Council of the European Union
General Secretariat

Brussels, 18 April 2018

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WORKING PAPER

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INFORMATION

From:	General Secretariat of the Council
To:	Working party on Pharmaceuticals and Medical Devices (HTA)
Subject:	Proposal for a REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL on health technology assessment and amending Directive 2011/24/EU - Presentation by the Commission on the Impact Assessment

Delegations will find enclosed the presentation on the above mentioned topic by the Commission delegation at the meeting of the Working Party on Pharmaceuticals and Medical Devices on 17 April 2018.

Impact Assessment supporting

Proposal for a

REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

on health technology assessment and amending Directive 2011/24/EU

DG SANTE - Health Systems and Products
Medical Products: safety, quality, innovation

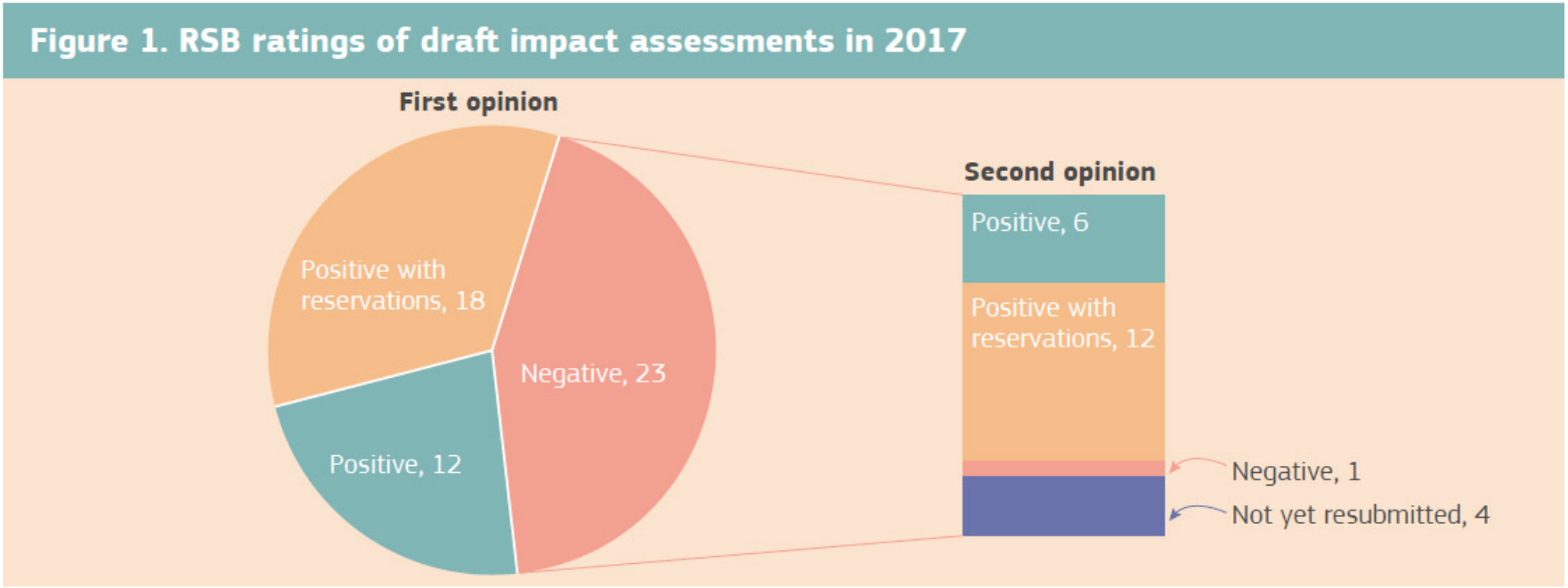
HTA initiative: key milestones

- **Inception impact assessment (IIA) -** published Sept. 2016
- **Inter-Service Steering Group** - set up in Sept. 2016
- **Consultation**
  - **Online public consultation** – Report published May 2017: https://ec.europa.eu/health/technology_assessment/consultations/cooperation_hta_en
  - Meetings with **EUnetHTA JA3** and **HTA Network**
  - Bilateral meetings with **Member States authorities**
  - Discussions with **stakeholders**
- **Studies** to support the IA process
- **Impact assessment** – finalised October 2017
- **Commission legal proposal** – 31 January 2018
- **Feedback period** for citizens/stakeholders – 2 April 2018

Consultation of the RSB and other EC services

- **Inter-Service Steering Group** set up in Sept. 2016 and consulted throughout the process (SG, LS, BUDG, GROW, RTD, CNECT, ECFIN, EMPL, TRADE, COMP, JRC, ENER)
- Upstream meeting with the RSB in December 2016
- 1st version of IA report submitted to RSB in Sept. 2017: **1st RSB opinion** of 27 Oct. 2017 (negative)
- 2nd revised version of IA report submitted to RSB in Nov. 2017: **2nd RSB opinion** of 4 Dec. 2017 (positive with reservations)
- 3rd revised version of IA report submitted to SG and Inter-Service consultation: **Final approved version of IA report** published on 31 January 2018

Comparison to other IAs assessed by RSB in 2017



See RSB Annual Report, 2017

RSB opinion addressed in final version of IA report

RSB main considerations	Adjustments made in final version of IA report
1) The baseline is treated as an option and not as a comparator for the options.	The final version of the IA report ensures that policy options are consistently compared to the baseline scenario. This has also been clarified for figures related to governance and budget. Adjustments were made accordingly in sections 5.3.1 and 6.5.
2) The report provides indications that the mandatory uptake of joint work would be sufficient to address many of the current shortcomings. However, it does not convincingly demonstrate that it is necessary. It is not clear what the resulting amendments to the existing Directive are.	Further clarifications have been provided on the **proportionality of the preferred option**, elaborating **why mandatory uptake of joint work is considered necessary** (see section 8.2) and clarifying the issue of legal/procedural hurdles to uptake (sections 2 and 8.1). Moreover, the final version of the IA report clarifies that some of the principles referred to in the current Article 15 of Directive 2011/24/EU (e.g. good governance, transparency) will also be present in the new legislative framework proposed under the preferred option (sections 3 and 8.1).
3) The report provides insufficient indications of Member States' support for key aspects of the options.	Further details have been provided on expected **Member States** support for key aspects of the initiative, including acceptability of mandatory uptake of joint work, willingness and capability to take a leading role in an EU framework and support for transparency measures (section 8.3). The choice of a **Commission-hosted secretariat** is also further elaborated (section 8.1.4).
4) The revised report insufficiently discusses the uncertainties, risks, trade-offs and implementation challenges associated with the preferred option.	Risks and possible unintended consequences of the initiative have been further discussed, to better contextualise/qualify the expected benefits of the initiative (sections 8.2 and 8.3).

Main evidence supporting the IA report

Studies conducted to support the IA

- Mapping of HTA National Organisations, Programmes and Processes in EU and Norway (Julia Chamova, Stellalliance A)
  - **Differences in HTA processes across the EU**
- Mapping of HTA Methodologies in EU and Norway (Finn Børlum Kristensen, Science & Policy)
  - **Differences in HTA methodologies across the EU**
- Study on Impact Analysis of Policy Options for Strengthened EU Cooperation on HTA (Austrian Public Health Institute, London School of Economics, Sogeti)
  - **Duplication of work for national HTA bodies and industry**
  - **Impacts of policy options**

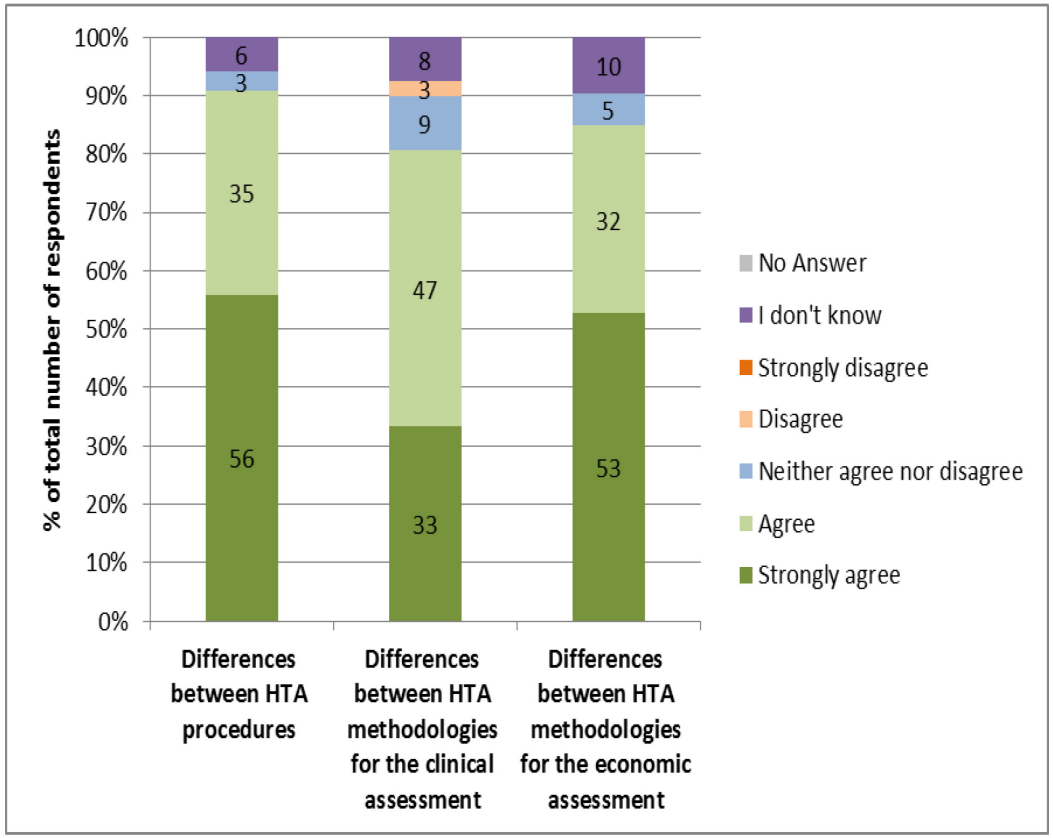
Accessible at: https://ec.europa.eu/health/technology_assessment/eu_cooperation_en

Extensive stakeholder consultation

- IA report, Annex II: Synopsis report on stakeholder consultation
- Report on open public consultation https://ec.europa.eu/health/technology_assessment/consultations/cooperation_hta_en

Results of open public consultation

Opinions on the existence of differences in HTA processes and methodologies across the EU



Results of open public consultation

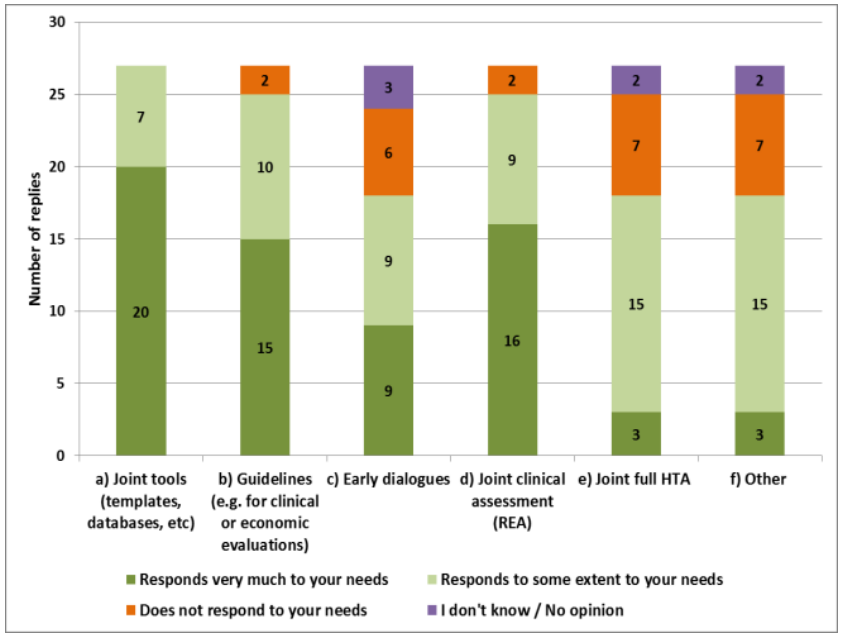
Opinions on consequences of differences in HTA processes and methodologies across the EU

European Commission

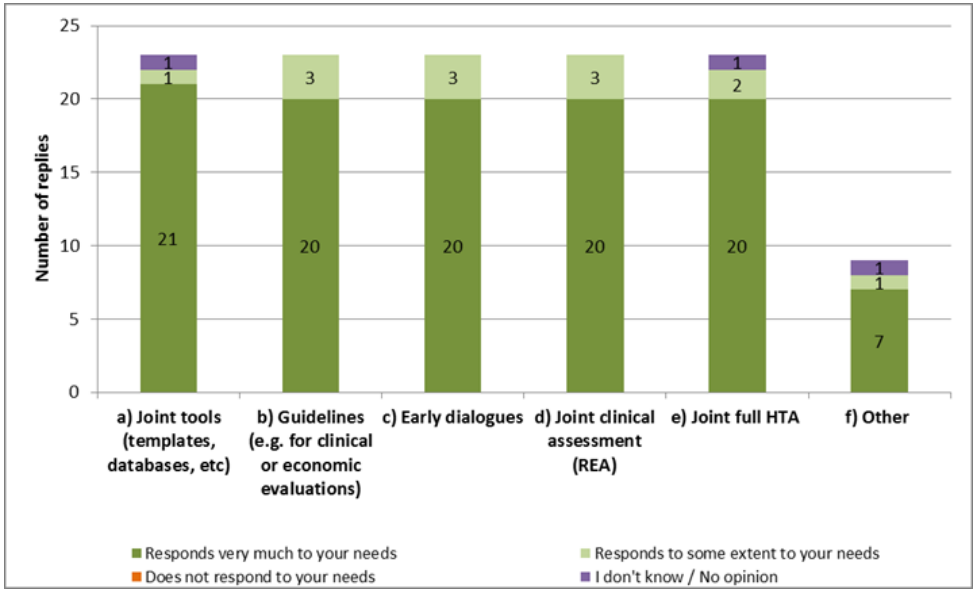
Results of open public consultation

Opinions on needs for particular types of joint outputs under future EU cooperation

Public administrations



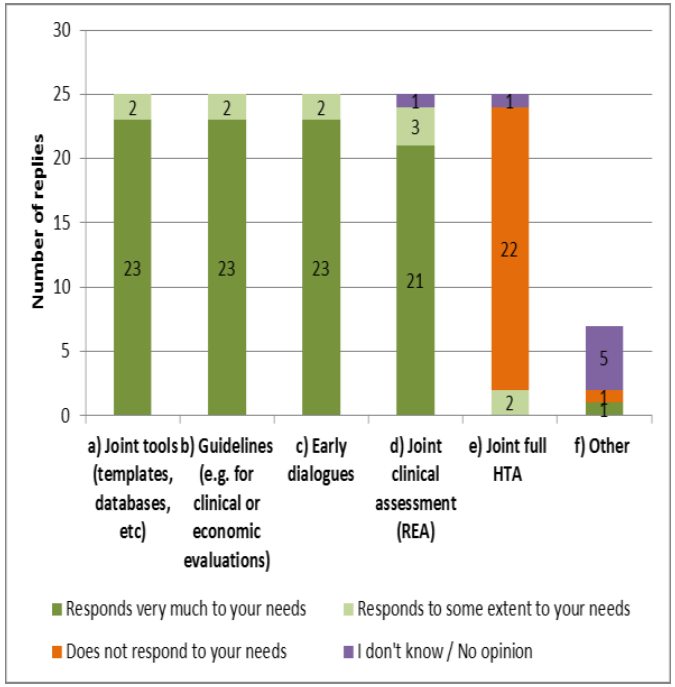
Patients and consumers



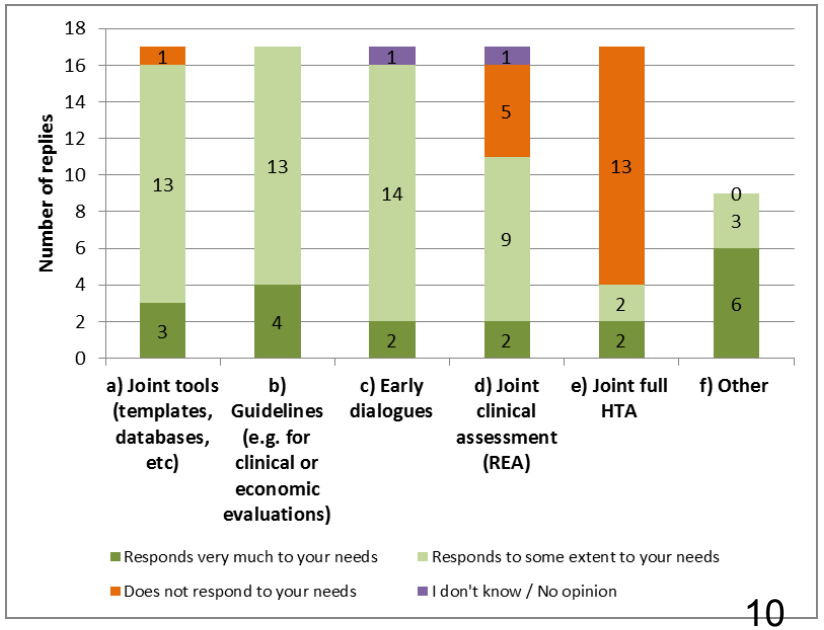
Results of open public consultation

Opinions on needs for particular types of joint outputs under future EU cooperation

Pharmaceutical industry

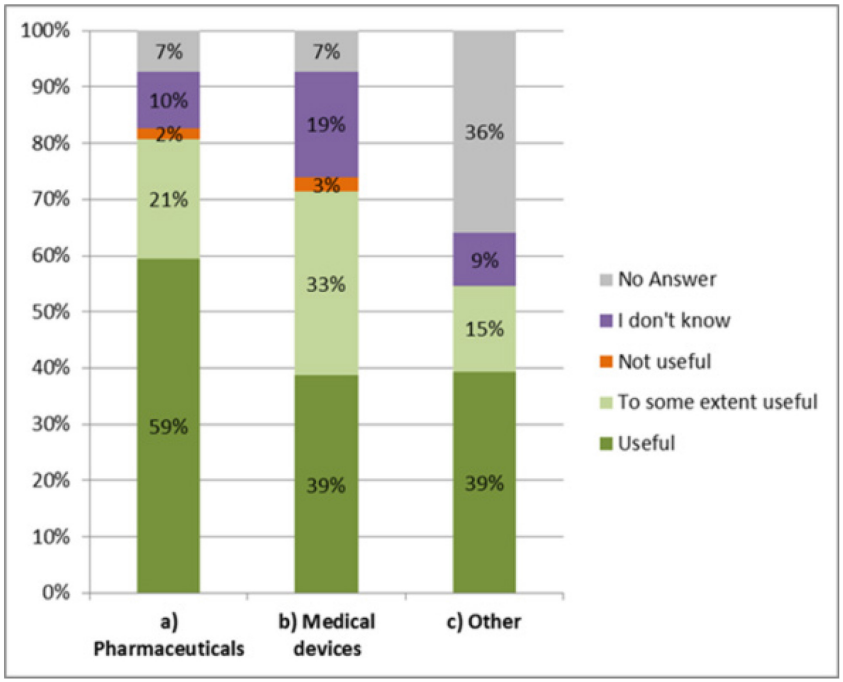


Medical technologies (devices/IVD) industry

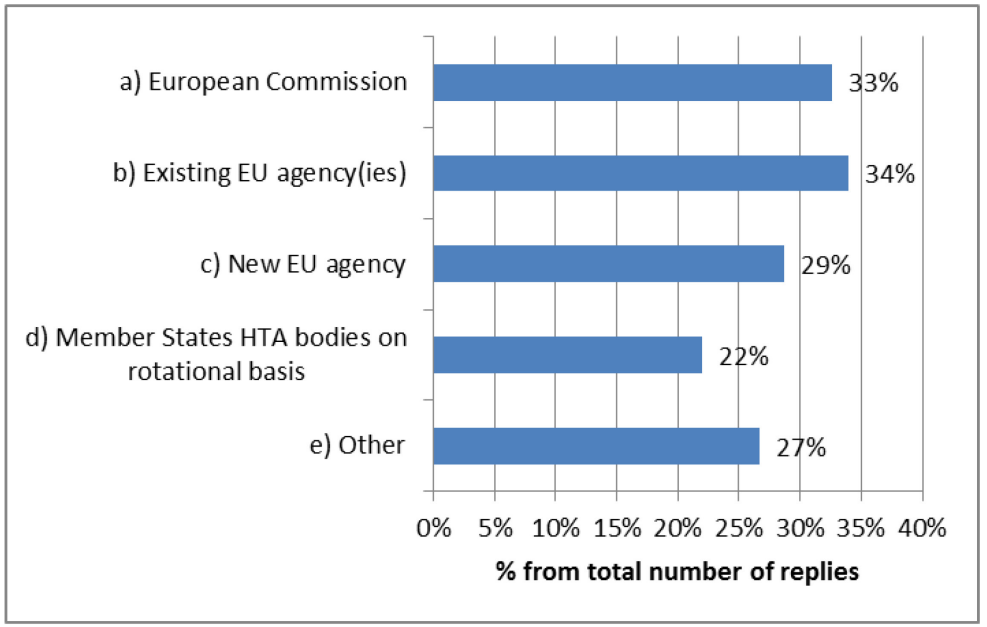


Results of open public consultation

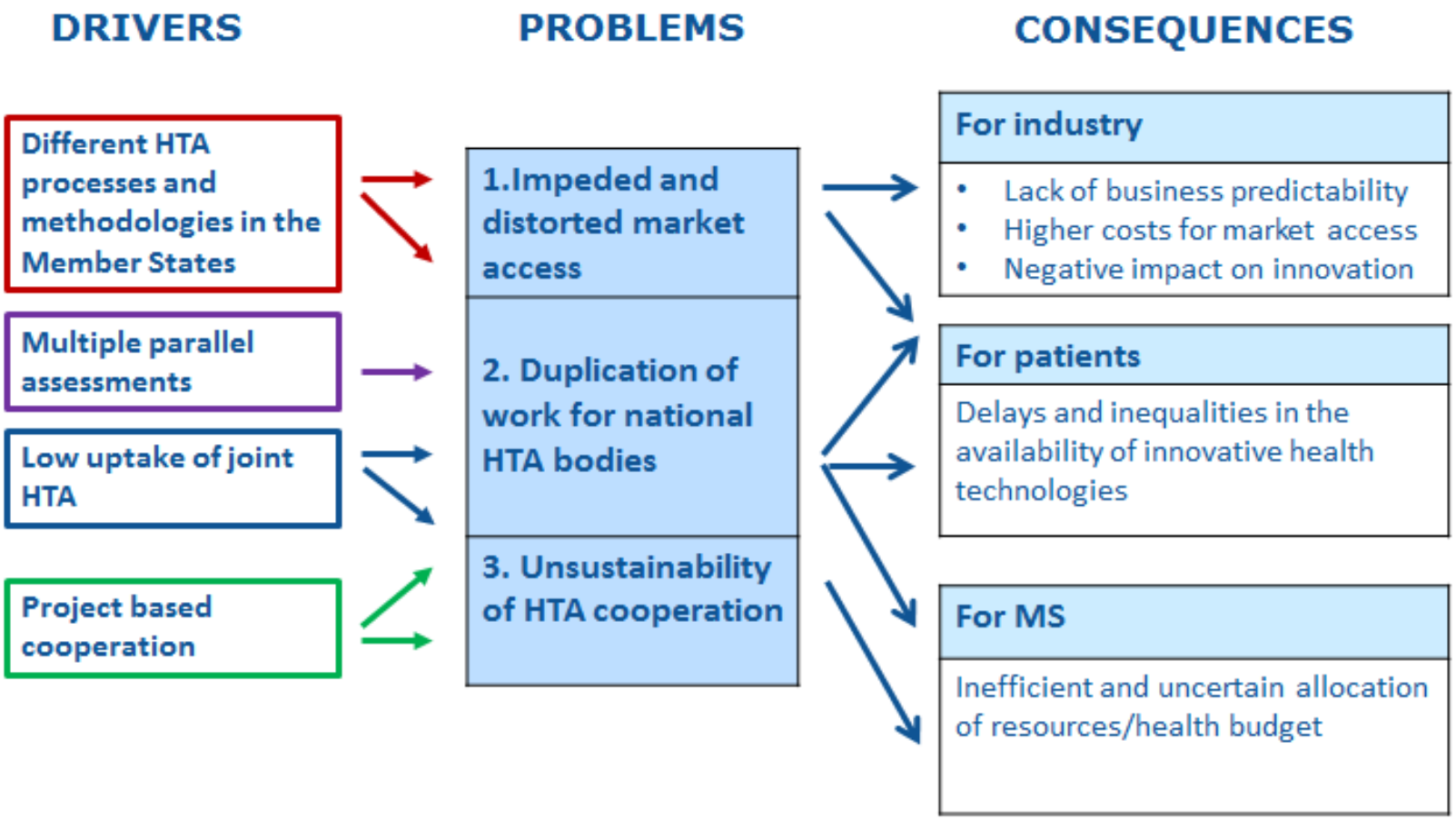
Technology scope of future EU cooperation



Governance mechanism for future EU cooperation



Problem analysis



European Commission

Why should the EU act?

- The **aims of this initiative cannot be achieved sufficiently without strengthened cooperation at EU level**. As described in section 2, the diversity and multitude of approaches to HTA across the Member States means that, due to their scale and effect, only action at Union level can eliminate the obstacles described. Without action at EU level, the current fragmentation of the single market would persist.

- While the on-going cooperation (EUnetHTA, HTA Network), has illustrated benefits of EU cooperation (professional networking, piloting of joint work), the **current voluntary cooperation model has not addressed the issues of fragmentation and duplication of efforts** across the EU.

Policy objectives

IA report: Section 4

General objectives

- Ensure a better functioning of the internal market
- Contribute to a high level of human health protection

Specific objectives

- Improve the availability of innovative health technologies for EU patients
- Ensure efficient use of resources and strengthen the quality of HTA across the EU
- Improve business predictability

Operational objectives

- Promote convergence in HTA tools, procedures and methodologies
- Reduce duplication of efforts for HTA bodies and industry
- Ensure the uptake of joint outputs in Member States
- Ensure the long-term sustainability of EU cooperation

Policy options

		Non-legislative		Legislative		
		PO 1	PO 2	PO 3	PO 4	
		No EU action after 2020 (baseline)	Project-based cooperation on HTA activities	Permanent cooperation on common tools, procedures and Early Dialogues	Permanent cooperation on common tools, procedures and Early Dialogues and REA	
					4.1 REA (MS opt-in)	4.2 REA (all MS)
Joint outputs	Common tools and procedures					
	Early dialogues					
	Joint REA					
	Joint Full HTA					
Technologies covered			Pharmaceuticals, medical and other technologies	Pharmaceuticals, medical and other technologies	Pharmaceuticals, medical and other technologies	
Governance		No EU support	Project based cooperation	Permanent structure	Permanent structure	Permanent structure
Financing		No EU support	EU+MS	EU+MS+fees from industry (for early dialogues depending on chosen governance model)		

Early dialogue = "Joint scientific consultation" (Legal proposal)
REA = "Joint clinical assessment" (Legal proposal)

Economic and social/health impacts assessed

	Member States/ Public Administrations	Patients/consumers	Industry (pharmaceutical and medical technologies)
Economic Impacts	- Costs - Efficient allocation of resources - Administrative burden - EU budget		- Functioning of the internal market - Costs - Business predictability - Innovation, research and competitiveness - Administrative burden
Social/health impacts	- Governance, participation and good administration - Sustainability of health systems - Public health	- Participation/ involvement - Availability of innovative health technologies	

Comparing policy options

	Effectiveness				Efficiency (benefit to cost)	Coherence - A deeper and fairer internal market - Support health systems - Foster research and innovation	Subsidiarity and Proportionality
Operational Objectives	**Promote convergence in HTA Procedures methodologies**	**Reduce duplication of efforts for HTA bodies Industry**	**Increase the uptake of joint output in MS**	**Ensure long term sustainability of EU HTA cooperation**			
Policy Option 4.2 ***Permanent cooperation on:*** ***→common tools*** ***→methodologies*** ***→early dialogues*** ***→joint REA*** ***(all MS from the start)***	+++ Ensured convergence in HTA procedures and methodologies in all EU MS. No risk of divergent outcomes for the clinical assessment, therefore business predictability considerably improves and ultimately patients will benefit from the availability of HTA.	+++ No duplication of work. Efficient pooling of resources and expertise. Expected increase of quality of HTAs.	+++ Mandatory uptake by HTA bodies is ensured.	+++ Long term sustainability is ensured by the permanent structure and the stable funding from EU budget + MS in kind contribution + industry fees for early dialogues.	+++ EU Patients: improved availability of innovative health technologies and also improved participation in the HTA process. For HTA bodies: better evidence is available, efficient allocation and use of resources /expertise. For industry business predictability considerably improves. Costs savings are expected. Benefit to cost ratio is expected to be the most advantageous compared to the other options.	+++ Positive performance concerning the contribution of this option to a fairer and deeper internal market of health technologies and EU patients are expected to benefit from it. The identified obstacles impeding a well-functioning internal market are addressed. Business predictability is expected to improve. Health care systems of EU MS will benefit from better quality evidence and efficiency gains.	+++ This option provides for a pooling of expertise and resources providing an EU added value to MS activities in the area of HTA.

PO	+	++	+++	0	-	- -	- - -	n/a	Total
1						*	*****	*	-17
2				*		******			-12
3	*****	*	*						+10
4.1		******	*						+15
4.2			*******						+21

Overview of the preferred option

Preferred option: PO 4.2 adjusted in light of IA and stakeholder comments, also integrating elements of other POs (4.1 and 2)

- **Joint outputs:** common tools/procedures, joint ED, joint REAs
- **Technology scope**
  - **Pharmaceuticals**: centrally authorised new active substances and extensions new therapeutic indications
  - **Medical technologies** (devices/IVDs): highest risk classes, selected by Member States based on additional criteria (incl. impact on healthcare systems across the EU)
  - **Other health technologies**: voluntary cooperation
- **Governance:** MS high-level group/sub-groups + EC secretariat
- **Financing:** EU budget + MS in-kind (no industry fees)
- **Timeline**: Deferred application + transitional period

Expected outcomes

Member States

- Pooling of resources and expertise (quality and **efficiency** gains)
- High **quality** and **timelinesss** of reports
- Support MS in **evidence-based decision-making**
- Contribution to **sustainability** of health systems

Patients

- Increased **transparency**
- Increased **engagement** in the HTA process
- Contribution to **improved access** to technologies with benefits for patients

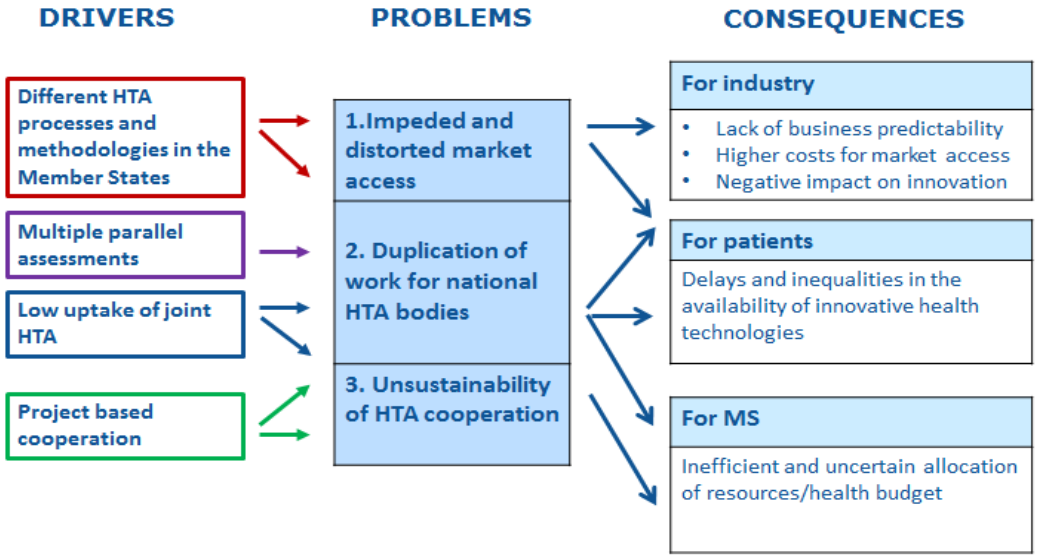
Industry

- Positive impact on **business predictability** (innovation investments)
- Increased **efficiency** of evidence generation and submission (reduced duplication)

Legal basis

Article 114 (TFEU) allows for the adoption of measures for the approximation of the provisions laid down by law, regulation or administrative action in the Member States, provided they are necessary for the establishment or functioning of the internal market, whilst at the same time ensuring a high level of public health protection.

Most health technologies are products which benefit from the free movement of goods within the internal market. Despite this, a number of obstacles to their free movement have been identified (see in section 2 of the IA report).



Subsidiarity

- **Joint work** focuses on **clinical aspects of HTA**, where EU cooperation can bring both quality and efficiency gains.
- The **assessment of more context-specific HTA domains** (e.g. economic, organisational, ethical) will remain **at Member State level**.
- Mandatory **use of a joint REA does not preclude the national appraisal process** which will continue to conclude on overall added value of a health technology.
- The initiative **fully respects Article 168(7) TFEU** which stipulates that the Union shall respect the responsibilities of Member States for the definition of their health policies and for the organisation and delivery of health services and medical care. In particular, Member States are responsible for decisions on **pricing and reimbursement**, which are **not within the scope of this initiative**.

Proportionality

- **Joint work** focuses **on clinical aspects of HTA**, where EU cooperation can bring both **quality and efficiency gains**, leaving assessment of more context-specific HTA domains and decision-making on pricing and reimbursement at Member States level.
- Mandatory production and uptake of joint work is limited to **specific types of pharmaceuticals and medical technologies**, focusing on the type of products **where current duplication of work** among HTA bodies is **most prominent**. For other technologies, the preferred option facilitates further voluntary cooperation.
- Mandatory uptake **does not go beyond what is necessary to ensure** that **joint outputs** (e.g. joint REAs) are **incorporated into national HTA processes**.
- Preferred option allows **sufficient time** for both Member States and industry **to adapt to the new EU system.**

Specific approach for Medical Technologies

- **Separate from but coherence with MD Regulations:**
  - Scope (expert panel opinions)
  - Timing – post-2022 phase-in approach
- **Limited scope** (highest risk classes) including permanent selection procedure by HTA Coordination Group
- **Limited volume** of assessments (< pharma)
- **Different timing** of assessments (≠ market launch)
- **Sector-specific**:
  - Tools and methodologies
  - Member Agencies of the HTA Coordination Group and dedicated sub-groups

Why mandatory uptake of joint REA?

- **Ensure full delivery on the objectives of the initiative** (functioning of the single market and public health across the EU, incl. efficient use of resources for Member States and the EU, and reduced duplication of efforts for HTA bodies and industry)

- **Ensure that all Member States consistently use the joint outputs** (rather than deciding on uptake only on a case by case basis, possibly only once the joint output has been produced)
  - **Stability and predictability of the system**

- **Incentivise Member States to invest capacities and resources** into the production of joint REA at EU level and to build scientific consensus and ensure **high quality**.

- **Coherence with mandatory submission requirement for industry**. Further incentivises manufacturers to submit **complete and high quality dossiers**.

What does "mandatory uptake" imply?

- A **jointly produced REA should not be repeated** again at national level, **but should be incorporated in the national HTA process** (i.e. used in the same way as an equivalent national clinical assessment would be used).
- Member States continue to be free to assess **more context-specific HTA aspects** (e.g. organisational, economic, ethical) **at national level**
- Member States continue to be free to conduct their **national appraisal processes, i.e. to draw conclusions on** the presence/absence or extent of **added value** (e.g. therapeutic, economic, societal).

Assessment vs. Appraisal

Legal proposal: Article 6, Recital 16

Joint clinical assessment:
(a) an analysis of the **relative effects** of the health technology being assessed on the patient-relevant **health outcomes** chosen for the assessment
(b) the **degree of certainty** on the relative effects based on the available evidence.

NATIONAL

NATIONAL APPRAISAL
of joint clinical assessment and additional context-specific considerations (e.g. number of patients affected in Member State, how patients are currently treated in the healthcare system, costs)

Conclusions on added value
(e.g. added therapeutic value, cost-effectiveness…)

Joint clinical assessment:
(a) an analysis of the **relative effects** of the health technology being assessed on the patient-relevant **health outcomes** chosen for the assessment
(b) the **degree of certainty** on the relative effects based on the available evidence.

Example (medicine A vs. comparator B, based on trial XXX):

Mortality

In the clinical trial XXX, an improvement in overall survival (OS) was observed in the treatment group (medicine A) compared to the comparator group (medicine B). […]

Morbidity and health-related quality of life

An improvement of disease symptom X was observed in the treatment group. […]

The quality of life questionnaire used in the trial did not reveal significant differences between treatment and comparator groups. […]

Safety

Serious adverse events (SAE) occured at similar frequencies in the treatment and comparator groups. […] Adverse events of any grade were more frequent in the treatment group […].

For each health outcome: Detailed discussion of the effect observed (e.g. statistical analysis and its interpretation; any limitations of the clinical trial which may affect certainty in the effect)

Note: The report may include analyses/discussions against several comparators (e.g. another section: medicine A vs. comparator C, based on trial YYY).

Thank you!