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Lack Of Industry Involvement In EU HTA Scoping Process Exacerbates 'Unworkable' Timelines

by Francesca Bruce

Scientific advice could help companies make up for the lack of involvement in scoping, but slots are in short supply.

Pharmaceutical industry groups want drug developers to get more opportunities to interact with health technology assessment (HTA) bodies to define the scope of the forthcoming EU-wide joint clinical assessments (JCAs). Without more engagement, together with difficult timelines, companies will struggle to put together their JCA dossiers. This will in turn compromise the goal of the HTA regulation, which is to improve access to innovative medicines, the groups say.

Interaction with assessors is crucial for companies to get their JCA dossiers right, said Alexander Natz, secretary general of the industry group EUCOPE, which represents small and mid-sized firms.

"A poor dossier leads to poor assessment results and that impacts reimbursement processes and price negotiations in member states," Natz said in an interview with the *Pink Sheet*. "Ultimately that could mean poor access to innovative medicines,"

Joint clinical assessments is a key pillar of the HTA Regulation ((EU) 2021/2282), which is aimed at increasing HTA cooperation across Europe to reduce duplication of work and improve access to medicines (see box).

The concern from the industry groups follows the draft implementing act (IA) on joint clinical assessments, published for consultation in March. (Also see "*EU Opens Long Awaited Consultation On Joint*

Joint Clinical Assessments

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Clinical Assessments" - Pink Sheet, 8 Mar, 2024.)

The groups "share serious concerns over the lack of workability of the procedures in the draft implementing act," according to a recent joint statement from EFPIA, EuropaBio, the Alliance for Regenerative Medicines, EUCOPE and Vaccines Europe.

"Industry welcomed the proposal for joint clinical assessments from the start," Matias Olsen, EUCOPE's senior manager of public affairs & policy, told the *Pink Sheet*. "We believe it could deliver a one stop shop for clinical assessments, which would be a marked improvement over having to follow procedures in 27 different member states," Olsen said. However, EUCOPE, along with the other groups, is unsatisfied with the draft rules proposed by the member states for the JCA process.

Joint scientific assessments are similar to relative effectiveness assessments conducted by EU member state health technology bodies but they are to be conducted on a European level for consideration in decision making by member state pricing and reimbursement authorities.

Advanced therapies and oncology medicines will be the first products to undergo a joint clinical assessment from 12 January 2025, followed by orphan products from 13 January 2028 and all other products from 13 January 2030.

The HTA Regulation also introduces joint scientific consultations, which allow companies to seek scientific advice from the Member State Coordination Group during early clinical development. However, the number of slots will be limited and priority will be given to certain products, as outlined in the regulation.

Timelines

According to EUCOPE, the 140 days that assessors have to develop the scope for the JCAs is inefficient, excessive, and disproportionate to the 90-day deadline set out in the IA for companies to submit their dossier. The submission deadline is shorter than current timelines included in national procedures and "poses major challenges for developers."

The 140-day timeline is designed to coincide with the first 120 days of the regulatory assessment of the drug marketing authorization application by the European Medicines Agency, during which the EMA's human medicines committee, the CHMP, produces its list of questions for the developer before the first clock stop in the review cycle. The JCA implementing act then provides a further 20 days for finalizing the scope after the CHMP has adopted the list.

The scoping phase needs to be more efficient, said Olsen. "There is no benefit in waiting until the first clock stop to finalize the scope. Companies will answer assessor questions at an earlier point if there is an opportunity for interaction." Less time should be spent on developing the scope and companies should be given more time to develop their dossiers, he recommended.

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Scoping

Meanwhile, the scoping process aims to set parameters of the assessments. PICO (Population, Intervention, Comparator, Outcomes) surveys are sent to member states so they can set out their needs in terms of the relevant patient population, the intervention, comparators and outcomes. The surveys are consolidated by assessors, and drug companies must submit a dossier that addresses these data requirements.

However, there are no opportunities for companies to interact with assessors during this process, said Olsen. According to the IA, developers should provide the HTA secretariat with information relevant to developing the scope of the assessment. This information is the proposed summary of product characteristics and the clinical overview section of the developer's submission to the EMA.

The act also says that the HTA secretariat can invite developers to offer further information for developing the assessment scope, but only if the JCA subgroup thinks it necessary.

This is inadequate, say the industry groups. "We completely understand that at some point the assessors need to close the door to companies to conduct the assessment and then there shouldn't be any interaction. But before that point, it is absolutely needed," said Natz.

Industry participation in the scoping process means companies can share their expertise and research to make the scope more relevant. It also means that companies can better anticipate PICO scenarios, which can help them prepare earlier and increase the quality of their submission.

Scientific Advice

The lack of participation in the scoping process is exacerbated by the inadequate number of opportunities for companies to seek scientific advice through the joint scientific consultation (JSC) slots that will be available to them.

Developers that do secure a JSC can discuss scoping with assessors, although this is not the ideal opportunity, said Olsen. "The consultations take place early from Phase II development. Lots of things could have changed since then by the time you get to the scoping, he said."

As such, EUCOPE, along with the other industry groups, is advocating for mandatory scoping meetings that involve all companies undergoing a JCA so they can provide relevant information based on their expertise and research.

They also argue that companies should be able to contribute all relevant information and evidence as a key input to the assessment scope when it applies for marketing authorization.

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If companies cannot organize a meeting with the HTA coordination group, they should engage with national authorities to get some understanding of PICO expectations, advised Natz. However, to get a broader EU picture, developers would have to meet with at least 10 national authorities. "The G-BA in Germany will not tell me about Lithuania or France. And Austria is a smaller market, but it could influence others, so I should speak to them too," he said.

"It's in the interest of all parties involved to have a robust dossier as this will lead to broad access of patients to innovative therapies in Europe," added Natz.