EFA response to the stakeholder consultation on the proposed Health Technology Assessment (HTA) Implementing Regulation on Joint Clinical Assessment for Medicinal Products

The European Federation of Allergy and Airways Diseases Patients' Associations (EFA) is the voice of 200 million people living with allergy, asthma, and chronic obstructive pulmonary disease (COPD) in Europe. We bring together 45 national associations from 26 countries and channel their knowledge and demands to the European institutions. We connect European stakeholders to ignite change and bridge the policy gaps on allergy and airways diseases so that patients live uncompromised lives, have the right and access to the best quality care and a safe environment and are involved in all decisions on their health.

EFA is a full member of the European Patients' Forum (EPF), and has contributed to the EPF's positions on HTA, fully supporting their response to this consultation, and specifically the points related to the need for strengthened and refined patient contributions, and integration of patient experience studies, such as core outcome sets and patient preference studies co-designed and co-developed with patients, to complement patient involvement.

EFA welcomes the adoption of the Health Technology Assessment Regulation (EU) 2021/2282 (HTAR), which contributes to improving patients access to innovative health technologies in the area of health, such as medicines and certain medical devices.

Since 2016, EFA has been deeply involved in the development of the Health Technology Assessment (HTA) legislative framework, bringing the allergy, atopic eczema, asthma and chronic obstructive pulmonary diseases (COPD) patients' perspective to inform the setup of HTA cooperation at EU level. We have joined the new HTA Stakeholder Network, launched as part of the implementation plans of the HTA Regulation, as well as the different stakeholder workshops organised by DG SANTE and leading to this draft Implementing Regulation on Joint Clinical Assessment (JCA) for Medicinal Products. We highly welcome the opportunity to provide written feedback on behalf of allergy, atopic eczema, asthma and COPD patients' associations across Europe.

Selection and involvement of patients, patient organisations, carers, and young patients

EFA welcomes the structural involvement of patients, clinical experts, and other relevant experts at the different stages of the joint clinical assessment process together with the JCA subgroup. We applaud the proposal to gather individual patients from the different European expert resources and lists that already exist, as proposed in Article 6 of the draft implementing Regulation.

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However, it is of utmost importance that the proposed text of Article 6 on the selection of patients, clinical experts and other relevant experts scopes not only individual expert patients with a diagnosis, **but also patient experts from European and national patients' organisations**, and carers, as the latter might be crucial in processes when the medicinal product assessed is intended for paediatric patients, elderly populations or people with disabilities not allowing for effective patient participation in the procedure.

As such, **EFA proposes the following recommendations to Article 6 of the proposed draft implementing Regulation** (in blue):

> (Article 6) "1. The JCA subgroup shall specify, for each particular joint clinical assessment, the disease, the therapeutic area concerned and other specific expertise, based on which the HTA secretariat shall identify patients, **carers, patient experts,** clinical experts and other relevant experts to be consulted during that joint clinical assessment.
>
> 2. The HTA secretariat shall compile a list of relevant patients, **carers, patient experts,** clinical experts, and where necessary, other relevant experts, in consultation with the JCA Subgroup and the appointed assessor and co-assessor. When compiling the list, the HTA secretariat ~~may~~ **shall** consult: [...]"

Justification: Patients organisations gather disease communities and therefore sustain wide sources of European and national expertise and lived experiences, and can provide with a diversified network of individual patients, representatives and patient experts for various but also very specific disease areas, such as it is the case for the respiratory patients' community at EFA, who can provide expertise, for example, on asthma, but also its subtypes (e.g. allergic asthma, nonallergic asthma, childhood asthma, severe asthma, etc.). On EU HTA procedures, it is crucial to ensure a solid and informed European patient perspective. While the interaction with **individual expert patients and clinicians** is crucial for the JCA for medicinal products, **EFA strongly recommends including within the individuals to be scoped patient experts, those stemming from and supported by European and national patients' organisations,** as a relevant category next to clinical experts. Moreover, we strongly encourage that the process allows for "carers" to represent patients, especially in processes when the medicinal product assessed is intended for paediatric patients, elderly or people with disabilities. This EFA proposal entails that **patient experts and carers should be included at all stages in the process** (e.g. Articles 7, 9, 10, 14, 15).

> (New 3). **The HTA secretariat shall seek the involvement of patients under 18 years old to provide input where appropriate in JCA processes relevant to the paediatric population.**

Justification: EFA proposes that, in the case of JCA applications targeted to the paediatric population, the HTA secretariat gives preference to children and adolescent patient experts and their carers. Children are alarmingly underrepresented in the medicines development, authorisation and HTA procedures, and their engagement should be encouraged. Children and adolescents have a unique perspective that should be sought. EFA recommends adding a specific article in the Implementing Act, guided by a set of principles on the involvement of young patients similar to the way in which the

European Medicines Agency (EMA) conducts their activities, with parental consent required and assessment of feasibility of such involvement.[1]

> (New 7). **Experts consulted during the joint clinical assessment shall be entitled to compensation for their contribution to the process proportionate to their required preparation, contribution, travel, and attendance in meetings.**

Justification: Experts who dedicate their time and resources to be involved in the joint clinical assessment process, especially patients, patient experts and carers, are volunteers who will be required to spend a considerable amount of time to participate in EU JCA. The involvement of patients and experts is very important in the overall assessment process, and it should be reasonably compensated, in order to increase patient involvement and ensure independence of experts. Therefore, EFA recommends that patients and experts involved in the joint clinical assessment are financially reimbursed and compensated for their work and contribution during the joint clinical assessment.[2]

> **(Article 8)** ~~At any time during the joint clinical assessment,~~ the JCA Subgroup ~~may~~ **must** seek input on the disease and therapeutic area from patient organisations, healthcare professional organisations or clinical and learned societies via the members of the HTA stakeholder network.

Justification: Patient organisations' involvement in the regulatory process is of utmost importance as they are great sources of data and expertise due to their structured patient networks and established governance and transparency mechanisms, providing a unique overarching perspective and knowledge from their communities in the decision-making process, on points such as quality of life, patient satisfaction and adherence to treatment. As such, patient organisations can play a pivotal role, and must therefore be consulted during the joint clinical assessment, while having their input strengthened and streamlined throughout the joint clinical assessment process.

Publication of joint clinical assessment & summary reports

EFA welcomes the steps taken in the context of the HTAR to make information widely available and accessible to the public about the results of joint clinical assessments, as laid down in Article 12 of HTAR. In order to ensure accessibility and health literacy of the overall population, **EFA proposes the following recommendation to Article 15 of the draft Implementing Regulation:**

> (New 6). **Following the endorsement of the reports by the Coordination Group in accordance with the procedure laid down in Article 12 (3) of Regulation (EU) 2021/2282, the final summary reports shall be made available in a timely manner in all EU official languages on the IT platform referred to in Article 30(1), point (a) of Regulation (EU) 2021/2282.**

[1] EMA, Principles on the involvement of young patients/consumers within EMA activities, May 2017, available at https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/principles-involvement-young-patientsconsumers-within-ema-activities_en.pdf

[2] EMA, Rules for reimbursement of expenses for delegates attending meetings, March 2023, available at https://www.ema.europa.eu/en/documents/other/rules-reimbursement-expenses-delegates-attending-meetings-effect-17-march-2023_en.pdf

Justification: Given the EU wide scope of the JCA, it is essential that summary reports are published in all official languages of the EU, to ensure appropriate access to information to all EU citizens independently of their language. Not translating the summary report will continue increasing health inequalities on access to care and to health information.

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