The anniversary series

The Regulation on Health Technology Assessment: a new building block towards a European Health Union

*On 13 December 2021, the Regulation on Health Technology Assessment[1] ("**HTA**") was adopted following its approval in the second reading by the European Parliament. The Regulation aims at boosting cooperation among European Member States in the area of HTA by providing a permanent and sustainable framework for the development of common HTA tools, methodologies, and procedures.*

HTA is defined in Article 2 as "a multidisciplinary process that summarises information about the medical, patient and social aspects and the economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased and robust manner". Essentially, it is a tool for evaluating the clinical added value and cost-effectiveness of a new health technology compared to existing alternatives, which is used to inform healthcare policy, pricing, and reimbursement decisions made by national health authorities. Health technologies include medicines, medical devices, assistive technologies, techniques and procedures developed to solve health problems and improve the quality of life.[2]

HTAs are not to be confused with clinical trials, which are research studies conducted by or on behalf of the developers of health technologies (mostly pharmaceutical companies) to test the safety and efficacy of their inventions and to produce the clinical evidence needed to apply for marketing authorisation. While HTAs rely heavily on the clinical evidence generated in clinical trials, they go further and compare the clinical properties of the new health technology to those of the current standard of care or of less costly alternatives. This means that, aside from clinical safety and efficacy, social, ethical, and economic dimensions may also have a role to play in the analysis. As the main purpose of HTAs is to inform national decision-making in the health area (mainly as regards reimbursement policy), they are usually conducted by public bodies on behalf of the government. Although EU cooperation on HTA is not entirely new, legislation on the processes and methodologies used to carry out such assessments, as well as the assessments themselves, are still highly fragmented across Europe today. In this article, we will discuss the new elements of cooperation introduced by the Regulation and whether these represent an important step forward in terms of supporting joint work between Member States on HTA.

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[1] Regulation (EU) 2021/2282 of the European Parliament and of the Council of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU.

[2] Definition used by the WHO: https://www.euro.who.int/en/health-topics/Health-systems/health-technologies-and-medicines/health-technologies.

A promising new piece of EU legislation

As a key deliverable of the EU Pharmaceutical Strategy[3], the new Regulation is considered to be a crucial step towards a stronger European Health Union[4], catering to some of its main objectives such as the fulfilment of unmet medical needs, and the improvement of patients' access to affordable health technologies. Indeed, increased collaboration between national HTA bodies in Europe enables them to join forces in terms of resources and expertise, and it saves them from duplicating their efforts in the assessment of identical products. This will enhance the efficiency and quality of the assessments and make sure that they are based on consistent criteria. The higher level of predictability that comes. with this may benefit developers of health technologies and allow them to improve the design of their clinical trials, promoting the generation of evidence of high scientific quality. This will in turn support and speed up decisions of national health authorities to invest in the development of and to improve patient access to vital innovative health technologies that are safe, effective and cost-efficient. Such evidence-based decisions will optimise the value of public spending on health.

New elements of cooperation

How does the Regulation intend to deliver on its promises and objectives? First of all, the Regulation establishes a Member State Coordination Group on Health Technology Assessment ("***Coordination Group***"), which will function as a permanent body overseeing the cooperation of Member States on HTA at Union level. This Coordination Group will be composed of Member States' representatives, ideally members of national HTA authorities, and will be divided into four subgroups, each taking care of one of the main areas of joint work provided for in the Regulation. Areas of cooperation include (i) joint clinical assessments; (ii) joint scientific consultations; (iii) the identification of emerging health technologies; and (iv) the development of methodological and procedural guidance on the foregoing.

(i) Joint clinical assessments

The first, and most important area of joint work introduced by the Regulation focuses on the clinical aspects of HTA, i.e. the scientific evaluation of the technical characteristics of the new health technology and of its relative clinical safety and effectiveness compared to the state of the art. This involves questions like "Does the new technology work better, equally well or worse than existing alternatives?" and "What are its potential side effects and influence on the quality of life of the patient?". Since individual EU countries will continue to be responsible for assessing the non-clinical aspects of a new health technology, such as the economic, ethical, social, legal, and organisational concerns related to its use, one cannot really speak of a "joint HTA" at EU level. This can be explained by the fact that such non-clinical domains tend to be more closely related to the national context and less suited to joint assessment at EU level. Another way in which the scope of the joint work is limited relates to the type of health technologies covered by such joint clinical assessments. While national HTAs are generally carried out in relation to all possible types of health technologies, including medicinal products, medical equipment, medical and surgical procedures and methods for diagnosis, treatment, prevention and rehabilitation, joint clinical assessments carried out at EU level will only cover new medicinal products and high-risk medical devices (cf. Article 7(1) and Article 16(2)).

It is important to note that these new rules do not interfere with Member States' exclusive competence to draw conclusions on the overall clinical added value of new health technologies for their national healthcare system and to make pricing and reimbursement decisions, or any other decisions relating to the management of medical care, on that basis.

[3] A Pharmaceutical Strategy for Europe (2020), https://ec.europa.eu/health/sites/default/files/human-use/docs/pharma-strategy_report_en.pdf.

[4] https://ec.europa.eu/info/strategy/priorities-2019-2024/promoting-our-european-way-life/european-health-union_en

(ii) Joint scientific consultations

Secondly, the Coordination Group will engage in joint scientific consultations with developers of new health technologies to give advice on clinical trial designs that may generate evidence meeting the requirements of the joint clinical assessments. In contrast to the joint clinical assessment reports, the outcome document of joint scientific consultations (which outline the scientific recommendations made) shall not give rise to any legal effects on Member States, the Coordination Group or the health technology developer.

(iii) Horizon scanning

The Regulation also provides for the joint preparation of reports on emerging health technologies expected to have a major impact on patients, public health or healthcare systems, which will allow to identify such promising health technologies at an early stage, and help national health systems prepare for them (also called "horizon scanning").

(iv) Methodological and procedural guidance

Finally, common rules and methodologies for carrying out the above joint work will be developed at Union level by the Coordination Group.

In addition to these mandatory aspects of HTA cooperation, the Coordination Group may also be used to support and facilitate voluntary cooperation and exchange of information among Member States in other areas, such as non-clinical assessments and HTAs on health technologies other than medicinal products and high-risk medical devices.

A final element worthy of note is the Stakeholder Network which will be set up to enable regular dialogue between the Coordination Group and stakeholder organisations, in particular patient associations, consumer organisations, non-governmental organisations in the field of health, health technology developers, and health professionals.

The Regulation will enter into force in January 2022, twenty days after its publication in the Official Journal of the European Union, but will only apply as of 12 January 2025. This will ensure that there is enough time to set up the necessary governance structures (such as the Coordination Group and the Stakeholder Network) and to prepare the necessary documents before the initiation of any joint work.

EU cooperation on HTA in the past and regional initiatives

Of course, the HTA Regulation did not appear out of thin air. It will supersede the current system of HTA cooperation between Member States, which has taken shape through two initiatives in which all EU countries participate on a voluntary basis (i.e. the HTA Network[5] and the EU-funded EUnetHTA Joint Actions[6]). Since the voluntary project-based cooperation supported by these initiatives, mainly due to the absence of a sustainable framework, has proven inefficient and the uptake of the results at Member State level has remained limited, the European Commission considered this system inadequate to deliver on the aforementioned objectives of the EU Pharmaceutical Strategy.

[5] Established by article 15 of Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare.
[6] https://www.eunethta.eu/about-eunethta/history-of-eunethta/.

At a regional level, one can observe that some Member States have not waited for the EU to act and have taken matters into their own hands by establishing HTA cooperation structures that go even further than the joint work imposed by the Regulation. For these member states, the HTA Regulation will be rather uncontroversial given the fact that they have already voluntarily relinquished part of their national autonomy in the interest of a more coordinated approach to HTA. The Beneluxa Initiative[7] can be seen as the most far-reaching example of such regional cooperation initiative[8] involving health authorities in Belgium, the Netherlands, Luxemburg, Austria, and Ireland. Instead of only focusing on the clinical aspects of HTA, the Beneluxa Initiative provides for the possibility of performing full-fledged joint HTAs covering all aspects, as well as for the re-use or mutual recognition of national HTA reports. Moreover, in order to tackle the variety in national policies on pricing, reimbursement and use of pharmaceuticals, and to improve their strategic position in the market, the Beneluxa countries also engage in joint pricing and reimbursement negotiations with the industry.

Member States participating in regional initiatives should keep in mind that they will have to consider the joint clinical assessment reports developed at EU level also when conducting joints HTAs at a regional level. Effective interaction between regional initiatives and EU level cooperation may of course be beneficial for both, which is why the Commission should also take into account the results of the work undertaken by regional cooperation structures in the development of common EU rules and methodological frameworks on HTA.

[7] https://beneluxa.org/.

[8] Other examples, similar to the Beneluxa Initiative, include the Valletta Declaration and the Nordic Pharmaceutical Forum. BeNeFIT is an example of a regional project-based cooperation initiative (similar to the EUnetHTA Joint Actions) between (the Dutch) ZonMw and (the Belgian) KCE. The BeNeFIT program provides funding for research that compares the effectiveness of different treatment options that are already in use in the health care systems of Belgium and the Netherlands.

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