Capsulas

Health technology assessment, where are we?

About Regulation (EU) 2021/2282

Background

No medicial product can be marketed without obtaining a prior marketing authorization (MA). MAs are granted based on scientific criteria regarding the quality, safety and efficacy of the medicinal product concerned. If an MA is granted for a medicinal product, this means that the product has a favourable risk/benefit balance, it is effective for the authorized indication and safe under normal conditions of use.

However, does the new medicinal product offer added value compared to existing alternatives/comparators? This question is not addressed in the MA procedure, but rather in the context of health technology assessment (HTA). "Health technology" not only includes medicinal products, but also medical devices and procedures, as well as measures for prevention, diagnosis or treatment of diseases.

The main purpose of the HTA is to contribute to informing decisions on allocation of resources in the health sector, especially pricing and reimbursement decisions of medicinal products.

HTAs have typically been carried out at Member State level. Article 168 TFEU is clear: Union action in the field of public health shall respect the responsibilities of the Member States for the management of health services and medical care and the allocation of the resources assigned to them. HTA, as a key element in determining the allocation of the resources, has remained in the orbit of national (or infra-national) HTA bodies and outside the scope of mandatory EU regulation.

Since 2004, numerous voluntary collaboration initiatives between states in this area have been launched, such as the "EUnetHTA Joint Actions" and, since 2011, the "HTA Network", which was created by virtue of article 15 of Directive 2011/24/EU.

The outcome of these initiatives has been positive, especially in producing valuable scientific documents such as methodological guides and guidelines. However, voluntary and project-based collaboration (EUnetHTA Joint Actions) has failed to address the major challenges faced by HTAs at European level, which generate difficulties, delays and inequalities in patients' access to treatments. These challenges include duplication of HTAs for the same technology, inconsistencies in developers' requests for clinical evidence, different results across countries, heavy administrative burden, etc.

Regulation (EU) 2021/2282 takes a step further to address these challenges and to institutionalise a stable framework for collaboration between Member States on HTA.

Material and temporal scope of Regulation (EU) 2021/2282

Regulation (EU) 2021/2282 will only apply to medicinal products for which a centralized MA application has been submitted after January 2025 (antineoplastic medicinal products and advanced therapies), January 2028 (orphan medicinal products) and January 2030 (all remaining medicinal products).

As an exception to the above, medicinal products with a "potential to address an unmet medical need or a public health emergency or […] a significant impact on healthcare systems" may become subject to the Regulation earlier than the dates set out above. This type of situations will require a decision of the European Commission by means of an implementing act.

What is new in Regulation (EU) 2021/2282?

Three main novelties: the creation of Coordination Group on Health Technology Assessment (CG), Joint Clinical Assessments (JCA), and Joint Scientific Consultations (JSC).

Coordination Group

The CG is the main body of the new structure. It is composed of representatives of the 27 Member States. The CG shall meet in plenary sessions to approve the matters listed in article 3(7) of the Rules of Procedure. These include the adoption of its rules of procedure, its annual work programme and appointing subgroups. Subgroups will be responsible for overseeing the implementation of JCAs and JSCs. They will also be responsible for identifying emerging health technologies and developing methodological and procedural guidelines.

As a general rule, the CG shall act by consensus. Where consensus cannot be reached, a simple majority will be valid except for the approval of annual work programme and the strategic direction of the subgroups, which will require a qualified majority. A qualified majority requires a favourable vote of 55% of the Member States representing at least 65% of the EU population (article 238 TFEU).

The CG was created and held its first meeting on 21 June in Brussels.

Joint Clinical Assessment (JCA)

JCAs are one of the main novelties of the new Regulation. JCAs will include a clinical assessment of new health technologies that will be conducted by representatives of two Member States (assessor and coassesor) and supervised by the CG. JCAs will be limited to describing the "scientific analysis " and will not contain " any value judgement or conclusions on the overall clinical added value of the assessed health technology ". JCAs will not include non-clinical assessment such as budget impact or cost-effectiveness assessments.

JCAs will be based on the dossier submitted by the developer (at the latest 45 days prior to the envisaged date of the opinion of the Committee for Medicinal Products for Human Use) and its initial draft will be ready no later than 30 following the granting of the MA.

What impact will JCAs have on the HTAs conducted by each Member State? This remains a significant uncertainty. The answer largely depends on the degree of Member States' commitment to the implementation of the new Regulation. At present, here is what we can say.

On the one hand, Member States retain their competence to carry out their own HTAs (article 168 TFEU). Moreover, according to the Regulation, JCAs will be "non-binding" and not affect "Member States' competence to draw their conclusions on the overall clinical added value of a health technology". On the other hand, the Regulation provides that Member States shall "give due consideration" to the published JCAs, which shall be "annexed" to national HTA reports (e.g. to the TPRs). Member States shall provide information to the CG on "how joint clinical assessment reports have been considered when carrying out a national HTA".

Will Member States be able to request information already requested by companies in the course of JCAs? The Regulation once again takes an ambivalent stance and leaves room for States to act.

On the one hand, the Regulation provides that "the health technology developer shall not submit any information, data, analyses or other evidence at the national level that has been already submitted at Union level" (Article 10(3)). On the other hand, article 10(3) also clarifies that the Member State may, in fact, require additional information on non-clinical assessments (not covered by the JCA) or early-access-related programs. Additionally, Recital 15 states that "Member States should be able to perform complementary clinical analyses, which are necessary for their overall national HTA process, on the health technologies for which a joint clinical assessment report is available"; and that "additional information, data, analyses and other evidence is needed for complementary clinical analyses, Member States should be able to ask the health technology developers to submit the necessary information, data, analyses and other evidence."

We are yet to see whether Member States will retain total control over national HTA procedures and limit their requests for information to health technology developers (under the exceptions outlined above) or whether they will act under the new Regulation and limit requests for additional information.

Joint Scientific Consultations (JSC)

Under the new Regulation, developers of health technologies eligible for JCAs will be able to conduct scientific consultations before the CG. By way of JCAs, technology developers will obtain guidance on how best to design their clinical studies and investigations. Aspects such as the definition of interventions, comparators, health outcomes and patient population may be subject to JSCs.

JSCs are intended to allow developers to liaise with the CG at an early stage so as to anticipate the requirements of HTA bodies. JCSs may be requested jointly and in parallel to the scientific advice received from the European Medicines Agency.

Despite serving as guidance and orientation, the content and outcome of the JSCs will not be legally binding neither for the technology developer nor for HTA authorities.

Will all this affect Therapeutic Positioning Reports?

In Spain, medicinal products authorised by the European Commission (eligible for JCAs under the new Regulation) are subject to Therapeutic Positioning Reports (TPRs). TPRs are drafted by REvalMED, a network of therapeutic assessment teams (led by the AEMPS), economic assessment teams (led by the Directorate-General of Common Portfolio of Services of the NHS and Pharmacy), management and clinical experts appointed by the autonomous communities and the REvalMED Coordination Group.

TPRs aim to "provide a comparative therapeutic and economic assessment of medicinal products in order to provide relevant scientific-based information on the position of the new medicinal products, or its new indication, when compared to existing therapeutic alternatives, whether pharmacological or otherwise".

At this stage, we are unable to predict how JCAs will affect TPRs. In any case, what is clear for now is that TPRs should, in theory, "take [JCAs] into account" and include them as an annex. In addition, REvalMED will have to inform the CG of how considers JCAs when developing TPRs;

and should not, except in exceptional cases, require additional information that the company may have already provided during the JCA draft procedure.