U.S. FOOD & DRUG ADMINISTRATION

Welcome To Today's Webinar

Thanks for joining us!
We'll get started in a few minutes

Today's Topic:
Draft Guidance: Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle

October 15, 2024

Draft Guidance: Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle

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Draft Guidance

- **Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle**
  - www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-voluntary-patient-preference-information-over-total-product-life-cycle

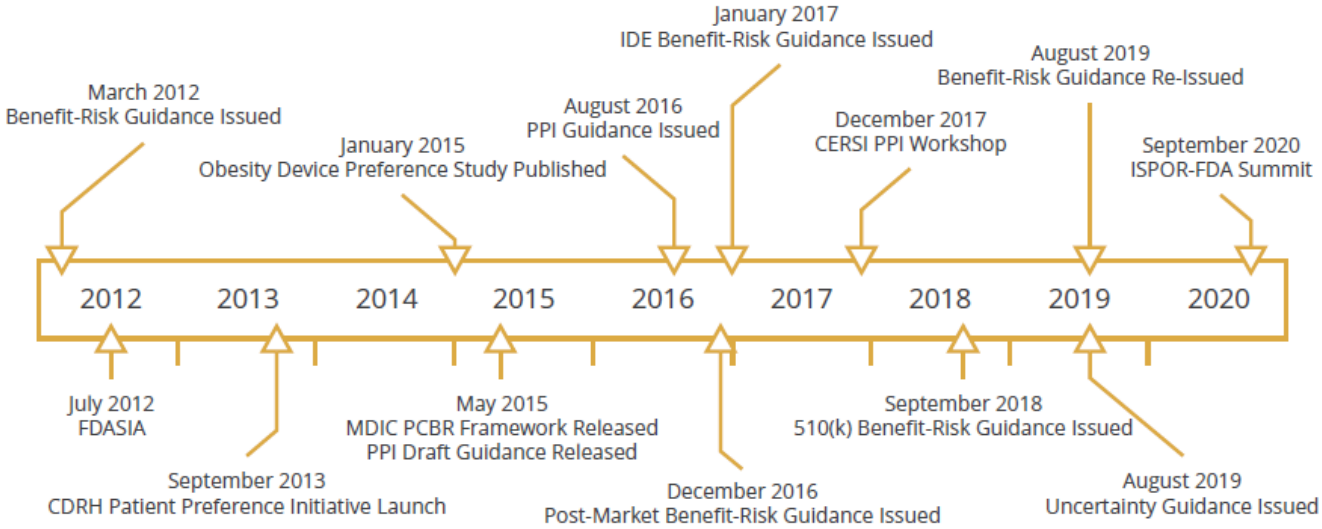
Draft Guidance: Not for Implementation

Learning Objectives

- Explain the rationale for the proposed updates to the current 2016 PPI Guidance
- Lay out the scope of proposed updates
- Describe the proposed modifications
- Describe how to submit comments on the draft guidance

PPI – Patient Preference Information

Patient Preferences Timeline at CDRH



CDRH indicates Center for Devices and Radiological Health; CERSI, Centers of Excellence in Regulatory Science and Innovation; FDASIA, Food and Drug Administration Safety and Innovation Act; IDE, investigational device exemption; MDIC, Medical Device Innovation Consortium; PCBR, Patient Centered Benefit–Risk; PPI, patient preference information.

Webber CM, Chen AL, Gebben DJ, Saha A, Tarver ME. Measuring Patient Preferences at the FDA Center for Devices and Radiological Health: Reflections and Projections. Value Health. 2021 Jul;24(7):1024-1029. doi: 10.1016/j.jval.2021.01.009. Epub 2021 Apr 28. PMID: 34243826.

Highlights of 2024 Draft Guidance Updates

- This draft guidance:
  - Integrates concepts from additional benefit-risk guidances and benefit-risk paradigm, which reflect the TPLC
  - Provides additional considerations and practical recommendations on experiences evaluating patient preferences for devices, from additional interactions with sponsors to additional CDRH sponsored PPI studies being completed
  - Addresses MDUFA V commitment (Section V.5.c of the MDUFA V Commitment Letter*)

*MDUFA Performance Goals and Procedures, Fiscal Years 2023 Through 2027

Principles Maintained from 2016 Version

- **Patient Preference Information** (PPI) is still 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*"

*For more information, see 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

Principles Maintained from 2016 Version Cont'd

- Maintains focus on PPI research as patient-centered
- Maintains expectation that research done in line with recommended practices from professional organizations

Rationale for Update

Feedback

- Feedback from sponsors and other interested parties
  - Submitted via docket FDA-2015-D-1580 and through regulatory submissions
- Identified gaps in current final guidance
- Requested clarity on analysis and uses of qualitative and quantitative methods
- The integration of feedback is part of our MDUFA V commitment to provide "pragmatic insights and to address common questions for those interested in the voluntary use of PPI in regulatory submissions*."

*MDUFA Performance Goals and Procedures, Fiscal Years 2023 Through 2027

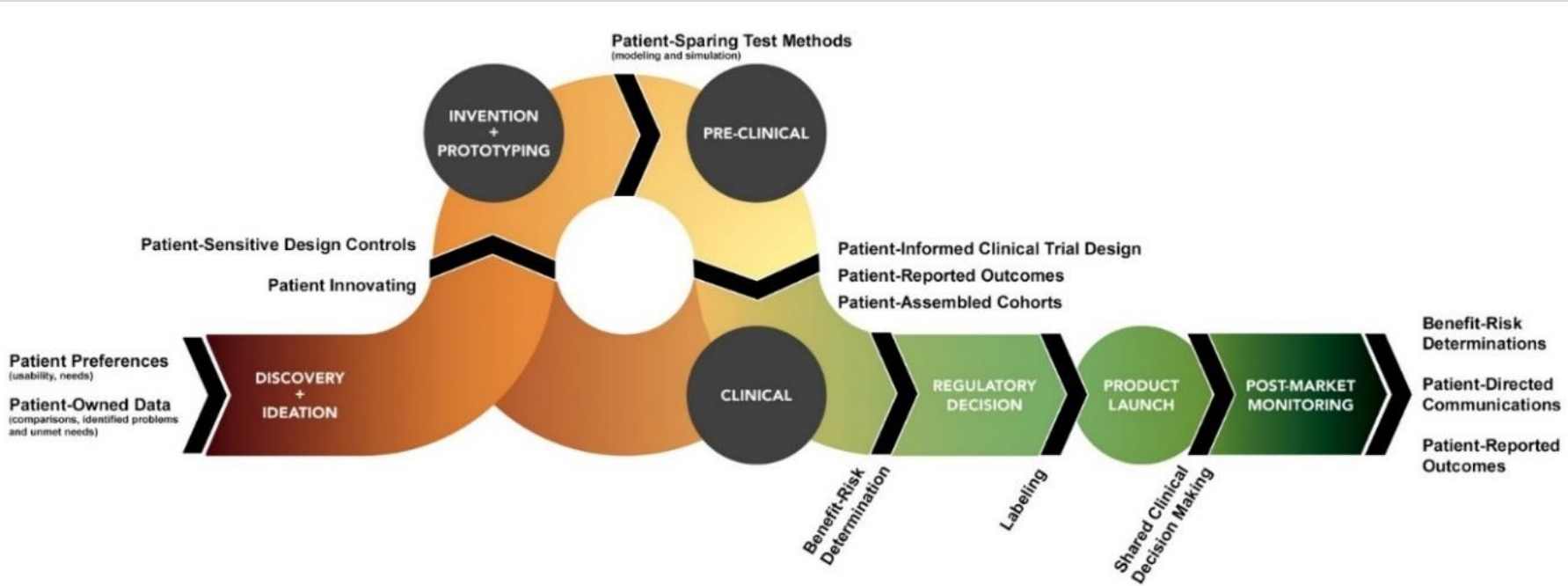
Questions Addressed in Draft Guidance

- Role of qualitative methods for eliciting PPI – when is it appropriate?
- What type of quantitative methods are available and is there guidance on the selection of methods?
- What is considered a fit-for-purpose PPI study?
- What attributes and attributes levels should be included?
- What research question should the PPI study address?

Questions Addressed in Draft Guidance Cont'd

- What should be included in a PPI study protocol submitted for FDA's review?
- What research question should the PPI study address?
- Why is it important to have early interactions with FDA?
- What should be included in a PPI study protocol submitted for FDA's review?

Patient Input in the TPLC



Final Guidance, 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

Potential Uses of PPI Across the TPLC

Development	Clinical Trial Design	Premarket Benefit-Risk Assessment	Post-Market
1. Identify unmet medical need 2. Understand what matters most to patients about their disease or treatment	1. Inform endpoint selection 2. Inform performance goal 3. Inform effect size	1. Analysis of condition 2. Current treatment options 3. Patient perspective on benefit-risk tradeoffs 4. Population subgroup considerations	1. Inform interpretation of new data affecting benefit-risk assessment 2. Inform studies of new/expanded use populations 3. Communicate benefit-risk information to patients

Reference Appendix A of Draft PPI Guidance

What are the updates?

Summary of Key Changes

	Current Guidance	Draft Guidance
Title Updated title to better reflect the current scope of PPI information which could be applicable in the regulatory context	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	Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle
Examples Comments to the docket, numerous responders asked for more examples of regulatory use of PPI	PPI to support benefit-risk decisions (for example, weight loss device for obesity)	• PPI to establish clinical study performance threshold (such as for novel pediatric ear tube system) • PPI to support benefit-risk decisions (for example, weight loss device for obesity) • PPI to support indication and labeling expansion (such as home hemodialysis)

Summary of Key Changes Cont'd

	Current Guidance	Draft Guidance
Methods In comments from docket and submissions since 2016, numerous requests for further clarification of appropriate methods	Should be aligned with professional organizations recommendations	Should be aligned with professional organizations recommendations, appendix listing possible methods that could be applicable
Fit-For-Purpose	Patient-Centric	Patient-Centric, highlights: scientific question, the study objectives, the study parameters
Opportunities for using PPI	Point of submission of marketing application	Expanded to other places in TPLC including Investigational Device Exemptions (IDEs)

Expansion of Practical Examples

- In the 2016 Guidance, the primary use-case was the obesity study* which focused on the benefit-risk decision context after clinical data had been provided
- Since 2016, more PPI studies have been done, including to support:
  - Label expansion: Solo home hemodialysis
  - Clinical effectiveness threshold: Ear tubes placement
- While this is not an exhaustive list, these examples, along with the hypothetical examples in Section IX, are meant to illustrate that there are more potential places for use of PPI within a regulatory context

*Ho MP, Gonzalez JM, Lerner HP, Neuland CY, Whang JM, McMurry-Heath M, Hauber AB, Irony T. Incorporating patient-preference evidence into regulatory decision making. Surg Endosc. 2015 Oct;29(10):2984-93. doi: 10.1007/s00464-014-4044-2. Epub 2015 Jan 1. PMID: 25552232.

Methods: Appendix B

- Multiple sponsors and docket comments requested more clarity on what methods could be used for analysis of PPI
- To address that concern, we added information about what types of methods could be applied to various research questions
- CDRH recognizes that analysis methods are changing and improving
- This appendix is not an exhaustive list

Reference Appendix B of Draft PPI Guidance

Fit-For-Purpose

- Multiple sponsors and docket comments requested more clarity regarding a "fit-for-purpose" study
- Key aspects:
  - the scientific question
  - the study objective
  - the study parameters
  - the type of study design, qualitative or quantitative, and method(s)
  - the study population, including the enrollment criteria and recruitment method(s)
  - if a survey method is used, the specific survey design

Reference Section IV.I of Draft PPI Guidance

Resources

Resource	URL
Consideration of Uncertainty in Making Benefit-Risk Determinations in Medical Device Premarket Approvals, De Novo Classifications, and Humanitarian Device Exemptions	www.fda.gov/regulatory-information/search-fda-guidance-documents/consideration-uncertainty-making-benefit-risk-determinations-medical-device-premarket-approvals-de
Benefit-Risk Factors to Consider When Determining Substantial Equivalence in Premarket Notifications (510(k)) with Different Technological Characteristics	www.fda.gov/regulatory-information/search-fda-guidance-documents/benefit-risk-factors-consider-when-determining-substantial-equivalence-premarket-notifications-510k
Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and De Novo Classifications	www.fda.gov/regulatory-information/search-fda-guidance-documents/factors-consider-when-making-benefit-risk-determinations-medical-device-premarket-approval-and-de
Factors to Consider When Making Benefit-Risk Determinations for Medical Device Investigational Device Exemptions	www.fda.gov/regulatory-information/search-fda-guidance-documents/factors-consider-when-making-benefit-risk-determinations-medical-device-investigational-device
Factors to Consider Regarding Benefit-Risk in Medical Device Product Availability, Compliance, and Enforcement Decisions	www.fda.gov/regulatory-information/search-fda-guidance-documents/factors-consider-regarding-benefit-risk-medical-device-product-availability-compliance-and
MDUFA Performance Goals and Procedures, Fiscal Years 2023 Through 2027	www.fda.gov/media/158308/download?attachment

A Note about Draft Guidances

- You may comment on any guidance at any time
  - see 21 CFR 10.115(g)(5)
- Please submit comments on this draft guidance before **December 5, 2024**
  - to ensure that FDA considers your comment on a draft guidance before we work on final guidance
  - Where to place comments: https://www.regulations.gov/docket/FDA-2015-D-1580/document

Summary

- Revisions in the Draft PPI guidance reflect updates to the state of PPI today, as well as reflecting concerns from sponsors and other interested parties
  - In better alignment with more recent published guidances
- Modifications to add clarity and fill gaps
- Scope widened to the entire TPLC
- This draft guidance is responsive to Section V.5.c of the MDUFA V Commitment Letter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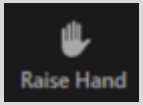
Let's Take Your Questions

- **To Ask a Question:**
  1. Raise your hand in Zoom 

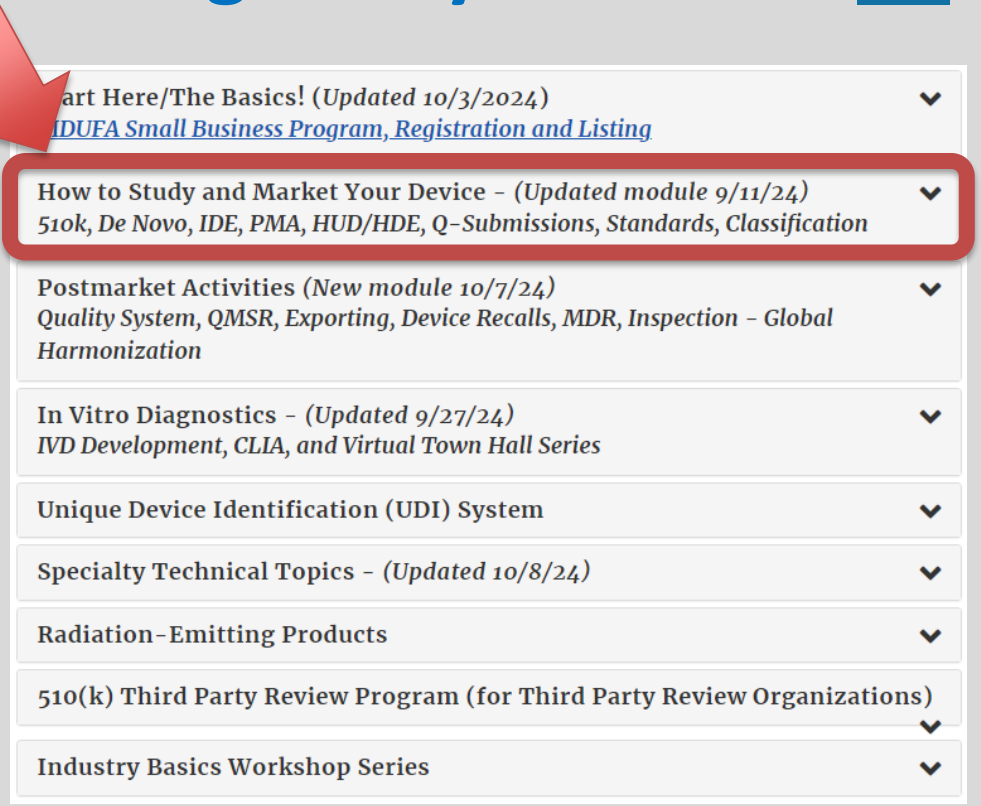
  2. Moderator will announce your name and invite you to ask your question
  3. Unmute yourself when prompted in Zoom to ask your question

- **When Asking a Question:**
  - Ask one question only
  - Keep question short
  - No questions about specific submissions

- **After Question is Answered:**
  - Mute yourself and lower your hand
  - If you have more questions - raise your hand again

Thanks for Joining Today!

- **Presentation and Transcript will be available at CDRH Learn**
  - www.fda.gov/Training/CDRHLearn
- **Additional questions about today's webinar**
  - Email: DICE@fda.hhs.gov
- **Upcoming Webinars**
  - www.fda.gov/CDRHEvents

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