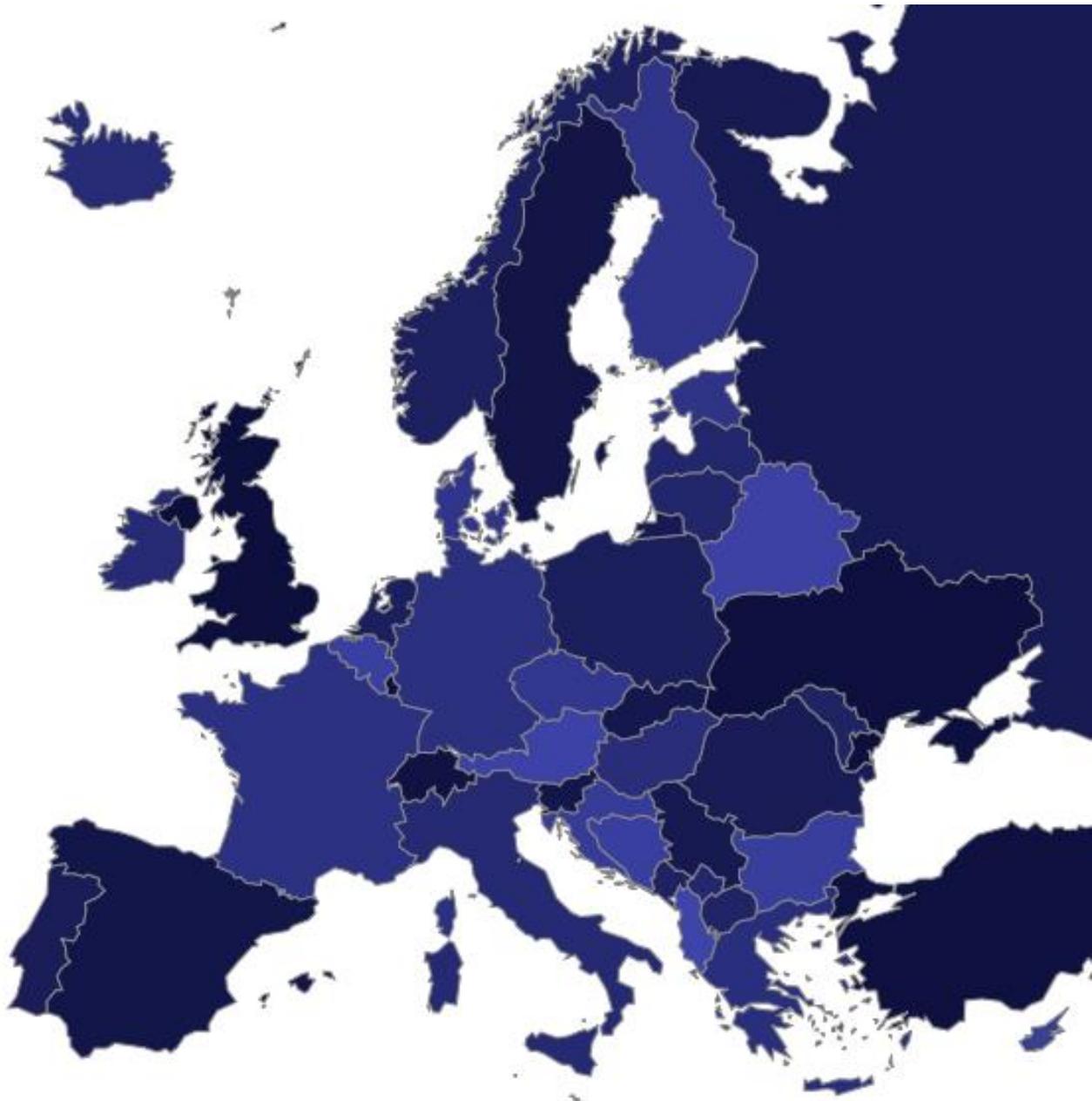


Interinstitutional negotiations on the Proposal for a Regulation on Health Technology Assessment and amending Directive 2011/24/EU

*HTA experts must be involved in the design of the clinical trials used for marketing
authorisation application*



June 2021

The [Access to Medicines Task Force](#) of the Association of European Cancer Leagues (ECL) and the [Anticancer Fund](#) warmly welcomed the decision of EU Ambassadors to move forward with the [latest Health Technology Assessment \(HTA\) proposal for a Regulation](#) which paves the way towards a more sustainable cooperation model. We were also pleased to see the important role given to affordability, availability and accessibility of medicines in the [draft Council conclusions on Access to medicines and medical devices for a Stronger and Resilient EU](#).

Half of oncology drugs approved in recent years have had only marginal clinical¹ and quality-of-life benefits² for patients, despite their promised added value before entering the market. For this reason, as reported in a previous [ECLs' statement](#), we are particularly delighted to see that joint clinical assessments would be starting from cancer drugs. ECL and the Anticancer Fund strongly support **a modern and effective regulatory system for HTA and clinical trials to ensure patients' safety while providing timely access to the latest medical technologies with proven clinical benefit.**

For this to happen, we insist that **7 Key Priorities be considered going forward:**

1. Establishing mandatory involvement of HTA bodies, payers, and patients since the early stages of clinical trials' design ahead of the marketing authorisation request;
2. Fostering the application of comparative trials in line with the [Declaration of Helsinki on human experimentation](#) to gather robust health data and reflect on the added value of the new health technology. The comparator should be the best standard of care available;
3. Unveiling the potential of disease-registries by including treatment outcomes and patient-reported outcomes (PROs) and generously investing in the infrastructure of cancer registries;
4. Ensuring that the collection and storage of data falls within the remit of public authorities;
5. Embedding the effective collection, storage, and analysis of PROs already at the clinical trials' design stage;
6. Leveraging the [European Health Data Space](#) to effectively collect, store, and use real-world evidence (RWE) and real-world data (RWD) for post-marketing authorisation efficacy studies, starting from the lessons that can be learnt with the European Reference Networks;
7. Allowing HTA bodies and non-profit parties (namely, academia and non-profit researchers) to access clinical trials' results and patient-level data over every clinical trial phase, including unpublished data from failed trials as already highlighted in a joint [ECL-EORTC statement on EMA's transparency policy](#), to enable high-quality assessments.

¹ New drugs: where did we go wrong and what can we do better? Available here: <https://www.bmj.com/content/366/bmj.l4340>

² Availability of evidence of benefits on overall survival and quality of life of cancer drugs approved by European Medicines Agency: retrospective cohort study of drug approvals 2009-13. Available here: <https://doi.org/10.1136/bmj.l4530>

Based on the articles included in the latest draft available of the proposal for a Regulation on HTA, we call for:

Adding clear definitions of “real-world data” and “real-world evidence” within Article 2 on ‘Definition’:

Real-world data and evidence, in fact, would affect the purpose and expected outcomes coming from the use of clinical data and patient-reported outcomes after marketing authorisation. Together with comparative clinical trials, they are complementary and informative to make evidence-based decisions, both in the regulatory and legislative fields. **We support the suggestion that the Council of the EU has put forward to the European Commission and Member States to explore the possibility of establishing an EU Real-World data collection and evidence generation action plan.**

Editing Article 8 on ‘Member States’ Rights and Obligations’ as follows:

When carrying out a national health technology assessment on a health technology for which reports have been published or for which a joint clinical assessment has been initiated, Member States shall:

- **Incorporate the published reports and all other information available on the IT platform referred to in Article 27, including the statement of discontinuation pursuant to Article 6b(6), concerning that joint clinical assessment in their health technology assessments at Member State level.**
- **Following this, the Member States should report to what extent the information has been taken into consideration and disclose the reason why the information has not been used, if the case. This shall not affect Member States' competence to draw their conclusions on the overall clinical added value of a health technology in the context of their specific healthcare system and to consider the parts of the reports relevant in this context.**

Clarifying the inclusion and exclusion criteria of Article 19 on ‘Voluntary Cooperation’:

Article 19 encompasses health technologies other than medicinal products, medical devices or in vitro diagnostic medical devices. Considering that more than 90% of products in the pipeline are biologics and small molecules and that cell, gene, and nucleotide therapies in clinical development continues to increase³, it is important to fully clarify the obligations of Member States and health developers in this regard. **We totally support the Council of the EU’s views regarding developing cooperation within the Network of Competent Authorities on Pricing and Reimbursement (NCAPR) to share information on pricing and reimbursement initiatives supervised by EU and/or intergovernmental organisations, such as the WHO and OECD.** The Slovenian Presidency of the Council may have an instrumental role to steer this initiative.

³EFPIA Pipeline Review 2021 Update https://www.efpia.eu/media/602564/iqvia_efpia_pipeline-review_final.pdf

Conclusions

The proposal for a Regulation on HTA is a critical file from a regulatory, economic, and policy perspective. It is essential that it reflects the scientific developments and the challenges that new health technologies coming to the market pose on national healthcare systems' budgets. An inclusive and transparent system would be beneficial to overcome challenges in the years to come.

In line with the [opinion of the European Economic and Social Committee](#), **we have some concerns about the timelines set for implementation and especially the delayed application of three years**. For the benefit of patients and cost-effectiveness, this should be shortened. As already reported in [ECL's reaction statement to the latest publicly available HTA text](#), the flexibility allowed in the uptake of the joint clinical assessment may undermine the success of this long-awaited step forward.

To conclude, we support the [Council's recognition](#) of the pivotal role that a suitable, existing platform, such as registries, can play in comparing treatment options with usual care in a routine patient population with patient-relevant outcomes, real-world evidence, and real-world data and in improving patient care.

About the Association of European Cancer Leagues (ECL)

ECL is a non-profit organisation that unites, supports and represents cancer leagues across the WHO European region. Established in 1980 by prominent NGOs and experts, and based in Brussels (Belgium), ECL provides a voice and forum for cancer leagues to collaborate and share knowledge, primarily in the areas of cancer prevention, tobacco control, access to medicines and patient support, and create opportunities to advocate for these issues at the EU level and beyond.

About Anticancer Fund

The Anticancer Fund is a Belgian non-profit organisation with an international scope. In 2018, the Anticancer Fund became a Foundation of Public Utility under Belgian law, an official recognition that the fund's effort is used to achieve a well-defined altruistic goal: investing in promising cancer treatments, putting patients' needs first.

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