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European Federation of Pharmaceutical Industries and Associations

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EFPIA position on proposal for a Regulation of the European Parliament and the Council on health technology assessment and amending Directive 2011/24/EU

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✱ Position Paper

The European Federation of Pharmaceutical Industries and Associations (EFPIA) welcomes the Commission proposal for a Regulation on health technology assessment (HTA). The proposal builds on many years of voluntary cooperation in HTA between Member States at EU level. It presents a unique opportunity for greater alignment in Europe on clinical evidence generation requirements, ensuring consistency, transparency and synergies in clinical assessments by Member States and evidence that is relevant for Europe. For patients, the availability of one common joint clinical assessment at the time of marketing authorization will expedite patient access to new medicines. For national healthcare systems, it would mean avoiding duplicative efforts on clinical assessments, leading to a better use of human and financial resources, while also helping Member States benefit from each other's expertise. For companies, the proposal would move towards more predictable evidence generation requirements at the development stage.

This paper outlines EFPIA's views on the four pillars of EU HTA cooperation included in the Commission proposal. EFPIA, its national associations and member companies look forward to continuing the dialogue with all stakeholders including patients, healthcare professionals and policy-makers to ensure that a final text facilitates our common objective to improve the availability of innovative health solutions to patients across Europe.

1. Joint Clinical Assessments

1.1. Scope of the proposal

Building on the extensive experience collected over the past years of collaboration, Europe now has a unique opportunity to assess a common set of clinical evidence through the proposed Member State-driven coordination mechanism. EFPIA therefore supports the scope of the Commission proposal, limited to a joint clinical assessment at European level for medicinal products subject to the centralised marketing authorisation procedure. Member States retain their national competence for performing country-specific appraisal and coming to a decision on pricing and reimbursement.

The clinical assessment is a comparative evaluation on the available evidence of a medicine at the moment of the submission for joint clinical assessment. For example, the evaluation will look at how this new medicine compares with existing treatment options. Member States will use this factual clinical assessment when they perform their national appraisal[1], which leads to a benefit rating tailored to the country-specific context. This rating will then inform national pricing and reimbursement decisions.

[1] Such as: burden of disease, health care system architecture, ethical imperatives and equity considerations, relative costs and affordability, patterns of medicines usage, market structure and distribution-chain related components


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In order to avoid delays in the joint clinical assessment, it will be important to clearly define what evidence is needed for the joint clinical assessment. EFPIA believes that the evidence to be used should be the data available at the time of the submission for joint clinical assessment.

Vaccines are treated the same as medicines in the regulatory framework, which means that the Commission's HTA proposal covers also vaccines. However, many EU countries do not undertake HTA evaluations for vaccines, but rather use immunization recommendations issued by National Immunization Technical Groups which advise national governments on optimal vaccination policies. Therefore, coordination between all stakeholders (HTA bodies/experts and NITAGs) that play a role in the joint clinical assessment of vaccines and vaccination programs in EU Member States is crucial. In addition, there is a need to adapt the framework of joint clinical assessment to vaccines to take into account the vaccines preventive nature that brings benefits to individuals and populations over a long time horizon.

1.2. Mandatory uptake

EFPIA views it as critical that the EU clinical assessment replaces the equivalent step in the national assessment process if the Regulation's core objectives of reducing fragmentation and duplication are to be achieved. Therefore EFPIA strongly supports that the use of a joint clinical assessment in a national process of HTA be mandatory. Industry is willing to accept that joint clinical assessments apply to all new centrally approved medicinal products and new indications of centrally approved medicinal products, as a reciprocal precondition for the mandatory use of joint clinical assessments by Member States. This reciprocal approach is critical to ensure a properly functioning system, since it guarantees that the joint clinical assessment reports will be used by Member States and do not lead to further duplication.

The added value of the proposed European joint clinical assessment is that it centralises the scientific part of the overall HTA process by replacing a multitude of national and even regional clinical assessments. The safeguards included in the Commission proposal through the mandatory use in a national HTA guarantee that there is no duplication at Member State level. It facilitates the objective for internal market harmonisation and it avoids today's problems with market access distortions, duplication of work and patient access delays. The proposal does not prescribe any type of binding nature of a national HTA for pricing and reimbursement decisions. This continues to be fully up to each Member State to determine.

Specifically, during the transition phase (after the date of application) EFPIA considers as most important that Member States participating in joint clinical assessments are obliged to apply the resulting reports and to not repeat the clinical assessment at national level. Those Member States which delay their participation in the joint work should also not participate in any activity that aims at prioritising medicines for these activities during the transitional period e.g. the identification of Emerging Health Technologies. In addition, Member States delaying their participation should be obliged to use the harmonised rules for their own assessments from the date of application onwards. This obligation will enable more predictable clinical assessments across all EU Member States already during the transitional period, and incentivise Member States to opt in the joint work programme early on.

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1.3. Appeal mechanism

An appeal mechanism for companies is missing in the proposal, which in most cases is an available recourse in national HTA systems. Given that the joint clinical assessment is to be the basis of subsequent national decision making, an opportunity should be given for an independent review of the assessment, if significant discrepancies exist in the interpretation of the evidence before the report is 'passed on' for use in Member States.

1.4. Quality criteria and timeliness of the reports

EFPIA believes that the joint clinical assessment needs to be reliable, transparent, and consistent. The required standards to achieve this need to be defined in tertiary legislation and should be based on a clear list of general criteria in the primary regulation that is currently missing. EFPIA proposes that the following principles be considered for inclusion in the primary legislation:

- Procedural rules and clinical assessment methodology should be in line with agreed best practices and clearly build on EUnetHTA methodological guidelines
- The best available evidence at time of joint clinical assessment should be considered (including acceptance of clinical study designs as agreed for the regulatory process)
- Advances in science should be taken into consideration
- The legislation should propose a clear framework around process timeline, including a maximum timeline with clearly defined clock stops and the necessity of a scoping meeting. The joint clinical assessment reports should be published at the time of the Commission positive decision on marketing authorisation in order to avoid any delays in access to medicines. At the same time, the legislation should allow for flexibility in the system to adapt over time, based on evolving science and patient needs.
- Confidential data should be protected by confidentiality agreements

The Commission proposal ensures that the regulatory process and the process for joint clinical assessments remain distinct. EFPIA is supportive of this approach as the two processes have different purposes[2]. However, EFPIA acknowledges the potential synergies through increased levels of information sharing and better alignment of procedure timelines and key milestones for the proposed joint clinical assessments and the centralized marketing authorization for medicinal products, which should run in parallel.

2. Joint scientific consultations

The inclusion of joint scientific consultations in the proposal is a critical element to achieve its key objective of greater alignment in Europe on clinical evidence generation requirements. EFPIA welcomes the opportunity for pharmaceutical companies to request a joint scientific consultation (in parallel with the EMA) in order to discuss early in the development process of a medicine clinical data requirements for the regulatory approval process and joint clinical assessment, while also facilitating the selection of the relevant comparator(s) for both regulatory approval and HTA purposes.

[2] The joint clinical assessment should not repeat the regulatory assessment with regard to efficacy and safety for the purpose of establishing the positive risk/benefit ratio required for the regulatory approval.

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EFPIA believes that proposed scope for the joint clinical assessments has to be the same for the joint scientific consultations. All medicines that eventually have to undergo joint clinical assessments should have the possibility of being submitted to a joint scientific consultation. In this respect, EFPIA would argue against a selection process that would effectively exclude medicines from such an early scientific consultation, thereby reducing the ability for a manufacturer to anticipate and meet the clinical evidence requirements that the member states will set at time of the Joint Clinical Assessment. Therefore, EFPIA asks to ensure that the envisaged joint scientific consultation process is adequately resourced from the beginning.

While the rules and documentation necessary for the joint scientific consultation are to be defined in tertiary legislation, EFPIA believes that a list of criteria establishing joint work should be included in the primary legislation. EFPIA recommends considering the following principles when developing the criteria for joint scientific considerations:

- Development of common procedures should be based on existing frameworks and principles developed by EUnetHTA and should be clearly stated in the Regulation
- Clear description of the content of the information, data and evidence required from the manufacturer
- Clear timelines for the process duration; EFPIA believes that the timeline for the final joint scientific consultation report should be ideally harmonised with the regulatory process, meaning that that it should be available at the latest, 100 days following the start of the preparation of the report
- Clear definition of what is considered additional evidence that could justify a delay in the preparation of the joint scientific consultation report
- Clear rules for determining the stakeholders to be consulted and the input expected from them.

3. Emerging health technologies

EFPIA considers the report on emerging health technologies as a reasonable tool to act as key input for the annual work programs during the transition period. It is unclear why horizon scanning would be needed once the process is fully operational, since by default all centrally authorized products and new indications would undergo joint clinical assessments. Prioritisation of products to be selected for the joint clinical assessments based on the conclusions of the report on emerging health technologies cannot be supported, if it leads to delayed access for “non-priority products.”

4. Voluntary cooperation

EFPIA is not supportive of including voluntary cooperation on non-clinical assessments for medicines in the proposed HTA Regulation. Non-clinical health technology assessments focus on evaluation criteria that are highly specific to the respective Member State context[3]. In line with the principle of subsidiarity and also for very practical reasons these aspects of HTA are best handled at Member State level. The inclusion of voluntary cooperation on HTA among the four pillars of the Regulation is likely to divert focus and scarce resources from the identified priority areas, namely joint clinical assessment and in particular joint scientific consultations.

[3] These include: burden of disease, health care system architecture, ethical imperatives and equity considerations, relative costs and affordability, patterns of medicines usage, market structure and distribution-chain related components

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