



独立行政法人 医薬品医療機器総合機構
Pharmaceuticals and Medical Devices Agency

ICH E22

General Consideration for Patient Preference Studies

患者選好試験の一般的考察

Montreal meeting, November 2024

独立行政法人 医薬品医療機器総合機構（PMDA） 新薬審査第一部

手塚 瞬（Topic leader）

Outline

- Patient Preference Studiesとは
- Overview of topic -トピックの概要-
- モントリオール会合前までの進捗
- モントリオール会合の成果
- 今後のWork Plan : Key Milestones and Activities

Patient Preference Studiesとは



独立行政法人
医薬品医療機器総合機構

● Patient Preference Information/Studies（患者選好情報/患者選好試験）

Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle

Draft Guidance for Industry, Food and Drug Administration Staff, and Other Interested Parties

DRAFT GUIDANCE

2024年9月公開
先日までパブリックコメント募集

B. What is patient preference information?

Patient preference information, for the purposes of this guidance, is defined as qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions.⁵

<https://www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-voluntary-patient-preference-information-over-total-product-life-cycle>

Patient-Focused Drug Development Glossary

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This glossary defines terms that will be used in the series of methodological Patient-Focused Drug Development (PFDD) FDA guidance documents that are required by the 21st Century Cures Act, and part of commitments made by FDA under the sixth authorization of the Prescription Drug User Fee Act (PDUFA VI). The goal of this glossary is to provide standardized nomenclature and terminologies related to patient-focused medical product development. As the science of patient input matures, or in response to comments received on FDA's guidance, this glossary may be updated.

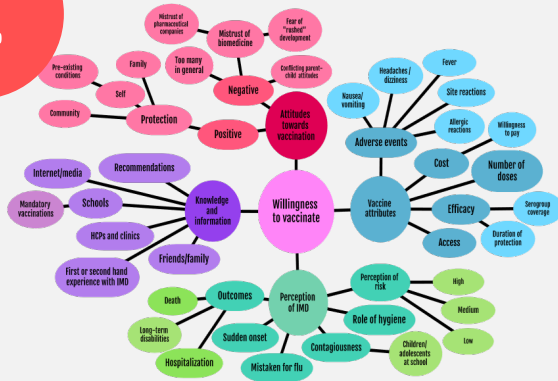
Patient preference information (PPI): Assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions. The methods for generating PPI may be qualitative, quantitative, or mixed methods. (Source: [FDA Guidance on PPI for medical devices](#))

<https://www.fda.gov/drugs/development-approval-process-drugs/patient-focused-drug-development-glossary>

治療のさまざまな特性（Attributes）について、患者にとって「何が」「どの程度」重要か（Patient Preference Information）を、質的（Qualitative）、量的（Quantitative）に検討すること

Patient Preference Studiesとは

1

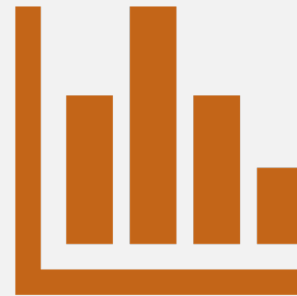


Coulter J, et al From Qualitative Research to Quantitative Preference Elicitation: An Example in Invasive Meningococcal Disease. Patient. 2024 May; 17(3): 319-33

What Matters to Patients

「何が重要か」

2



How Much it Matters

「どれだけ重要か」

3



Tradeoffs Patients are Willing to Make Between Benefits and Risks

「許容できるトレードオフ」

● Patient Preference Information (PPI)

：治療のさまざまな特性（Attributes）について、患者にとって「何が」「どの程度」重要か

● Patient Preference Studies (PPS)

：PPIを質的（Qualitative）、量的（Quantitative）に調査すること

Overview of topic -トピックの概要-

E22の背景

- Patient preference studies (PPS) は、様々な医療介入で異なる側面（Attributes）について、患者の選好（relative desirability or acceptability）を検討することを含む。
- PPSはベネフィットリスク評価において臨床試験データを補足し得る。
 - メディカルニーズの特定
 - エンドポイントの選択と臨床的に意義ある効果の大きさの見積り
 - 異なる選好を有するサブグループの同定

Overview of topic -トピックの概要-



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Read the PREFER recommendations

The PREFER recommendations in brief

Finding out what patients PREFER

On 28 April 2022 we launched the PREFER Recommendations on why, when and how to assess and use patient preferences in medical product decision-making.

ScienceDirect

Contents lists available at www.sciencedirect.com
journal homepage: www.elsevier.com/locate/jval

ELSEVIER

ISPOR Report

A Roadmap for Increasing the Usefulness and Impact of Patient-Preference Studies in Decision Making in Health: A Good Practices Report of an ISPOR Task Force

John F.P. Bridges, PhD, Esther W. de Bekker-Grob, PhD, Brett Hauber, PhD, Sebastian Heidenreich, PhD, Ellen Janssen, PhD, Alice Bast, BA, Janel Hammer, MD, PhD, Andriy Danyliv, PhD, Eric Low, MSc, Jacqueline C. Bouvy, PhD, Deborah A. Marshall, PhD

ABSTRACT

Many qualitative and quantitative methods are readily available to study patient preferences in health. These methods are now being used to inform a wide variety of decisions, and there is a growing body of evidence showing studies of patient preferences can be used for decision making in a wide variety of contexts. This ISPOR Task Force report synthesizes current good practices for increasing the usefulness and impact of patient-preference studies in decision making. We provide the ISPOR Roadmap for Patient Preferences in Decision Making that invites patient-preference researchers to work with decision makers, patients and patient groups, and other stakeholders to ensure that studies are useful and impactful. The ISPOR Roadmap defines the steps to increase questions and the ongoing process.

Keywords:

MEDICAL DEVICE INNOVATION CONSORTIUM (MDIC) PATIENT CENTERED BENEFIT-RISK PROJECT REPORT:

A Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology

By Medical Device Innovation Consortium (MDIC)

MDIC
MEDICAL DEVICE INNOVATION CONSORTIUM
A BOLD & ACCURATE

Contains Nonbinding Recommendations

Draft – Not for Implementation

New

Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle

Draft Guidance for Industry, Food and Drug Administration Staff, and Other Interested Parties

DRAFT GUIDANCE

This draft guidance document is being distributed for comment purposes only.

Document issued on September 6, 2024.

You should submit comments and suggestions regarding this draft document within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to <https://www.regulations.gov>. Submit written comments to the Dockets Management Staff, Food and Drug Administration, 5630 Fishers Lane, Room 1061, (HFA-305), Rockville, MD 20852-1740. Identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions about this document regarding CDRH-regulated devices, email cdrh-ppi@fda.hhs.gov. For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010, or by email at ocod@fda.hhs.gov.

When final, this guidance will supersede “Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling,” issued August 2016.

FDA U.S. FOOD & DRUG ADMINISTRATION

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research

海外では既に様々なガイダンスが発出

- ✓ PREFER recommendations / EMA Qualification
- ✓ MDIC Benefit-Risk Framework and Compendium of Methods
- ✓ ISPOR Good Research Practices
- ✓ FDA CDRH Guidance on Patient Preference Information; CDRH/CBER Draft Guidance on Patient Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle

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Food and Drug Administration
Center for Drug Evaluation and Research

海外では既に様々なガイダンスが発出

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- ✓ MDIC Benefit-Risk Framework and Compendium of Methods
- ✓ ISPOR Good Research Practices

医薬品開発への示唆や規制当局への申請の一貫性を促進するために、PPSのデザインや実施に関する一般的な考察に焦点を当てた国際的に調和の取れたガイダンスが必要

Overview of topic -トピックの概要-

E22の目的

- 患者選好情報の利用を最適化すること

- 医薬品開発へのインプットとして
- ベネフィットリスク評価へ示唆を与えるものとして

(PPSの結果のlabelへの記載はE22のscope外)

- PPSのHigh levelな原則と実践的なガイダンスを提供する

- PPSが有用な場合, PPSのデザインや手法, PPSの研究文書
- その他, 国際的な適用可能性の考慮, 品質チェック, CTDへの影響等の考慮

モントリオール会合前までの進捗

- ✓ IWG発足→定期Webミーティング開始（2024年3月）
- ✓ コンセプトペーパーの承認（2024年6月）→EWGへ移行
- ✓ ガイドラインのアウトライン（枠組み）の作成
- ✓ ドラフト初稿の作成

モントリオール会合の成果

Reached consensus on コンセンサスに至ったもの

- ✓ ガイドラインにおける重要な要素
- ✓ ガイドラインのアウトライン（枠組み）

Began to 検討を開始したもの

- トレーニングマテリアルに含めるべき要素
- Stakeholder Engagement Plan

その他

- ◆ EWGメンバーによるPPSの経験の共有

今後のWork Plan - Key Milestones and Activities -

Expected Completion date	Deliverable
Summer/Autumn 2025	<ul style="list-style-type: none"> Stakeholder Engagement Meeting
December 2025	<ul style="list-style-type: none"> Step 2A/B <ul style="list-style-type: none"> ICH Parties consensus on Technical Document Draft Guideline adoption by Regulators
February 2026	<ul style="list-style-type: none"> Step 3 <ul style="list-style-type: none"> Regulatory consultation and Discussion
May 2026	<ul style="list-style-type: none"> Begin to develop educational materials
December 2026	<ul style="list-style-type: none"> Step 4 <ul style="list-style-type: none"> Adoption