Feedback from ESMO on the proposed Implementing Act on Joint Clinical Assessments of Medicinal Products

Representing more than 35,000 oncology professionals from over 172 countries, the European Society for Medical Oncology (ESMO) welcomes the proposal for the Implementing Act on Joint Clinical Assessments (JCAs) of medicinal products as well as the other ongoing preparations for the implementation of the Health Technology Assessment (HTA) Regulation.

Robust HTA is of crucial importance for improving patient access to new and innovative treatments, including treatments for cancer. The HTA Regulation, by introducing JCAs enabling the assessment of the clinical aspects of new health technologies, holds enormous potential to enhance and reinforce HTA across the EU Member States, provided that the healthcare professional community is well involved in each step of the implementation. ESMO, as one of the largest societies pooling oncology expertise, is committed to help deliver the benefits of the HTA Regulation.

ESMO considers the proposal for the Implementing Act to be a robust first draft of the text, clearly specifying the key procedural steps and timelines of the JCA process for medicinal products. Whilst welcoming the proposal, we believe that it is important that the tools and resources developed by the medical oncology community are used in settings where these can be beneficial, and that healthcare professionals - including medical oncologists - are involved and consulted during JCAs.

As such, ESMO would like to highlight the following:

- **1. ESMO-Magnitude of Clinical Benefit Scale (ESMO-MCBS)**: Launched in 2015, the ESMO-MCBS aims to facilitate improved decision-making regarding the value of anti-cancer therapies, promote the accessibility of these therapies and reduce inequity of access to high value cancer treatments. The ESMO-MCBS is a validated and reproducible scale that is applicable across the full range of solid tumours and, since 2023, also in haematological malignancies (ESMO-MCBS:H). It is a dynamic tool, and its criteria are revised on a regular basis, to address the rapid evolving landscape of new treatment development and regulatory decisions;

  The ESMO-MCBS utilises a structured, reproducible and robustly validated approach to data analysis that reduces the risk of idiosyncratic or biased evaluation. It is used to support HTA decision-making regarding oncology medicines in many countries, both in Europe and beyond. Of note, in a retrospective analysis by the London School of Economics (LSE) of HTA decisions regarding oncology medicines by 7 HTA bodies, a high ESMO-MCBS appraisal was the foremost predictor of a positive HTA appraisal, suggesting that it is a tool with potential utility in HTA exercises;

Given its value for supporting the prioritisation of medicines for evaluation, we believe that it would be of benefit for patients with cancer if the ESMO-MCBS is used as a supportive tool for the new joint HTA processes to signal new compounds with a large magnitude of benefit that should require immediate attention from health authorities for accelerated review and coverage-reimbursement decisions to prompt patient access;

We also consider it important that sufficient JCAs will be conducted for health technologies that were developed using single-arm trials, given their importance for the development of treatments for rare cancers. The ESMO-MCBS has been designed to help score single-arm trials;

ESMO will soon be launching a scoring tool, which will automatically execute the rules of ESMO-MCBS based on the information inserted and submitted by the user to generate an ESMO-MCBS score. This could be used in the JCAs (as well as by regulatory agencies and industry) to simulate the ESMO-MCBS scoring during trials and before a medicine's approval;

- **2. ESMO Clinical Practice Guidelines**: The ESMO Clinical Practice Guidelines, developed and reviewed by leading experts and based on the findings of evidence-based medicine, provide medical oncologists with a set of recommendations for optimal patient care. We believe that the ESMO Clinical Practice Guidelines can be used as a benchmark ensuring that patients receive appropriate treatment and care. For instance, ESMO guidelines are used as the EU standard for cancer treatment in several regulatory reports, and acknowledged as a reference to identify 'best practice' within several European Public Assessment Reports (EPARs);

- **3. Involvement of healthcare professionals in JCAs**: We consider it essential that healthcare professionals are among the experts to be consulted during JCAs (Article 6, Paragraph 1). For JCAs of antineoplastic therapies, medical oncologists should be involved in a consultative role. Moreover, when compiling lists of relevant experts for JCAs (Article 6, Paragraph 2), professional societies like ESMO should be amongst the groups to be consulted by the HTA secretariat;

- **4. Alignment with the EMA**: Further information should be provided on the steps to be taken in cases where the outcomes of JCAs diverge substantially from the conclusions of the benefit/risk evaluations done by the European Medicines Agency (EMA) in relation to marketing authorisations;

- **5. Information retrieval**: As part of the information retrieval process set out in point 4.2.1. of Annex I, patient reported outcome data (including quality of life assessment) as well as the statistical analysis plan will have to be included in the sources of information that are to be considered in the retrieval process;

- **6. Information on treatment alternatives**: Annex II sets out the proposed template for JCA reports. We believe that information on context and country-specific treatment alternatives, including medicines that are

already available for the concerned indication, should be part of the overview of the medical condition (point 2.1.);

- **7. Included studies**: As to the included studies covered in point 4.2.1. of Annex II, it is important that information on the study design also addresses primary, secondary and exploratory endpoints. Moreover, characteristics of the study interventions should indicate how often patient reported outcome metrics were included;

- **8. JCA Subgroup**: Further clarity should be provided on the composition and ways of working of the JCA Subgroup, especially as to the selection of experts from professional societies like ESMO who are to be consulted during JCAs.

ESMO stands ready to collaborate with the EU institutions and the Member State HTA Coordination Group on the development of the Implementing Act for JCAs of medicinal products and offers to mobilise its expert groups and network of medical oncologists to support a successful implementation of the HTA Regulation.