EUCOPE

European Confederation of Pharmaceutical Entrepreneurs AISBL

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EUCOPE'S STATEMENT ON EU HTA PORTUGUESE PRESIDENCY COMPROMISE

EUCOPE welcomes the important collaborative effort made by the Council of the EU to reach a compromise on the EU on a *Proposal for a Regulation on health technology assessment (HTA) and amending Directive 2011/24/EU*. However, we note that the direction of the current compromise is substantially changing central parts of the proposed Regulation.

We express our concerns regarding a number of provisions that might bring additional regulatory and administrative burden and **an added level of uncertainty for small to mid-size companies**.

To address these concerns, we call for the following aspects to be included in upcoming inter-institutional negotiations:

1. **Sufficient management of evidentiary uncertainties for OMPs and ATMPs to prevent delays in patients' access.**
2. **Mandatory uptake of joint clinical assessment at national level to reduce regulatory burden.**
3. **Joint scientific consultation for all manufacturers for increased predictability.**

1. SUFFICIENT MANAGEMENT OF EVIDENTIARY UNCERTAINTIES

EUCOPE welcomed the initial Regulation Proposal put forward by the European Commission in 2018 and its aim of contributing to increased predictability, faster time to market and overall reducing the regulatory burden and compliance costs by proposing a mandatory use of the joint clinical assessment reports by Member States.

Since the beginning of the legislative procedure EUCOPE raised the need for adjustments to provide for a sufficient level of flexibility in specific cases, such as **for Orphan Medicinal Products (OMPs) and Advanced Therapy Medicinal Products (ATMPs)**.

A "one-size-fits all" methodology not accounting for specificities of therapies developed for small patient populations would inadvertently cause considerable delays in assessments and ultimately hinder patients' access to those medicines across the EU. **We therefore reiterate the need for a flexible regulatory framework that can manage evidential uncertainty in specific cases where appropriate**.

2. MANDATORY UPTAKE OF JOINT CLINICAL ASSESSMENT AT NATIONAL LEVEL

Article 8 of the original Commission proposal made joint clinical assessments **binding for Member States**, i.e. they were not to carry out their own clinical assessments and "**apply joint clinical reports**". The Council compromise, however, proposes that Member States draw their own conclusions on the clinical value and use the parts of the reports they consider relevant. Further, where Member States were not to carry out their own clinical assessment on a health technology subject to a joint assessment, the compromise proposal merely states that evidence submitted in **joint clinical assessments** at EU level shall not be requested again at Member State level.

Member States would, *inter alia*, be free to draw their own value judgments and conclusions on the overall clinical benefit and shall only give due consideration to the methodological guidance established by the Regulation. **Since the Council compromise proposal does not allow for a full binding effect on Member States**, the compromise might in fact **result in duplication of work**, posing continued burdens on companies. The heterogeneity of HTA processes across Member States require significant administrative and financial resources and time from developers and adding another dimension to the existing HTA processes without adequate harmonisation will further increase the required resources as additional clinical assessments could be demanded at Member State level. This would be **especially cumbersome for small to mid-size companies**, that lack the resources to face both the uncertainty and the additional workload that the process might bring.

3. JOINT SCIENTIFIC CONSULTATION FOR ALL MANUFACTURERS

The Council proposal in its current state provides selection criteria for access to joint scientific consultation based on application by the manufacturer. Not every manufacturer that enters into the evaluation process and seeks scientific advice will therefore receive it. For the pharmaceutical industry, **scientific advice is a crucial procedural step** and essential to all future HTA cooperation at EU level.

Limiting early consultations within the framework of the EU procedure will reduce transparency and predictability for the industry, reduce the quality of Joint EU HTA procedures and can lead to duplication of work. Lack of scientific consultation could give rise to situations where developers and the Coordination Group responsible for the joint clinical assessment do not align on relevant methodologies, evidence and endpoints. This, in turn, would increase the likelihood of a discontinued joint clinical assessment and lead to duplication of work and lost time as the joint assessment would have to be restarted at a later date with an updated dossier.