Final Concept Paper
E22 EWG: General Considerations for Patient Preference Studies
Dated 27 May 2024
Endorsed by the Management Committee on 2 June 2024

1. Type of harmonisation action proposed

A new ICH efficacy guideline is proposed about general considerations for patient preference studies (PPS) to inform drug development and related decisions for medical products. The guideline aims to optimize the use of patient preference information as input to pharmaceutical product development using a globally harmonised framework and allow regulatory authorities and the pharmaceutical industry to benefit from harmonised approaches for PPS throughout drug development including, where applicable, submission to different regulatory agencies.

2. Background to the proposal and statement of the perceived problem

PPS include any qualitative or quantitative assessment of the relative desirability or acceptability to patients of aspects that differ among alternative health interventions. PPS can add to the body of evidence and supplement clinical trial data when assessing benefit-risk. PPS can, among other things, help with characterization of medical need, endpoint selection and meaningful effect size estimation, and identify subgroups with different preferences.

Incorporating patient perspectives has become more systematic throughout drug development to better align decisions with patients' values and needs, and in the benefit-risk assessment, as reflected in ICH M4E (R2) and the ICH Periodic Benefit-Risk Evaluation Report (PBRER) E2C(R2). Relevant examples of available technical documents include the FDA, United States guidance on patient-focused drug development and devices, the IMI PREFER Recommendations and the EMA Qualification Opinion on IMI PREFER, the Medical Device Innovation Consortium (MDIC) Patient-Centered Benefit-Risk Framework, and the Professional Society for Health Economics and Outcomes Research (ISPOR) best practice documents on Patient Preference Methods and Quantitative Benefit-Risk Assessment. This guideline will cover one of the proposed initiatives in the ICH Patient-focused Drug Development Reflection Paper (2021). Harmonised guidance is needed focusing on general considerations for the design and conduct of PPS to inform pharmaceutical product development and promote consistency in regulatory submissions. The guidance will focus on areas lacking information and draw from established frameworks where appropriate.

3. Issues to be resolved and expected deliverable(s)

The proposed guideline intends to provide high level principles and practical guidance for regulatory implementation, in the following areas:

- Describe situations where PPS could be informative to pharmaceutical product development;
- Study design and methodological considerations, including:
  - Objectives, preference-elicitation method(s), and application of preference data;
  - Population(s) to be studied;
  - Attributes and attribute levels;
  - Plans for instrument development, pretesting, internal validity checks, and testing;
  - Statistical analyses;

  - Consideration of preference heterogeneity;
- Study documentation;
- Operational aspects and additional considerations, including:
  - Global applicability and cross-cultural context;
  - Good practices including quality checks;
  - Reporting and submission including impact on CTD Modules 2 and 5.

The detailed feedback received during the public consultation of the Reflection Paper (2021) will be considered during the writing of the guideline.

The working group will consider the possibility of detailed technical harmonisation but will generally avoid duplicating methodological aspects already published outside ICH. Guideline implementation training material will also be developed alongside the guideline.

A dedicated stakeholder engagement plan will be developed to ensure timely engagement with stakeholders outside regulatory and industry organisations (e.g., the patient community) around key milestones.

The placement of PPS data in labelling is considered a regional matter outside the scope of this guideline.

4. Planning

There is regulatory experience about advice on the design, and assessment of PPS. Available recommendations and guidance will provide valuable input. A standard ICH Expert Working Group (EWG) is sufficient to achieve the intended goals. The EWG will require broad expertise in the clinical development of medicines (e.g., biologicals, chemical entities, vaccines); biostatistics; clinical assessment; decision analysis; health outcome research.

The aim is to complete the guideline within 3 years from the appointment of the working group (approximately 24 months to reach *Steps 2a* and *2b*). Key milestones:

- High-level outline of guideline: October 2024;
- Expected start of regulatory consultation and discussion: December 2025;
- Stakeholder consultation (see below): February 2026;
- Adoption of the harmonised guideline: December 2026.

Stakeholder consultation: A public consultation and engagement is planned to follow the release for public consultation.

5. Impacts of the project and post-hoc evaluation

The new guidance will help identify situations where PPS can have greater impact, recommending suitable approaches depending on the study objectives. The activation of this ICH project is likely to create momentum and benefit to the field already during drafting and after publication of the *Step 2a/b* document (foreseen after 18 months from initiation of EWG activities). The major benefits would likely be realised shortly after *Step 5* (foreseen after 3 years from the initiation of activities). No post hoc evaluation of the impact of the project by the EWG is currently planned.