EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

ICH E22 - General Considerations for Patient Preference Studies (PPS)

Francesco Pignatti, Scientific Advisor, EMA

Common Questions: Why Preference *Studies*

- Being more systematic about value judgments in development and regulatory decisions

Similar positive benefit-risk balance formulations:

Implicit	$drug$	$\succ$	$placebo$
	$drug$	$\succcurlyeq$	$active$
Explicit (PPS)	$\sum w\,(benefit^{drug} - benefit^{placebo})$	$>$	$\sum v\,(harm^{drug} - harm^{placebo})$

Weights w, v, determine the trade-offs between benefits and harms. The $\succ$ symbol is used to indicate "preferred to".

What Role of *Patient* Preferences in Benefit-Risk Decisions?

Decision Making	Impact on Decision	Impact on Information

Directive ("Regulator knows best")	• Provide context for:
	  • Regulators' preferences
	  • Acceptable risk levels and precautionary principle
	• May tip the balance if everything else is equal
	• Increase public trust
		• Inform risk management plan
		• Inform communication strategies that align with patient values
		  • May improve compliance

Informed choice ("Patients and doctors know best")	• Theoretical (may be informative if utility is not self-evident)	In addition to the above: • Enhance patient autonomy by guiding the presentation of information • Inform development of decision aids (heterogeneity) • Prioritise empowerment, like shared decision making

What Methodology?

- Familiarity with preferences and elicitation methods is evolving
- PPS often align with observational studies
- Adherence to ICH E2E and E9 language likely needed
  - How PPS fit into the Drug Development Plan
  - Objectives and purpose; estimator; handling variability across subgroups; missing data; changes in preference; analysis plan

Flexible stepwise implementation not to overwhelm key actors and stakeholders

Harmonisation of Regulatory "Requirements" for Patient Preference Studies (E22)

Key regulatory guidance:

- PREFER recommendations / EMA Qualification
- MDIC Benefit-Risk Framework and Compendium of Methods
- FDA CDRH Guidance on Patient Preference Information; CDRH/CBER Draft Guidance on Patient Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle (NEW)
- FDA CDER Guidance on Collecting Patient Input
- Other: E.g., ISPOR Good Research Practices

E22 Current Step 1: EWG Drafts Technical Document

- Concept Paper adopted; Expert Working Group(EWG) Established in June 2024
  - Robyn Bent (Regulatory Chair, FDA); Laura Lee Johnson (FDA), Xinyi Ng (FDA)
  - Sheila Dickinson (EFPIA); Brett Hauber (PhRMA); Bennett Levitan (PhRMA)
  - Douwe Postmus (EMA)
  - …
- Currently, building consensus and drafting Technical Document
- Public consultation on Draft Guideline expected no later than December 2025

E22 EWG General Considerations for Patient Preference Studies

Rapporteur

Dr. Francesco Pignatti (EC, Europe)

Regulatory Chair

Ms. Robyn Bent (FDA, United States)

Experts

ANVISA, Brazil
Ms. Kalinka de Melo Carrijo

BIO
Ms. Allison Martin

EC, Europe
Dr. Douwe Postmus

EDA, Egypt
Dr. Dalia Kamal

EFPIA
Ms. Sheila Dickinson

FDA, United States
Dr. Laura Lee Johnson
Dr. Xinyi Ng

IFPMA
Ms. Lizis Kimura Lopes

IGBA
Dr. Ravi Shankar

JPMA
Ms. Inaha Okuda
Dr. Yasuo Sugitani

MHLW/PMDA, Japan
Dr. Madoka Inoue
Dr. Shun Tezuka

NMPA, China
Ms. Cong Zhao

National Center, Kazakhstan
Dr. Elmira Tulentayeva

PhRMA
Dr. Brett Hauber
Dr. Bennett Levitan

SFDA, Saudi Arabia
Dr. Shatha Almuhaidib

Swissmedic, Switzerland
Dr. Justyna Kozik-Jaromin

TFDA, Chinese Taipei
Dr. Kuan Ting Chen

Rapporteur Supporter

Ms. Charlotte Hallin (EC, Europe)

*The Rapporteur Supporter does not participate in technical WG discussion.

Disclaimer: Expert Working Groups members are appointed by their nominating ICH Member or Observer party and are responsible for representing the views of that party, which may not necessarily reflect their personal views. Working Group experts do not respond personally to external inquiries but are directed to forward any inquiries they receive to their nominating party or the ICH Secretariat for a response on behalf of either their ICH party or the ICH Association as appropriate. For questions to the ICH Secretariat, please use the contact form on the ICH website.

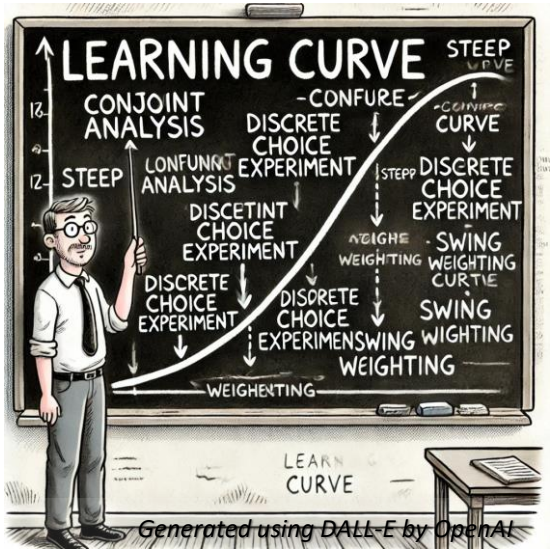
E22 Concept Paper: Key Messages

- Guidance on **high level** principles and practical guidance, drawing from **established frameworks** where appropriate
  - PPS can **add** to the body of evidence and supplement clinical trial data when assessing benefit-risk
  - PPS to **inform** pharmaceutical product development and promote consistency in regulatory submissions
- Ensure timely engagement with **stakeholders** outside regulatory and industry organisations (e.g., the patient community)
- The placement of PPS data in labelling is considered a regional matter (**out of scope**)

https://database.ich.org/sites/default/files/ICH_E22_ConceptPaper_2024_0602.pdf

Summary

- E22: New ICH guideline on patient preference studies, public consultation expected in about one year (December 2025)
- Numerous authoritative sources of guidance available (IMI PREFER; MDIC; FDA; ISPOR)
- Challenge: Familiarity with concept, application, and methods is evolving; how to keep guideline simple and aligned with existing statistical guidance



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Thank you!

francesco.pignatti@ema.europa.eu

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