EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Joint EMA/EORTC workshop on: How can patient-reported outcomes (PRO) and health-related quality of life (HRQoL) data inform regulatory decisions?

PCWP-PEC joint meeting – 18 June 2024

Presented by Caroline Voltz-Girolt
Oncology external engagement coordinator, Office of advanced therapies and haematological diseases

The views expressed in this presentation are the personal views of the speakers and may not be understood or quoted as being made on behalf of or reflecting the position of the EMA or one of its committees or working parties

Outline

- **Background: How EMA has involved patients in the evaluation of medicines**
- **Why a workshop on HRQoL in Oncology?**
- **Main discussions on the workshop**
- **Conclusions**

Journey of patient involvement

1995
EMA created

1996
Dialogue with patients initiated

2000
Patients become Committee members

2005
Framework of interaction with patients, consumers and their organisations

2006
Patient & Consumer Working Party (PCWP)

2014
EMA's Public Engagement Department created

2017
- Public hearings
- Involving young people

2020
Patient engagement in crisis management

2022
Patient experience data

Ongoing…
Systematic patient input along medicine lifecycle

EMA engagement with patients in regulatory activities

Patient Experience data part of the EMA regulatory science strategy

- Reinforcing patient relevance in evidence generation is **key priority for the EU Network Strategy** and in EMA's **Regulatory Science Strategy**
- **Need for systematic inclusion of Patient Experience data (PED)** in medicines development and regulation
- **PED is a new scientific discipline** – balance difficult methodological discussions with stakeholder engagement
  - Collaboration of multi-disciplinary experts cross-Agency and within EU Network
- **Opportunities for patient-generated digital data** thanks to novel technologies
- The **EU Network Strategy's delivery plan** and **CHMP's 2024 workplan** incorporate two key deliverables:
  - **Reflection paper** on the best EU approach to generate, collect and analyse PED
  - Explore how to **improve transparency in the Assessment Report**

Ongoing activities on using patient experience data

- ICH reflection paper - proposed ICH guideline work to advance Patient Focused Drug Development
- Implementation of the estimand framework (ICH E9)
- EMA 2022 workshop Patient Experience Data (PED)
- EMA Reflection paper to PED planned for 2024
- EMA early dialogue with patient organisations for orphan marketing authorisation applications
- Qualification procedures:
  - Validation studies
  - Use cases
- Revised D80 Clinical Assessment Report


ICH harmonisation for better health

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24 June 2021
EMA/CHMP/ICH/338534/2021
Committee for Medicinal Products for Human Use

ICH reflection paper - proposed ICH guideline work to advance Patient Focused Drug Development (PFDD)

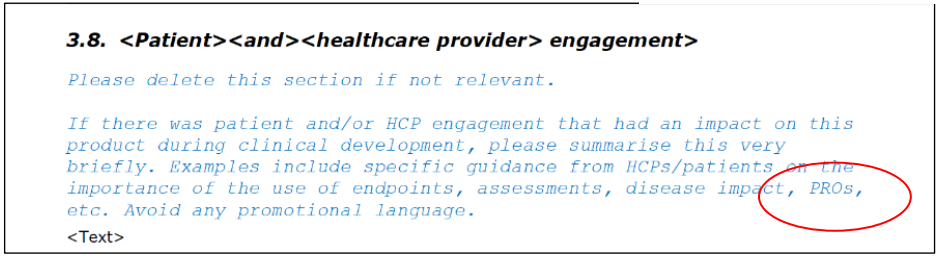
Release for information	24 June 2021

30 August 2017
EMA/CHMP/ICH/436821/2017
Committee for Human Medicinal Products

ICH E9 (R1) addendum on estimands and sensitivity analysis in clinical trials to the guideline on statistical principles for clinical trials
Step 2b

Transmission to CHMP	July 2017
Adoption by CHMP for release for consultation	20 July 2017
Start of consultation	31 August 2017
End of consultation (deadline for comments)	28 February 2018

Comments should be provided using this template. The completed comments form should be sent to ich@ema.europa.eu


3.8. <Patient><and><healthcare provider> engagement>

Please delete this section if not relevant.

If there was patient and/or HCP engagement that had an impact on this product during clinical development, please summarise this very briefly. Examples include specific guidance from HCPs/patients on the importance of the use of endpoints, assessments, disease impact, PROs, etc. Avoid any promotional language.

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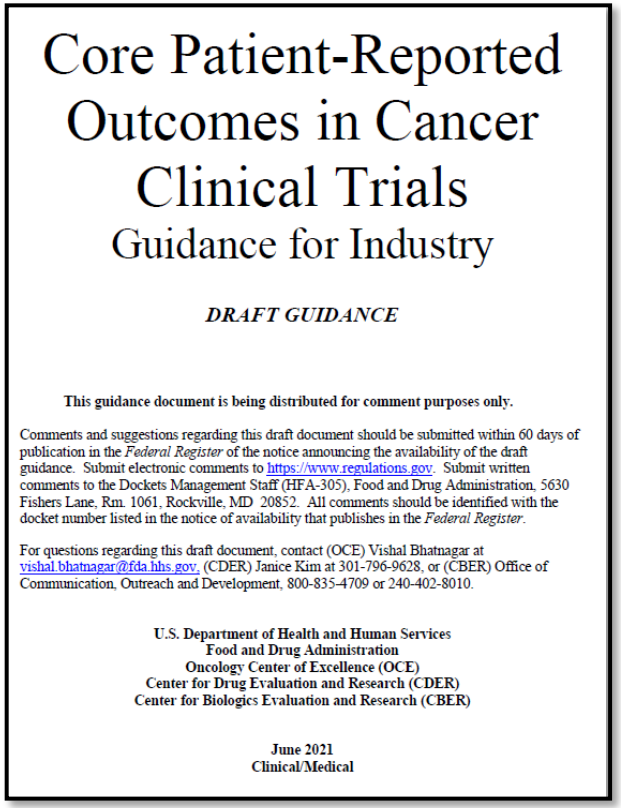
Existing guidance on PROs



Core Patient-Reported Outcomes in Cancer Clinical Trials

Guidance for Industry

DRAFT GUIDANCE

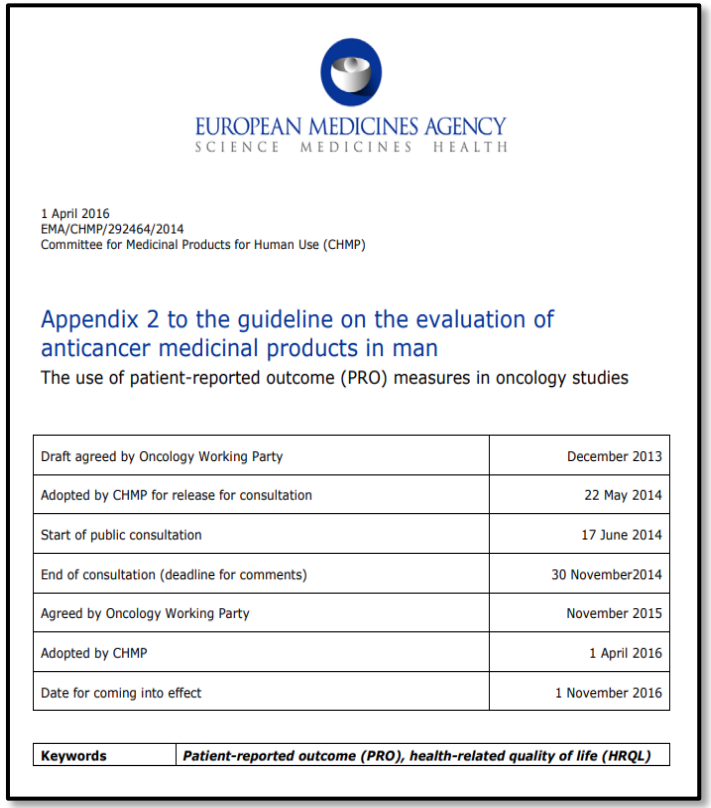
This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted 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 (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (OCE) Vishal Bhatnagar at vishal.bhatnagar@fda.hhs.gov, (CDER) Janice Kim at 301-796-9628, or (CBER) Office of Communication, Outreach and Development, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Oncology Center of Excellence (OCE)
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

June 2021
Clinical/Medical



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1 April 2016
EMA/CHMP/292464/2014
Committee for Medicinal Products for Human Use (CHMP)

Appendix 2 to the guideline on the evaluation of anticancer medicinal products in man

The use of patient-reported outcome (PRO) measures in oncology studies

Draft agreed by Oncology Working Party	December 2013
Adopted by CHMP for release for consultation	22 May 2014
Start of public consultation	17 June 2014
End of consultation (deadline for comments)	30 November2014
Agreed by Oncology Working Party	November 2015
Adopted by CHMP	1 April 2016
Date for coming into effect	1 November 2016

Keywords	***Patient-reported outcome (PRO), health-related quality of life (HRQL)***

Joint EMA/EORTC workshop: How can PRO and HRQoL data inform regulatory decisions? 29 February 2024

Problem statement: gap in the systematic implementation of PROs in regulatory and HTA decision-making due challenges decision makers face in interpreting PRO data

Objective: How can PROs and HRQoL data inform regulatory decision and cover HTA needs with an International collaboration?

- 1007 registered participants
- Presentations and video available on EMA website

14:10 **Session 1: How can PROs and HRQoL data inform regulatory decision as well as cover HTA needs?**

Chairs: Jaap Reijneveld (EORTC) and Pierre Demolis (EMA)

Learnings from PROs used for regulatory approval of oncology medicines in the European Union 15'
Carla Torre, CHMP co-opted member, INFARMED

FDA views, practices and challenges in assessment of PROs – what does FDA need? 15'
Vishal Bhatnagar, Associate Director for Patient Outcomes, Oncology Center of Excellence, FDA

Application and importance of HRQoL/PRO assessment from HTA perspective 15'
Beate Wieseler, Head of Department Drug Assessment, IQWIG

A Patient-Reported-Outcome-based Multi-State-Modelling approach to Benefit-Risk Assessment 15'
Douwe Postmus, University of Groningen, NL and Seconded National Expert, EMA

15:10 **Coffee Break**

15:30 **Panel Discussion**

Moderators: Jaap Reijneveld (EORTC) and Peter Mol (EMA)

Paul Kluetz, Deputy Director, Oncology Center of Excellence, FDA
Maxime Sasseville, Clinical manager, Oncology Division 2, Health Canada
Friedrich Wittenbecher, Swissmedic
Shun Tezuka, Medical officer, Office of New Drug I/IV/V, PMDA
Harald Enzmann, Chair of CHMP, Bfarm, EMA
Anja Schiel, Special Advisor, Norwegian Medicines Agency
Bettina Ryll, Founder of MPNE and WECAN representative
Joseph Cappelleri, Executive Director of Biostatistics, Pfizer
Christopher Booth, Director, Division of Cancer Care and Epidemiology, Queen's Cancer Research Institute

- Learnings from regulators and HTAs and discussion aiming at providing clarity on the role of PROs for decision-making
- What are the expectations and what can be achieved
- Facilitated discussion amongst International regulators, HTAs, patients, academics and industry

17:00 **Session 2: Novel Approaches to the measurement of HRQOL and other PROs**

Chairs: Chantal Quinten (EMA) and Madeline Pe (EORTC)

EORTC's strategy on the development and implementation of PRO and HRQoL measures for cancer clinical research — 15'
Mogens Groenvold, Professor in Palliative Care and PRO Assessment, University of Copenhagen and Bispebjerg/Frederiksberg Hospital

Measuring HRQoL core outcomes and disease specific symptoms — 15'
Johannes Giesinger, Assistant Professor of Health Outcomes Unit, Medical University of Innsbruck

Measuring treatment-specific side effects from the patient perspective: trial-specific item lists from PRO item libraries — 15'
Alexandra Gilbert, Associate Professor in Clinical Oncology, University of Leeds
Claire Piccinin, item Library researcher, EORTC

Application of the use of static and flexible PRO measures in global cancer trials: Challenges and opportunities — 15'
James W. Shaw, Executive Director and Head of PRO Assessment, Bristol Myers Squibb

18:00 **Panel Discussion**

Moderators: Pierre Demolis (EMA) and Madeline Pe (EORTC)

Mogens Groenvold, University of Copenhagen and Bispebjerg/Frederiksberg Hospital
Chantal Quinten, EMA
Hans Schuerer, Vice-Chair, WECAN
Jill Bell, Head of Measurement Science Center of Excellence, Oncology R&D, AstraZeneca
Michael Schlichting, Director Biostatistics, Merck Healthcare KGaA
Corneel Coens, Lead Statistician, EORTC
Ashley Wilder Smith, Chief Outcomes Research Branch, US NCI

18:45 **Closing Remarks**

Wrap up and conclusions — 15'
Peter Mol, CHMP and SAWP member, MEB, EMA

- Discussion on the tools and measures to use
- Discussions on case examples
- Like for any other endpoint, need for consistent methodological standards and clear research objectives

Conclusion

- Engaging with patients in medicines evaluation:
  - Very positive experience to date
  - Brings relevant outcomes for patients, such as PROs and HRQoL, into scientific discussions
  - Increase transparency - Update of Assessment Report
- New Regulations coming in Europe:
  - HTA Regulation to be implemented in January 2025 for oncology medicines
  - Pharma legislation
- Workshop aimed towards convergence between Regulators and HTAs on use of PROs and QoL data in decision making for the patients benefit

Thank you for your attention

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