European Haemophilia Consortium (EHC)
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EHC response to the European Commission's consultation on the implementation act on Joint Scientific Consultation for Medicinal Products

On the content

Inclusion of Real-World Evidence (RWE) in JSC Discussions
Real-world evidence (RWE) is a valuable complement to randomized controlled trials (RCTs) in understanding the real impact of health technologies. RWE should be designed and discussed during the JSC process, with attention to its feasibility. Although RWE should not replace RCTs, its integration should be achievable with reasonable resource demands. This approach will provide a broader perspective on patient experiences and outcomes.

Assessment of Relevant Patient Outcomes
The patient outcomes evaluated in the JSC should directly reflect the needs and priorities of patients. Data collection must focus on outcomes that matter most to patients, ensuring that the assessment truly reflects patient-relevant endpoints. This ensures that the technologies being evaluated are assessed in ways that matter to those directly affected. Furthermore, it is essential that the patient perspective and preferences is meaningfully and consistently included throughout the whole JSC at the earliest stage possible.

Coordination with EMA
EHC welcomes the emphasis on coordination with the European Medicines Agency (EMA). The field of bleeding disorders is currently seeing the entry of cutting-edge therapies such as gene therapy, therefore it is important to allow for parallel consultations in order to harmonise regulatory and clinical assessments and allow innovative therapies to reach patients faster.

On individual patient expert participation in the JSC

Intelligibility of the Briefing Package
The briefing package must be written in an easily understandable language. Clear, plain language ensures that patient experts can engage meaningfully and that their insights are integrated into the scientific discussions.

Language Support for Individual Patient Experts
Many individual patient experts possess deep knowledge of their conditions but may not be fluent in English. To ensure inclusivity, the JSC subgroup should offer tools and support for non-English speakers. This would allow patient experts to contribute their unique insights

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without language barriers limiting their participation. Furthermore, it is equally important to provide necessary training to patient experts so that they could participate as equal partners.

Adequate Time for Review of the Briefing Package
Drawing on past experiences, particularly with early dialogues under the EUnetHTA, we emphasise the importance of allowing sufficient time to review extensive documentation. Briefing packages are often dense and complex, making it difficult for patient organisations to provide thorough input if time is too limited. A more flexible and extended timeline would improve the quality of contributions from patient experts.

Sufficient Speaking Time for Patient individual experts in Meeting with Health Technology Developers
During the meeting with the health technology developer, individual patient experts must be given adequate time to express their concerns and perspectives. Meeting facilitators should ensure that patient voices are heard and valued, as these insights provide unique and irreplaceable contributions to the decision-making process.

A feedback interview with individual patient expert improves JSC subgroup expertise
To strengthen patient involvement in the Joint Scientific Consultations (JSCs), we propose the introduction of a feedback interview between patient experts and the JSC Subgroup, a practice already successfully implemented during the EUnetHTA JA3. This interview would allow patient experts to share their experiences and suggest improvements for future consultations, fostering a more inclusive and patient-centered approach. By gathering direct feedback, the JSC Subgroup can better understand patient needs and perspectives, ensuring their involvement in the HTA process is impactful.

On patient organisation participation in the JSC

Patient organisations should not be limited to commenting on disease and therapeutic areas. They should also provide insights on the potential impact of emerging technologies. Their broader perspective can help the JSC Subgroup and the Emerging Technology subgroup to anticipate and prepare for future advancements, ensuring that evaluations stay relevant and forward-thinking.