vfa. Die forschenden Pharma-Unternehmen

Position paper

European Benefit Assessment: Creating Synergies – Harnessing Opportunities

With the new EU HTA Regulation, the benefit assessment of new therapies is regulated for the first time at the European level. The assessment will take place in parallel with the European regulatory approval process. The objectives are to accelerate the patient access to new therapies, reduce duplication of work, and to harmonize the clinical assessments. Cooperation at EU level can strengthen Europe as a pharmaceutical industry location. It is now important to implement EU HTA as a predictable, workable, and coordinated framework to create the greatest common value and to maintain the rapid patient access to new therapies in Germany.

The way is clear for the benefit assessment of new health technologies at European level (EU HTA, Health Technology Assessment). A compromise in the legislative process on the EU HTA Regulation was reached in June 2021 which will enter into force in January 2022. The EU Commissions' declared goal was to accelerate patient access to new medicines, reduce duplication of work, and harmonize clinical assessments. The compromise now on the table in particular allows flexibility concerning the binding nature of EU health technology assessment for the member states.

The new regulation establishes the legal and organizational framework for member states to cooperate on joint clinical assessments of new medicines and joint scientific consultations. The framework specifications now need to be fleshed out. EU HTA processes, methods and requirements will be shaped by the member states over the next three years. Newly approved oncology drugs and advanced therapy medicinal products (ATMP) will then be assessed first, the aim being to extend the procedure step by step to include other classes of medicinal products.

The clinical assessment of studies will take place at EU level in the future. However, Germany will retain national sovereignty in terms of assessment of the added benefit and pricing. Specifically, the regulation now governs that EU HTA assessments are to be given due – but not legally binding – consideration by member states in their decisions on clinical added value, price, and reimbursement. A legally binding mechanism only governs the one-time submission of clinical evidence at EU level, which cannot be requested again and resubmitted at the national level. However, member states may conduct additional clinical assessments and request additional information for this purpose.

Cooperation at the EU level can strengthen Europe as a pharmaceutical industry location and boost the global competitiveness of the sector. The vfa and its European umbrella organization, EFPIA (European Federation of Pharmaceutical Industries and Associations), have therefore advocated for an efficient EU HTA with demands for harmonization and mandatory use of the clinical assessments by the member states. However, the compromise text gives member states wide latitude for national re-assessments, which imposes

boundaries in terms of the harmonization objectives. It is now up to the member states to decide where those boundaries should lie in terms of their cooperation and when it comes to determining processes and methods. The member states should now harness the opportunity to grow closer together as Europe and create synergies. In that process, Germany should work to reduce divergences in the clinical assessment of innovative therapies in Europe without conceding national sovereignty in reimbursement issues and without compromising on rapid patient access to innovative therapies.

EU HTA implementation should be predictable, workable, and coordinated

Properly coordinated implementation of EU HTA is essential to maximize joint benefit while maintaining rapid patient access to new therapies in Germany. That being the case, the member states should now set the course for a clear, workable, and plannable assessment framework while looking to leverage synergies. To achieve this, the following points should be considered regarding the implementation at EU level and at national level in Germany.

Integrating European processes

EU process timescales should be defined precisely and aligned with AMNOG process timelines in a plannable manner. The AMNOG commenting process and hearing should be based on the evidence submitted. Calls for additional evidence at national level must be predictable for manufacturers and realizable within reasonable time limits. Adaptation of national procedures should involve industry participation and should be implemented at an early stage to create legal and planning security.

Coordinating European methods

Method guidelines should be designed such that the resulting clinical assessments can be used by member states and require only few re-assessments. To achieve this, a uniform European best practice model on method guidance should be developed based on EUnetHTA experience to facilitate consensus in assessments. Methods should be harmonized as much as possible and should not constitute a collection of national methods and approaches. Methods should be developed with industry participation. The specifics of orphan drugs, pediatric therapies, ATMP, vaccines, and chronic diseases should be given appropriate consideration when creating method guidelines.

Protecting manufacturers' rights

The procedural and participation rights of manufacturers in the EU HTA process must be safeguarded to enable participation of manufacturers in the processes in an appropriate manner and for a sufficient period of time. This would include general rules concerning, for example, the processes of scoping, to transmit data, to establish confidentiality of trade and business information, to address comments, and to review reports. The industry should be involved in the process to ensure that manufacturers' rights are properly defined. The framework should be designed such that those rights are clearly identifiable and reliably applicable.

Strengthening scientific consultations

Joint scientific consultations must deliver clear, reliable and timely guidance on the required clinical evidence. The requirements of the member states should be streamlined as effectively as possible to leverage synergies. They should also be coordinated with the EMA in parallel scientific consultations, to promote clinical trial designs with the greatest common value for both regulatory approval and for EU HTA. Thus, availability of joint scientific consultations must be sufficient to meet demand and be equipped with adequate capacities. Prioritization in the event of higher demand should be avoided at all costs, since any such prioritization would increase the risk of inappropriate clinical evidence for EU HTA and might jeopardize rapid patient access to new medicines.

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