Annexe: The Regulation Proposal for an EU Cooperation in HTA

Summary description of the European cooperation on HTA – a Regulation proposal

How does it work?

Structure of the HTA cooperation: a scientific secretariat as part of a European Commission unit

The proposed EU cooperation on HTA does not imply the creation of an EU Agency, nor does it propose to make European HTA activities part of the European Medicines Agency. Rather it establishes a scientific and technical secretariat responsible for coordinating the joint activities as part of the European Commission.

The core of the structure will be the Member States' **Coordination Group**

- The Coordination Group will be Members State-led
- It will be composed by EU Members States' representatives, namely HTA experts from national or regional bodies
- It will be responsible for endorsing the work of all four working groups (see below CG Sub-groups), including the production of joint HTA reports, and for distributing the workload among the national HTA Agencies participating in the cooperation
- It will ensure appropriate involvement of stakeholders

All joint HTA reports shall be approved by the Coordination Group. When adopted, they will be the basis for MS to complete the evaluation with more contextual elements: the joint HTA report will contain an evaluation of the medical aspects, and MS will add contextual elements such as costs, economic and organisational aspects. With both components, the medical evaluation, and the contextual elements, decision-makers will decide to cover/reimburse the technology or not.

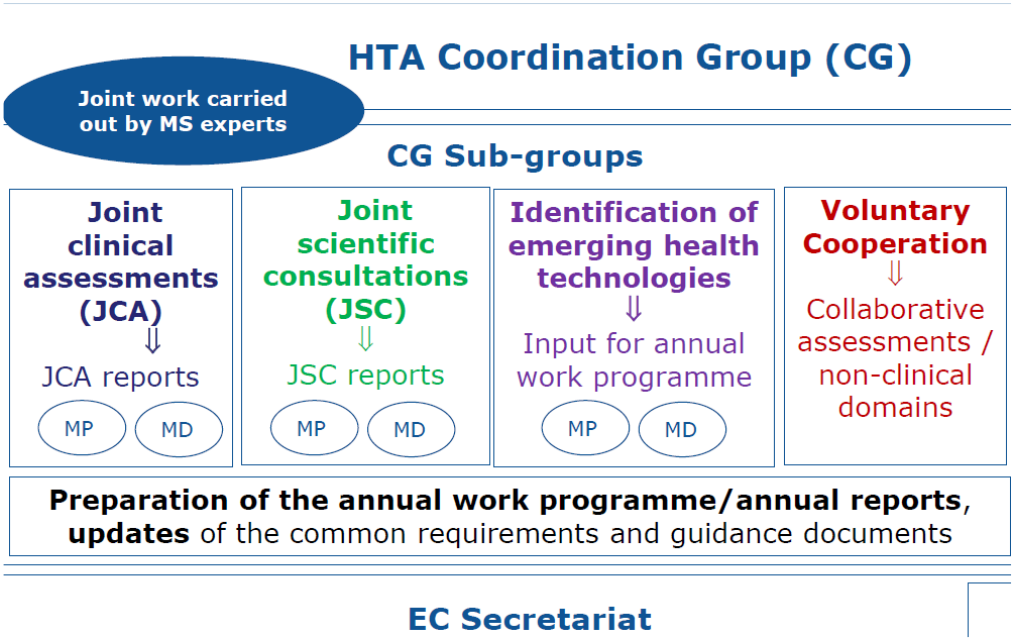

Figure 1: structure of the European Cooperation on HTA. The HTA coordination group will be composed of representatives of HTA agencies, both for pharmaceuticals and for other technologies (medical devices...). When a MS will be represented by several agencies, it will have one vote to keep the balance between all MS. The work will be divided amongst four working groups: MS will decide which working group(s) they want to be part of, on a voluntary basis. If a MS wishes to comment on the joint reports or to be the main assessor for some technologies, it needs to be part of the working group on joint clinical assessment (JCA). Else, it may miss an opportunity to comment on the reports prior to their adoption.

Activities of the working groups

The national HTA Agencies that participate in the EU HTA Cooperation will carry out different activities, for which different working groups will be set-up.

All national agencies involved in the cooperation are supposed to participate in some way to the working groups.

The joint activities are:

1. Joint clinical assessment (Joint HTA report)
2. Joint Scientific Consultation (so-called Early Dialogue; the equivalent for HTA of the EMA Scientific Advice) and parallel scientific consultations with regulators (HTA/EMA)
3. Identification of emerging technologies (so-called Horizon Scanning)
4. Voluntary cooperation

1. Production of Joint Clinical Assessment

This group will assess the relative effectiveness of new and/or obsolete health technologies. Each assessment will be coordinated by one (or maybe two) agencies, and a draft report will be submitted to other agencies that are part of this working group. The agency coordinating the report will be paid for the work done, and a procedure will be created to distribute the workload among agencies that have the capacity to coordinate such a work.

In accordance with the rationale of the EC proposal, this joint HTA report will be strictly limited to an evidence-based scope such as the clinical aspects of the technologies.

EUnetHTA, the European Network of HTA Agencies created the HTA Core Model©, as a common tool to carry out assessments. It includes nine (9) domains of the technology to be assessed. Four (4) domains are clinical ones (description and current use, efficacy, safety and relative effectiveness, the others are non-clinical (costs and economic aspects, ethical aspects, organisational aspects, patient impact and social aspects, and legal aspects).

For the proposed European cooperation on HTA, four domains out of the nine will be addressed: the non-clinical domains such as organisational and social aspects and, economic and budget impact analysis, remain a matter of national analysis.

If needed, other national elements can be analysed that complete the European report, for example when specific treatments are used in a given Member State.

The joint HTA report will not recommend a health intervention for reimbursement or coverage. It will analyse the medical evidence, and conclude on its relative effectiveness compared to existing treatment options.

The text of the proposal leaves the possibility for all national agencies participating in the cooperation to contribute to the work of the authors at a draft stage, before its approval by the sub-group and the endorsement of the Coordination Group.

In that way, it should be assured that no joint work authored or reviewed by certain agencies might be imposed to others.

- Use of joint clinical assessment: the so-called *mandatory uptake*

There is no sense in perpetrating the current *status quo* with its burden of duplication and inconsistency.

The only obligation provided for in this Regulation is the prohibition of duplication at national level of the assessment of the same clinical aspects already assessed jointly and adopted by all representatives of the Member States.

National agencies will be able *to complete the joint report, but not to duplicate the assessment of the four clinical domains already assessed jointly* through common methodologies and data.

Thus, as all Member States will be able to contribute to the joint report. This will not interfere with their assessment of the costs and economic aspects that are needed for the national decision-making.

1. Joint scientific consultation

As for the Scientific Advice performed by the European Medicines Agency for pharmaceuticals, manufacturers could ask for a scientific advice on which information need to be collected to reduce the risk that inadequate information are submitted to HTA agencies.

The advice given will not be legally binding. The conclusions of a potential future joint clinical assessment will remain independent from the conclusions of that scientific consultation. Some will be parallel HTA and regulatory advice.

2. Identification of emerging technologies

Horizon Scanning consists in detecting emerging technologies that are likely to reach the market with a significant impact on public health and/or of the healthcare budget, and payers/health insurances need to be alerted early enough to plan their budgets.

3. Voluntary cooperation (for those who want to do more together)

For those activities and products falling outside the scope of the joint work, the Regulation provides a series of further actions to be taken jointly on a voluntary basis.

In fact, for those Members States who wants to accomplish more together and improving their cooperation, it is possible to perform collaborative assessments for technologies that were not selected for the joint clinical assessment, or produce non-clinical assessments by sharing their expertise and tools on the evaluation of supplementary HTA domains, as social and economic ones.

Scope

The European Cooperation on HTA aims at assessing different types of health technologies:

- All new active substances (pharmaceuticals) and new indications for known substances passed through the Centralised procedure for Marketing Authorisation at the European Medicines Agency
- Certain classes of medical devices which have undergone the scrutiny mechanism in the context of their conformity assessment (CE marking) and in vitro diagnostic medical devices

Not all newly approved medicinal products or medical devices can be assessed from the beginning. Then, they will be selected by the Coordination Group on some general criteria.

Criteria:

- Unmet medical need
- Potential impact on patients, public health, or healthcare systems
- Significant cross-border dimension
- Union-wide added value (relevance to a large number of MS)
- Available resources for the activity

About EURORDIS

EURORDIS - Rare Diseases Europe is a unique, non-profit alliance of over 700 rare disease patient organisations from more than 60 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe.
By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.
EURORDIS works across borders and diseases to improve the lives of people living with a rare disease.

The EURORDIS vision is better lives and cures for people living with a rare disease.
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