Patient organisations' joint statement on the first Joint Clinical Assessment (JCA) report

15 patient organisations from the HTA Stakeholder Network welcome the publication of the Joint Clinical Assessment (JCA) of tovorafenib, **marking an important milestone under the EU Health Technology Assessment Regulation. As patient organisations, we have been heavily involved in the process,** including by supporting the identification of patients and carers who could contribute to the assessments. **The publication of this first JCA is therefore an important step towards a more coordinated and patient-centred European HTA system.**

In particular, we recognise the efforts made to involve patients and carers in the assessment process. The report confirms that the carer was invited to provide input on the assessment scope and to comment on the draft JCA and summary reports. This is a positive step towards ensuring that the experiences and priorities of patients, carers and families are reflected in European health technology assessments.

At the same time, this first experience shows that patient involvement in JCAs can and should be further strengthened. **Drawing on our practical experience and expertise in patient involvement, we would like to make recommendations on how to ensure effective patient involvement in JCAs. Such involvement should provide valuable evidence on the lived experience of living a disease, navigating treatments and understanding the potential impact of health technologies on their quality of life.**

- **First, patients and carers should be involved from the very beginning of the process, including in the preparatory phase**. While we welcome the opportunity given to the carer to comment on the assessment scope, earlier involvement would allow patient perspectives to have greater influence on the development of the scope proposal and participation in the PICO survey, leading to the identification of outcomes that matter most to patients and families**.**

- **Second, broader patient representation in future processes should be considered**. While individual patient or carer perspectives are valuable, a single voice may not be fully representative of the wider community. Effective representation requires not only lived experience, but also the skills, knowledge of clinical trial data, treatments and health systems, as well as the capacity to understand, contextualise, and convey the perspectives and needs of the broader patient community. **As part of this discussion, the role of patient organisations should be further recognised and strengthened**. While the HTA Secretariat consults patient organisations when compiling a list of relevant patients and carers, their contribution should extend beyond this initial stage of the JCA process. Patient organisation involvement in HTA is a standard part of many HTA systems globally. Patient organisations, with the right tools in place, can indeed play a pivotal role in bringing broader community perspectives and the collective expertise at the very onset and during the whole process.

- **Third, greater transparency is needed on how the input of patient representatives is used.** While the report confirms that input was collected and "considered", it provides limited information on how this input influenced the assessment process, the choice of outcomes, the interpretation of evidence or the final conclusions. Patients and carers should receive clear, concise and understandable feedback on how their contributions have been taken into account, which should be included in the final report. This feedback can support continuous improvement by helping patients, and patient organisations conducting training sessions for their communities, understand how to provide effective input in future JCAs and identify where their insights can be most valuable.

- **Fourth, the assessment highlights the continuing challenge of limited evidence on outcomes that matter most to patients and families, including health-related quality of life, patient-reported symptoms, functional outcomes, and treatment burden.** As new therapies are evaluated, evidence generation must better capture these outcomes so that decisions reflect not only clinical endpoints, but also the realities of living with the condition and undergoing treatment. **The inclusion of patient experience data at all stages of medicine development and regulatory decision-making** could ensure that new medicines address the outcomes and preferences that matter to patients and therefore be particularly relevant to the HTA process.

- **Finally, JCA findings must be communicated in a way that is accessible to patients, carers and the wider public.** The current summary report remains difficult to understand for people who are not familiar with HTA or the JCA process. Future summary reports should therefore be written in clear, lay language and should highlight information that is relevant to affected patient communities. **Besides, given the European scope of the JCA, the summary reports must be published in all official EU languages to ensure adequate access to information for all EU citizens, regardless of their language**. As part of this reflection and for future discussions, the summary report could also be reviewed beforehand by patient organisations to ensure that the information is in lay language. The provision of translations is essential for promoting health equity, as it enables patients, carers and patient organisations across Europe to fully understand and engage with the assessment reports. The lack of translation of the summary report can exacerbate inequalities in access to healthcare and health information. Translations further support national health authorities and patient organisations in their local dissemination efforts, facilitating more effective communication at the national level.

We would also like to bring a broader reflection to the ongoing discussions regarding disease areas where the generation of robust comparative evidence is per se challenging and where important uncertainties may arise. **In this context, innovative methodologies that can help address these evidence gaps while maintaining scientific rigour should be considered.** Further methodological discussion and future methodological guidance for HTDs on how to integrate additional evidence sources could help strengthen future assessments in situations where robust comparative data cannot realistically be generated.

As implementation of the EU HTA Regulation continues, patient organisations look forward to working with all stakeholders to ensure that patient involvement becomes meaningful, transparent and impactful throughout the assessment process. We celebrate this important milestone, while recognising that continued efforts are needed to improve the process. We now call on national HTA bodies to fully play their part, ensuring that national-level appraisal and decisions of reimbursement build efficiently on the JCAs.

Signatures:

European Patients’ Forum (EPF)

European Liver Patients’ Association (ELPA)

Cancer Patients Europe (CPE)

International Diabetes Federation Europe (IDFE)

Global Heart Hub (GHH)

European Federation of Psoriasis Movements (EUROPSO)

Europa Donna – The European Breast Cancer Coalition

Childhood Cancer International Europe (CCI-E)

European Pulmonary Fibrosis Federation (EU-PFF)

Myeloma Patients Europe (MPE)

International Patient Organisation for Primary Immunodeficiencies (IPOPI)

European Federation of Allergy and Airway Diseases Patients' Organisations (EFA)

European Institute of Women's Health (EIWH)

SMA Europe

European Haemophilia Consortium (EHC)