Pharmaceutical innovators are concerned over not enough advice meetings being offered in 2025 for discussing trial designs and evidence generation plans, with serious ramifications on the ability to undergo Joint Clinical Assessments

ARM, EFPIA, EUCOPE, EuropaBio, Vaccines Europe

From 12 January 2025 the Regulation (EU) 2021/2282 on health technology assessment will apply and the first Joint Clinical Assessments (JCA) will be performed to assess the relative clinical effectiveness of new innovative cancer therapies and cell and gene therapies approved by the EMA. In just three years, by 13 January 2028, these joint assessments will also be performed for all new Orphan Medicinal Products (OMPs).

We welcome the new procedure and its aims to provide a transparent and inclusive framework for quality HTA, to reduce duplication for national HTA authorities and industry and to facilitate business predictability and ultimately, to accelerate access of medicines to EU patients. However, we are concerned that only a fraction of companies submitting for a JCA will be able to discuss their clinical trial plans at the critical planning stage as **in 2025 the HTA Coordination Group has only planned for 5-7 Joint Scientific Consultations for medicinal products.**

The number of planned JSCs does not reflect the realities of product development where there are more products at the development stage than those that are submitted for a Marketing Authorisation. The forecasted number of slots for advice meetings will not be sufficient to meet the expected high demand, as all companies launching their products in Europe will need to adjust to the new assessment framework when planning their studies.

- **The results of the JSC subgroup survey of companies from earlier this year showed a high demand for JSCs with companies identifying over 50 products where they were likely to submit a request for advice.** Further, since OMPs will become subject to JCA from 13 January 2028, the JSCs offered in 2025 will also have to meet the demand of advice requests for OMPs, with phase III trials being planned next year.

- In contrast, over the course of EUnetHTA Joint Action 3 (2016-2021) a total of 38 early dialogues were provided for companies. As the new procedure will be mandatory and the number of affected products significantly higher, the number of advice meetings offered should also be scaled up.

It will be mandatory for all aforementioned products to undergo the new EU HTA procedure, and the JCAs will be based on evidence and data submitted by companies. If misunderstandings are not resolved at the earliest possible stage, there is a higher risk of assessments becoming delayed, impacting patients access to innovative treatments. **If the EU HTA Regulation is to successfully deliver on its aims of reducing burdens for companies and speeding up access for patients, it is critical to offer more JSC slots, starting in 2025.**

Every company that applies for a JSC should have the possibility to receive advice. It is in the interest of everyone to ensure that the JCA produces a high-quality report that is of use to national decision-making.**The opportunity to receive early advice is critical for companies to ensure that they can adapt their clinical trial designs to generate adequate evidence and compile a quality dossier.** If there are not enough advice opportunities for companies, it will have a significant impact on patients' access to new treatments as companies will be less prepared to deliver the evidence required by the assessors.

- **Companies require clarity on evidence requests from Member States assessors during the critical time of clinical trial planning.** The risk is too high that future JCA's will either be

delayed due to perceived evidence gaps or that national HTA procedures will be protracted. While the JCA is meant to speed up access decisions, Member States might have to request additional evidence or studies from companies after the JCA, which would cause avoidable delays in access for patients to new treatments.

- **For small to mid-sized companies, the ability to discuss their evidence generation plans with the assessors is especially important as they often have less exposure to the European market due to smaller product pipelines**. In addition, they have smaller market access teams that will be less able to navigate the different national procedures in which the JCA will be applied, and less resources available for external support. At the same time, smaller companies are developing a large number of the cell and gene therapies and orphan medicinal products currently on the market and will require sufficient opportunities to receive advice as they will be among the first companies subject to the new requirements.

Currently all products in development that are in scope of the centralised European Marketing Authorisation are eligible for Scientific Advice on regulatory questions. These advice opportunities enable companies to better meet the evidence needs of assessors. According to the 2023 EMA activity report, in 65 percent of EMA approvals companies received Scientific Advice.

- A predictable framework for JSCs is required that allows companies to anticipate in advance when advice will be received, in order to align with EMA scientific advice as well as scientific advice received from other jurisdictions outside the EU. More frequent request periods with the possibility to anticipate possible dates for advice meetings throughout the year would provide more predictability for companies' development plans.

- **As it is in the interest of everyone to ensure a high-quality dossier, there is also a shared responsibility for clearly communicating the value of JSCs.** If not enough slots are offered and only a fraction of companies that are likely to go through a JCA will be able to receive advice, companies will be even less likely to apply for a JSC that might not take place.

We ask for a solution to be identified urgently and look forward to discussing how JSC capacity can be increased in 2025 and beyond. All options should be considered including introducing a fee-paying system to provide sufficient resources to support additional advice meetings as well as the prolongation of the current interim scientific advice model coordinated by the German HTA body G-BA.

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