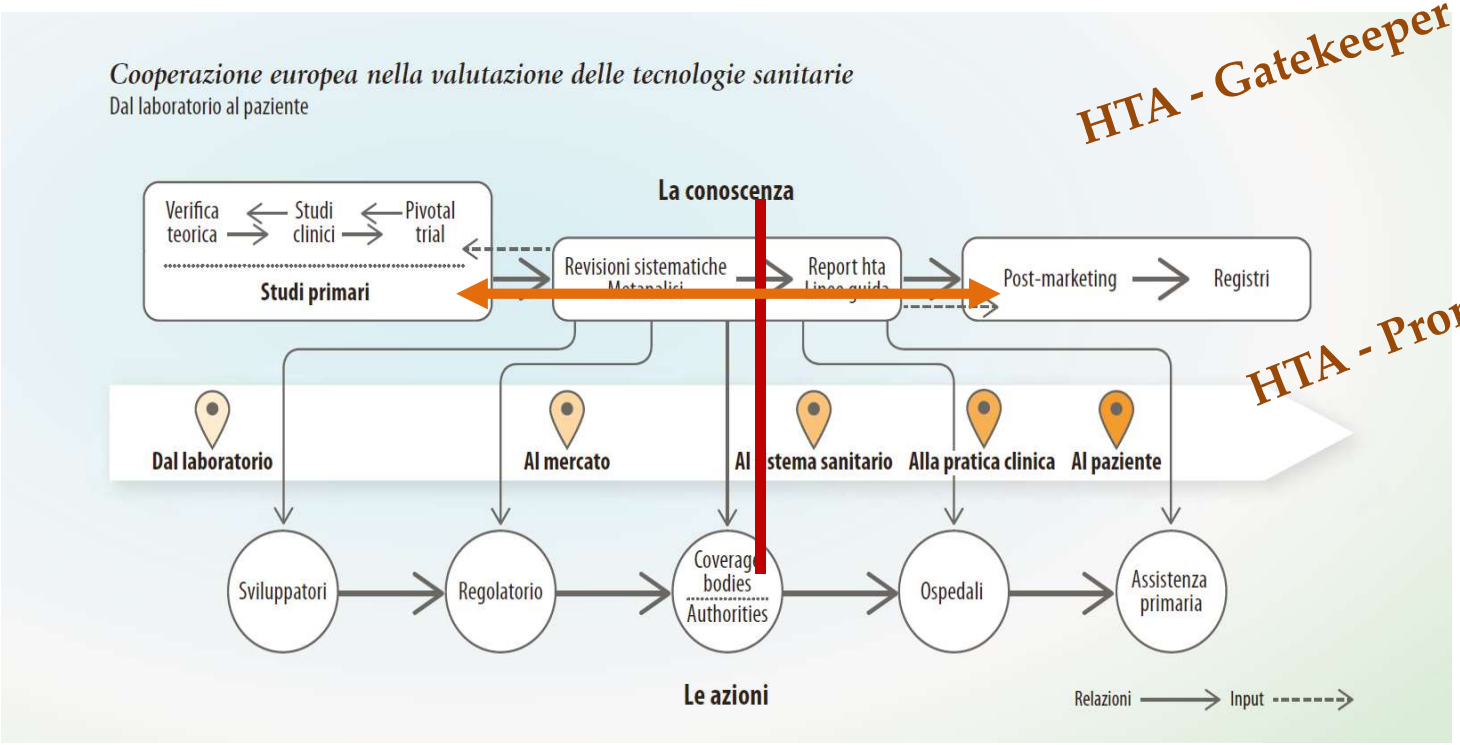
Evidence Generation – on the road to standardisation along the life-cycle of health technology

HTA - Gatekeeper delle innovazioni

HTA - Promotore di ricerca utile



Scoping: the assessment question

Scoping	Assessment questions
Population	**Who will use / be treated**
Intervention	**Which technology or procedure is under assessment**
Comparator	**Which are the best available alternatives**
Outcomes	**How are benefits and harms be measured**
Time	**Over what time period**
Studies	**Which study design is used**

Communicating research / evidence needs

IMPROVING INITIAL EVIDENCE GENERATION FOR HEALTH TECHNOLOGIES

Dealing with research / evidence gaps

IMPROVING ADDITIONAL EVIDENCE GENERATION FOR HEALTH TECHNOLOGIES

Additional Evidence Generation

Evidence gaps are often identified at the time of an initial Health Technology Assessment for new technologies. In order to reduce uncertainty, HTA bodies can make recommendations/requests for Additional Evidence Generation (AEG), which may include trials, observational studies, registries, data on appropriateness of use, etc.

Work Package 7 / Subgroup 2

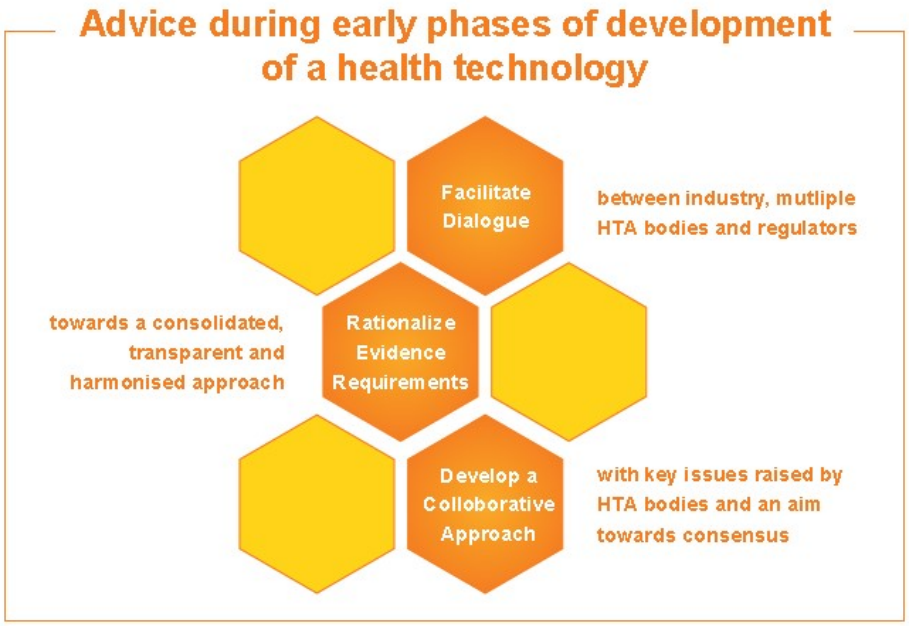
Objectives

- Help HTA doers define more specific research recommendations, that may better serve decision maker and researcher needs, by addressing the following two questions (two EUnetHTA position papers):
  - how to formulate a research question;
  - how to decide on the appropriate study design.
- Reduce multiple uncoordinated study requests and favor the collection of more robust data through a pilot of a common core study protocol.

Multi-HTA Early Dialogues

Objectives

The Early Dialogue (ED) overarching aim is to provide prospective, transparent and timely advice by HTA bodies to product sponsors so that they may integrate specific HTA needs in the product development.



to reduce the risk of producing data, that would be inappropriate for HTA purposes and inadequate to support the company's future reimbursement request.

Multi-HTA Early Dialogues

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Scope

Pilot EDs may be requested for a new technology, with supposed added benefit for patients, during the initial phase of its clinical development (e.g. end of phase II) to address questions pertaining to relative effectiveness and cost effectiveness.

Pilot EDs are: ■ prospective in nature ■ focused on development strategies and not on pre-evaluation of available data ■ limited to one indication and/or one line of treatment ■ non-binding ■ confidential ■ free of charge.

EDs allow companies developing health products to meet with European HTA agencies in order to present their development plan for the product in question and to ask specific questions relative to their plan

Multi-HTA Early Dialogues

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Main topics addressed during ED	
Population	Who will be enrolled
Intervention	How will the intervention be delivered
Comparator	Which alternatives will be chosen
Outcomes	Which benefits and harms will be measured and how
Time	Over what time period
Studies	Which study design and statistical analyses will be used

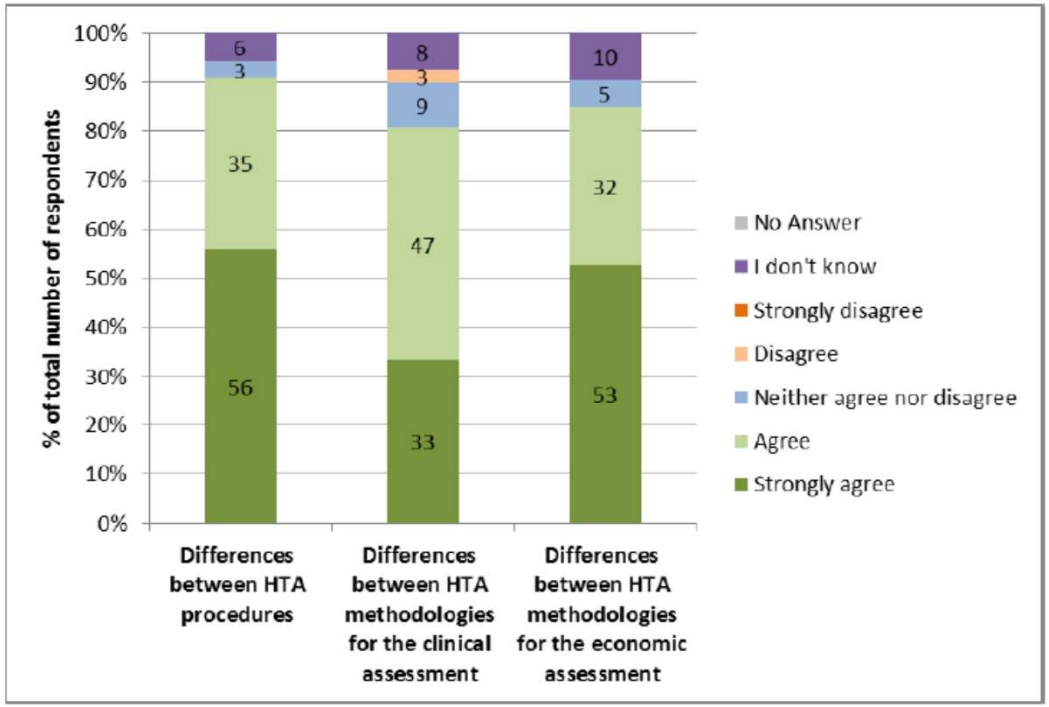

Fig. 12. Overview of the opinions expressed by administrations, organisations and associations on the existence of differences in HTA processes and methodologies across EU

Multi-HTA Early Dialogues

Some expected results

- ➢ Effort to reach consensus in compiling HTA answers
- ➢ Transparency of assessment process
- ➢ Quality of Scientific Advice
- ➢ Reduce unexplained variations in data requirements
- ➢ Effectiveness and efficiency of product development process
- ➢ Timeliness of access to effective innovation
- ✓ Pursue common scientific basis among HTA agencies

Multi-HTA Early Dialogues

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EUnetHTA Partners: 8 countries - HAS (France), HVB (Austria), KCE and INAMI (Belgium), RER-ASSR and AIFA (Italy), G-BA and IQWiG (Germany), GYEMSZI (Hungary), ZIN (Netherlands), TLV (Sweden).

11 EUnetHTA early dialogues (EDs)

- 9 drugs
- 2 medical devices

→ Multi HTA procedure

SEED Partners: HAS (France), HVB (Austria), KCE (Belgium), HIQA (Ireland), RER-ASSR and AIFA (Italy), G-BA and IQWiG (Germany), GYEMSZI (Hungary), ZIN (Netherlands), AVALIA-T, ISCIII and AETSA (Spain), NICE (UK)

SEED
Shaping European Early Dialogues

- Therapeutic domains covered: Rheumatoid Arthritis, Haematology, Neurolog, Gynaecology and Dermatology.

11 SEED early dialogues (EDs)

- 8 drugs → 4 EDs → EMA –Multi HTA procedure
- 3 medical devices → 4 EDs + 3 EDs → Multi HTA procedure

Multi-HTA Early Dialogues + JA3

Some challenges ahead

- From a EU sponsored model to a self-sustainable model.
- Choice of participating HTA bodies
- Relatives roles of national vs international
- Code of conduct
- Production of disease specific guidelines when necessary
- Adaptive pathways pilots: combines two approaches:
  Early Dialogues + Additional evidence generation

Francois Meyer (HAS)

Fees ?

Up to industry?

Regulatory capture?
Confidentiality?

Prioritization?

Early Dialogue for timely access
And
Early Dialogue for timely «stop»?

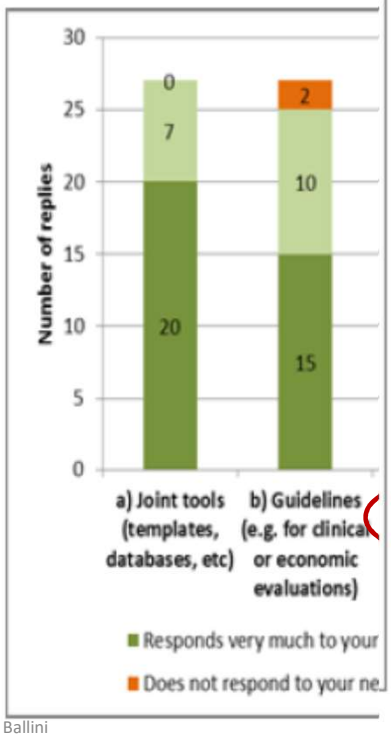
EU cooperation beyond 2020

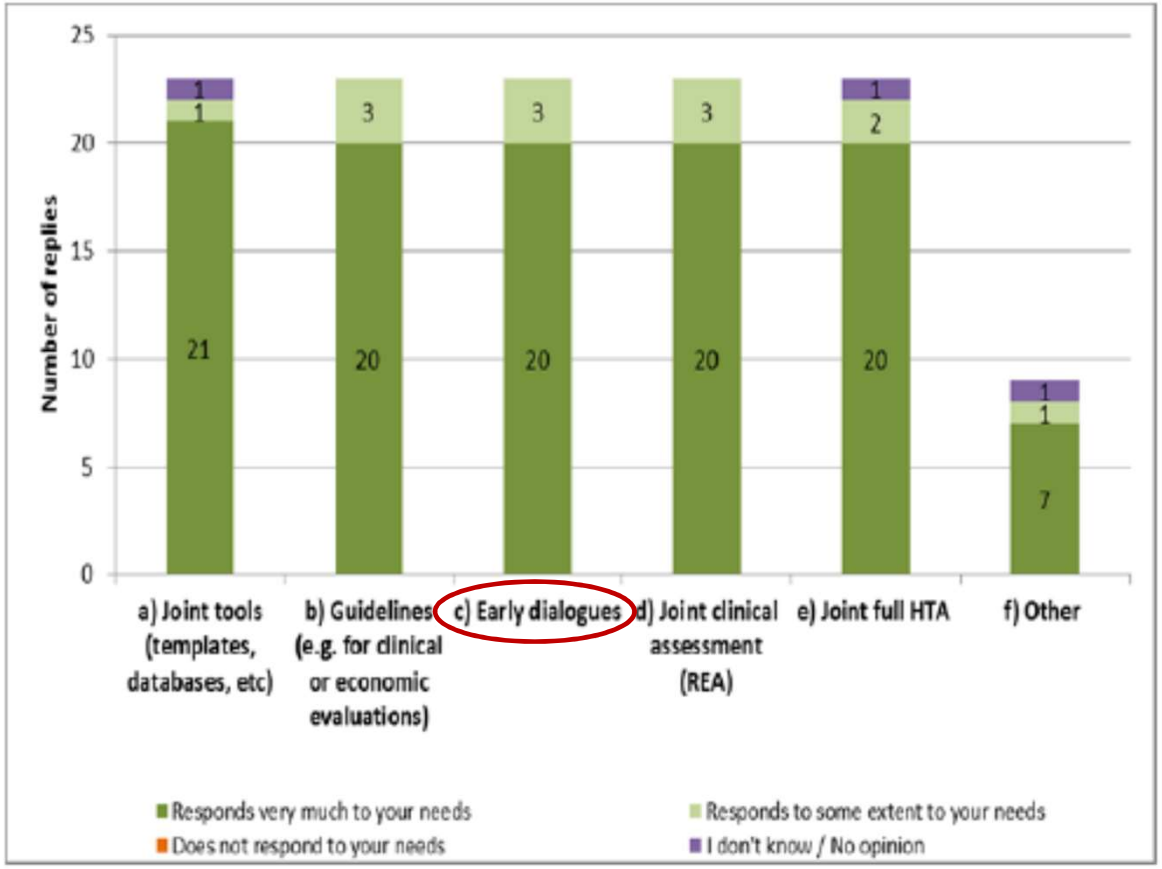
Public consultation

b) Patients and consumers

Different ne

Public





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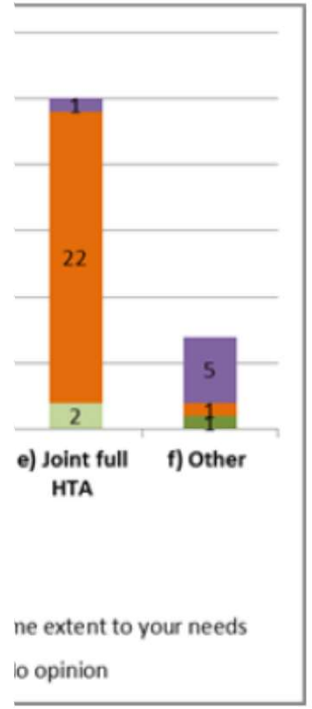

Policy Objectives of the HTA initiative

GENERAL OBJECTIVES:

1. *Ensure a better functioning of the internal market;*
2. *Contribute to a high level of human health protection.*

SPECIFIC OBJECTIVES:

1. *Reduce discrepancies of procedures and methodologies;*
2. *Reduce duplication of efforts;*
3. *Ensure the uptake of joint work in Member States; and*
4. *Ensure the long-term sustainability of EU HTA cooperation.*

6

Tensioni		
Cittadini/professionisti	Beneficiari di innovazioni efficaci Strumenti inconsapevoli di profitti	oppure
Sistemi sanitari	Sostenibilità attraverso costo-beneficio ottimale Tetti indifferenziati di spesa	oppure
Industria	Velocizzazione dell'accesso al mercato Selezione con onere della prova	oppure

Disease specific guidelines: extension of scope of Early Dialogue to guidelines on technology development