EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA current and future activities on Patient Experience Data (PED), including PROs and HRQoL in medicines' development and evaluation

EMA and EORTC workshop: How can PRO and HRQoL data inform regulatory decisions

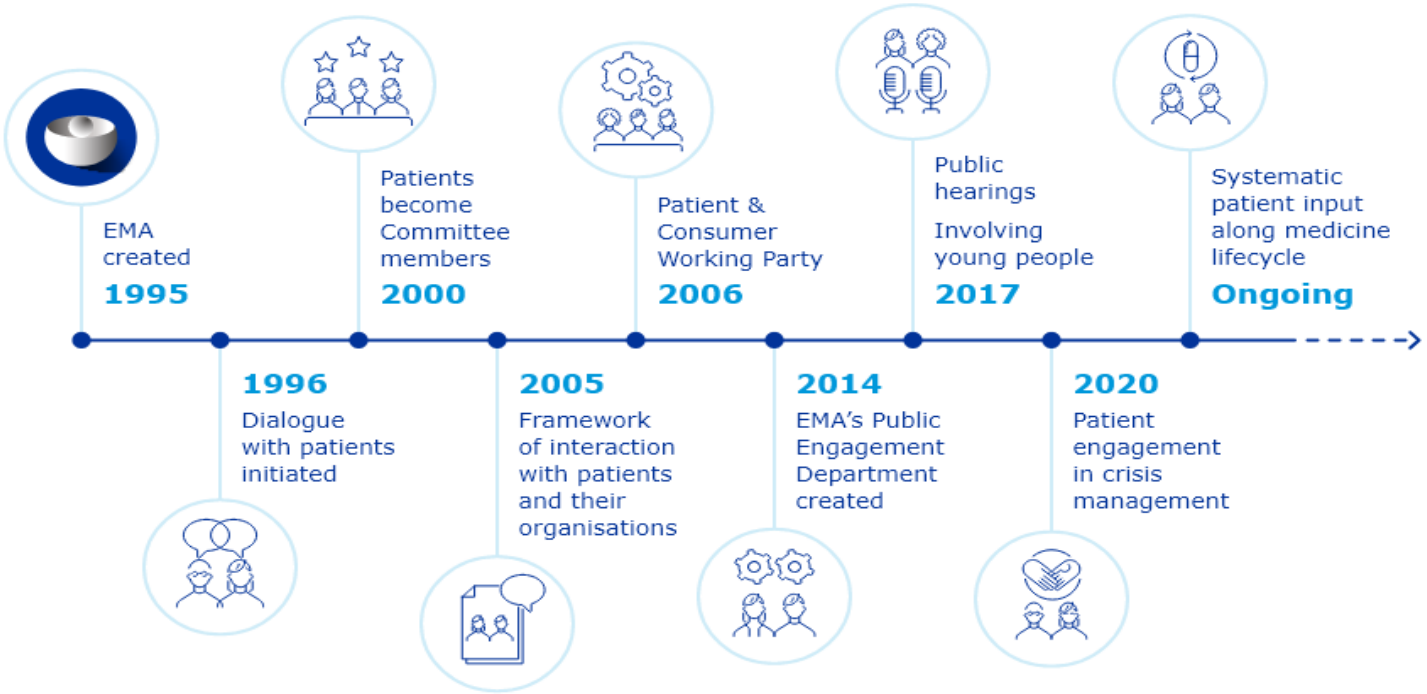
Presented by Juan Garcia Burgos on 29 February 2024
Head of Public and Stakeholders Engagement Department

An agency of the European Union

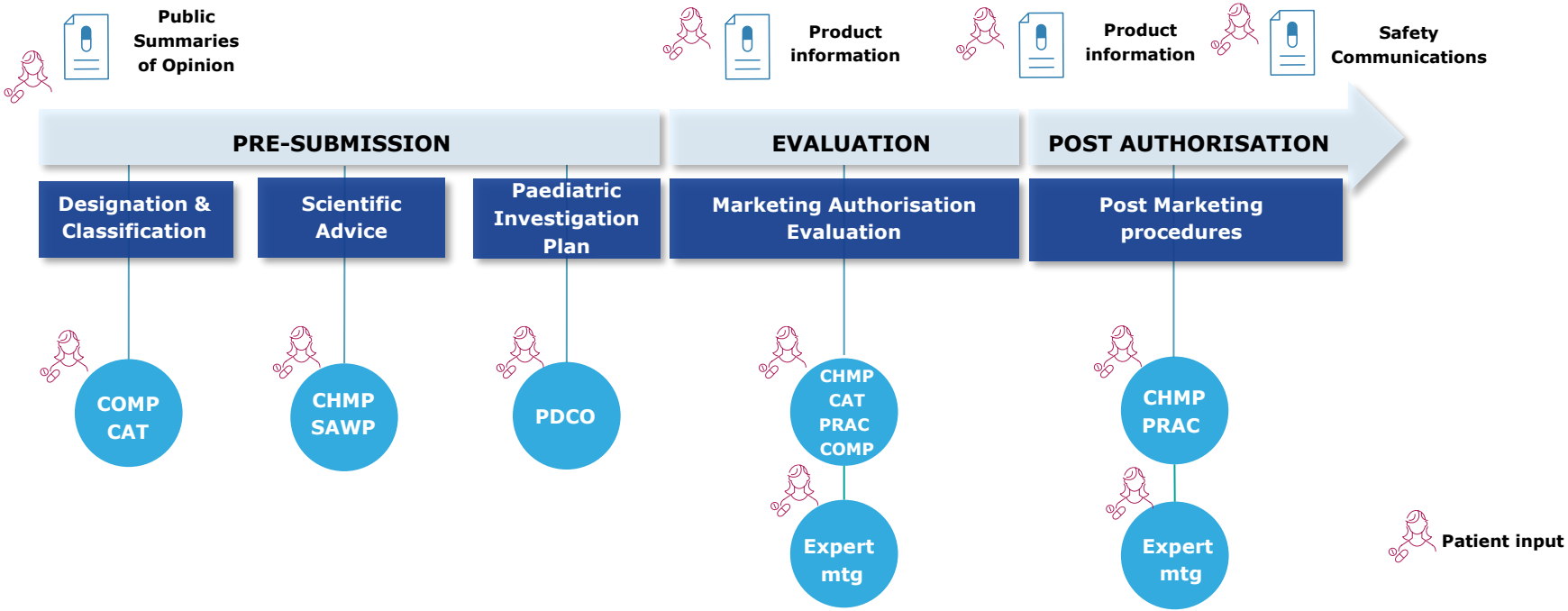
Outline

- EMA's journey of patient involvement
- How patients participate in EMA regulatory activities
- Definitions of Patient Experience Data (PED)
- Why is Patient Experience Data important?
- Status of Patient Experience Data in the EU
- Reflection Paper on Patient Experience Data
- Scientific advice and qualification of novel methodologies
- Conclusions

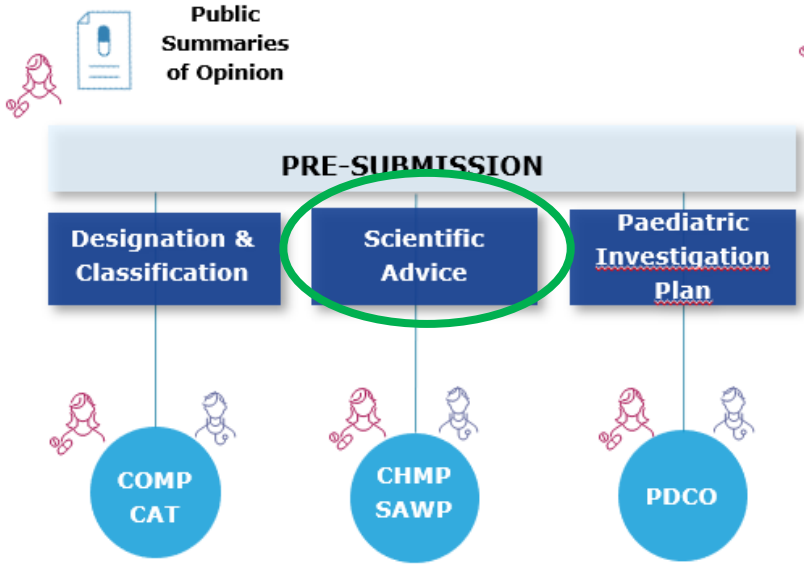
EMA's journey of patient involvement

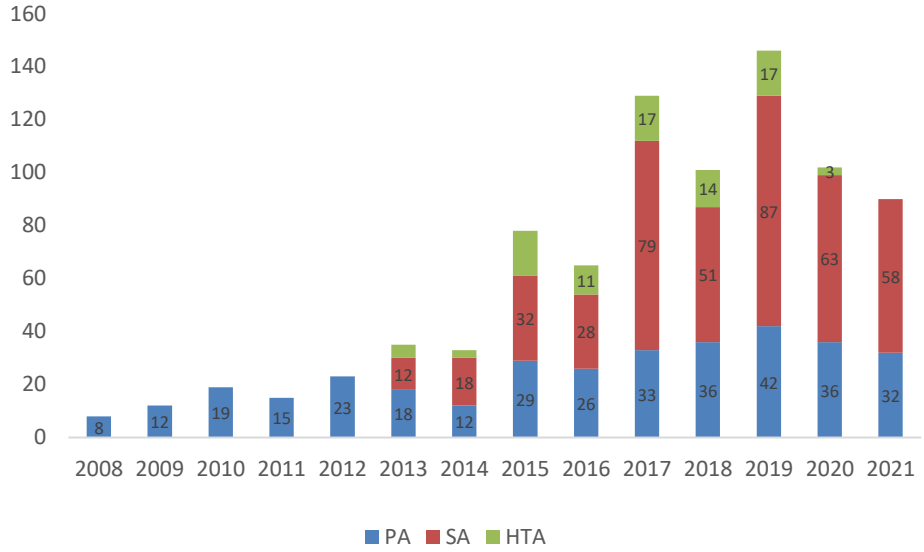


How patients participate in EMA regulatory activities



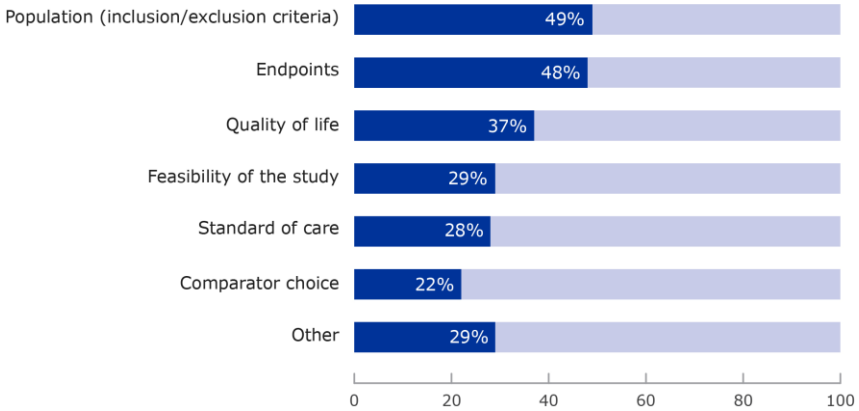
Patient Engagement in pre-submission phase: Scientific Advice



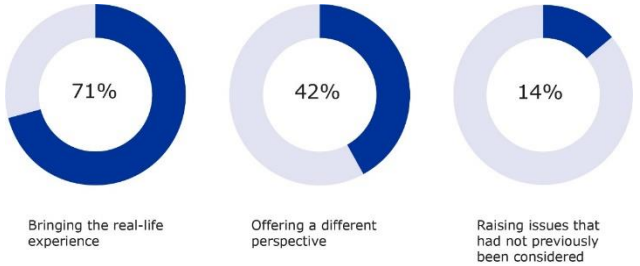


Published in Frontiers in Medicine

Where patients gave input



Added value of patient input and involvement

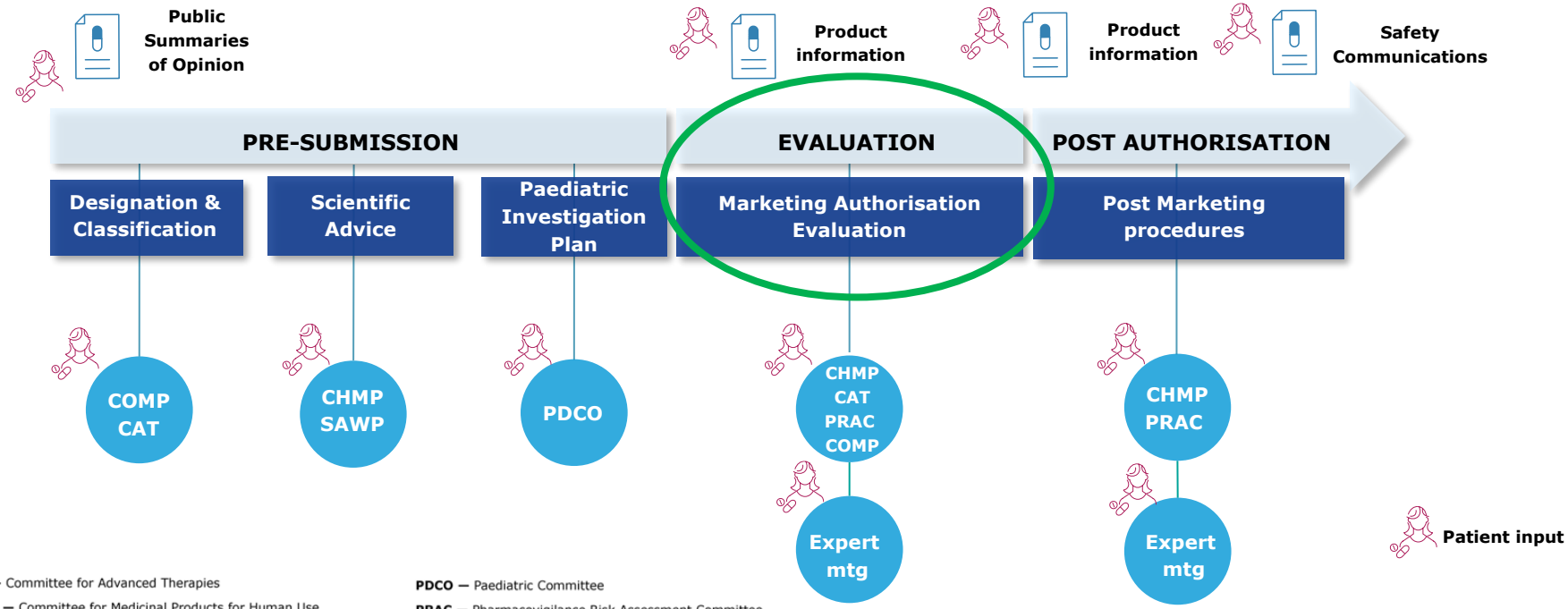


Patient input resulted in further reflection in **52%** of cases.

20% of cases - recommendations made to the developer were modified based on patient contributions.

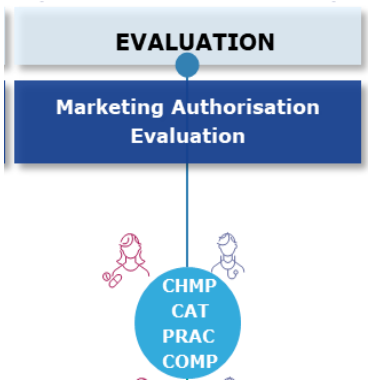
>85% cases: patient **agreement** with the proposed development plan.

Patient involvement in the medicines regulatory lifecycle



CAT — Committee for Advanced Therapies
CHMP — Committee for Medicinal Products for Human Use
COMP — Committee for Orphan Medicinal Products
PDCO — Paediatric Committee
PRAC — Pharmacovigilance Risk Assessment Committee
SAWP — Scientific Advice Working Party

Patient Engagement in evaluation phase: CHMP



EVALUATION

Day 1: Start

Consultation of ORGANISATIONS

Day 80: Assessment Report

Day 120: List of Questions

Day 150: Joint Assessment Report

Day 180: List of Outstanding Issues/ Oral explanation

Day 210: Opinion

Day 277: Commission Decision

Scientific Advisory/ad hoc expert Groups

Oral explanations

Consultation of INDIVIDUALS

Information from patients during early contact with CHMP

- daily impacts,
- treatment options,
- perspectives and perceptions of adverse effects,
- what constitutes important improvements and
- desired benefits for new treatments

Can include information on PROs and HRQoL

PATIENT / CARER EXPERIENCE OF:

<condition>

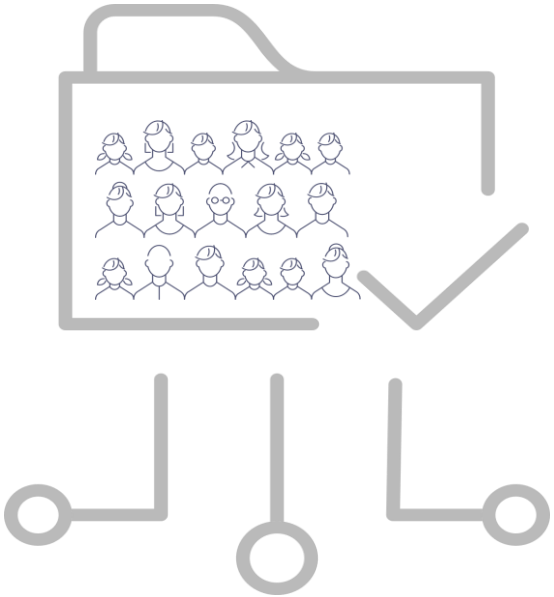
Please include below any aspects that are of particular importance to patients/carers, such as quality of life, standard treatments and how acceptable they are, therapeutic/unmet medical needs, what benefits they would hope for in new medicines as well as what level of side effects they would consider acceptable.

- Highlight if there are large differences between groups of patients/carers about these aspects or if these views are generally similar across the condition.
- Please also mention any aspects about the condition or its treatments that you feel are not well-understood or not sufficiently considered.
- Please include anything else you feel is important for EMA to know. Try to keep your main points to 1-2 pages, if necessary, include more details in an appendix.

Please do not include any individual patients contact details or health data.

☐ Tick here to confirm you give consent for EMA to share your views anonymously with third parties, as applicable.

Definition of Patient Experience Data in the EU



- **Data reported directly by patients or their carers**, without interpretation by clinician

Proposed EU definition as part of the EMA 2022 workshop:

Data collected via a variety of patient engagement activities and methodologies *to collect patients' experience of their health status, symptoms, disease course, treatment preferences, quality of life and impact of health care*

- **Reflects patient experience and preferences** of medicines and their views on their conditions

Definition **to be agreed with stakeholders**

Types of Patient Experience Data

- **Patient Reported Outcomes (PROs)** refer to a health/treatment outcome reported directly by the patient without the interpretation of a clinician or another person.
- **Patient Preferences (PPs)** refer to how desirable or acceptable is to patients a given alternative or choice among all the outcomes of a given medicine.
- **Patient Engagement (PE)** refers to all activities involving interaction with patients to gather their experience on disease, preferences, outcomes and treatments.

- not only **quantitative sources** of evidence (e.g., PROs, PREMs) **but also qualitative sources** (i.e., information obtained as part of patient engagement activities reflecting broader patient perspective e.g., outcome of focus groups)

- **Patient Experience Evidence (PEE)** is patient experience data **qualified as valid scientific evidence following a scientific assessment**

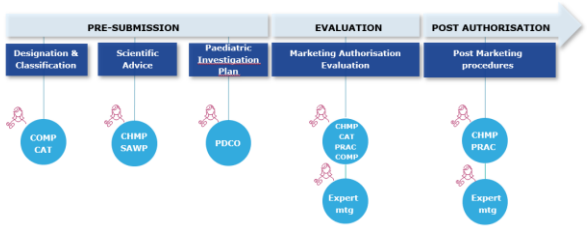
*Defined and agreed during the Multi-stakeholder workshop (Sept 2022)

Why is Patient Experience Data important?

- Patients are **users of medicines**
- Patients are experts **in their disease and treatment**
- PED helps ensure **more patient-relevant outcomes**
- Patients are instrumental in helping to **optimise medicines development and regulatory decision-making**

Patient experience data is relevant at different stages:

- During **clinical trial design**
  - Selection of endpoints (which matters more to patients)
- During **benefit-risk assessment**
  - Patient preferences (trade-offs)
- Post-marketing for **Pharmacovigilance and Risk Minimisation**
  - Adverse Drug Reaction reporting



Multi-stakeholder workshop (Sept 2022)

Status of PED in the EU

- 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 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 2023 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**

Update on progress

√ 2022

EMA workshop on PED

√ 2023

PED cross-Agency group & Action Plan

√ 2023

PED Expert group

2024

Reflection Paper & Public Consultation

Update of Assessment Report

Upcoming reflection paper on EU approach to PED

- **Reflection paper:** framework for discussion or clarification particularly **in areas where scientific knowledge is fast evolving or regulatory experience is limited**
- **General EU framework or principles** – not a methodological guidance
- **Key action** derived from the 2022 PED workshop - requested by stakeholders
- Reflection paper is in the **Work Programmes of both CHMP and PRAC**
- **Publication for public consultation expected Q3 2024**

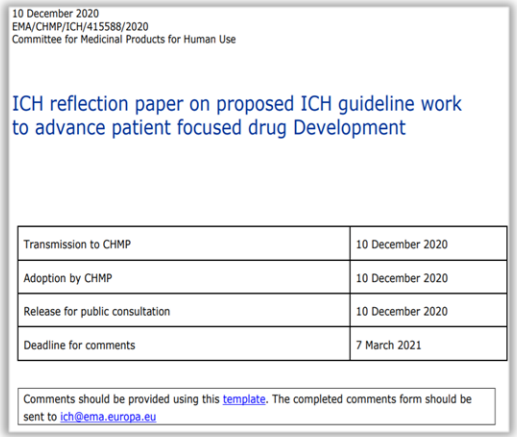
EU reflection paper to complement ICH Guidelines on PED

- Proposal for **new ICH guidelines** will provide **globally harmonized approach** to inclusion of **patient's perspective in a methodologically sound** way, to improve quality, relevance, safety and efficiency of drug development and to inform regulatory decision making.

  1) Focus on informing the drug development process, patient-reported outcomes

  2) Focus on patient preferences regarding benefits and risks

- Scope of Reflection Paper will differ from that of ICH guidance
  - Reflection paper will not cover specific methodological guidance



10 December 2020
EMA/CHMP/ICH/415588/2020
Committee for Medicinal Products for Human Use

ICH reflection paper on proposed ICH guideline work to advance patient focused drug Development

Transmission to CHMP	10 December 2020
Adoption by CHMP	10 December 2020
Release for public consultation	10 December 2020
Deadline for comments	7 March 2021

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

Scientific advice & qualification of novel methodologies

The EU approach is to encourage companies to liaise early with regulators during Scientific Advice or Qualification, to discuss best way to generate and collect PED, and have a case-by-case discussion on their specific development plans

Scientific Advice

- **The developer of a medicine presents plans to develop a medicine** and identifies questions and possible solutions.
- **EMA gives advice** on the developer's proposals
- Scientific Advice can be provided on **any PED scientific question** (e.g., clinical trials)

Qualification of novel methodologies

- **Opinion on the acceptability of a specific use of a PED method**, such as the use of a novel PROs
- **Advice on protocols and methods** intended to develop a novel method with the aim of moving towards qualification

Conclusions

- **Engaging with patients in medicines evaluation**
  - Very positive experience to date
  - Brings relevant outcomes for patients, such as PROs and HRQoL, **into scientific discussions**
- **PED is a new scientific discipline**
  - **Collaboration of multi-disciplinary experts** and stakeholders is needed
- **EMA is working to progress on key PED deliverables**:
  - **Reflection paper** on the best EU approach to generate, collect and analyse PED
  - **Increase transparency** - Update of Assessment Report

Thank you
Any questions?

Further information

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