# Multi-stakeholder workshop on Patient Experience Data

Patients and Consumers Working Party/Healthcare Professionals Working Party,
15 November 2022

Presented by Rosa Gonzalez-Quevedo
Public and Stakeholders Engagement Department

An agency of the European Union

# Disclaimer

These PowerPoint slides are copyright of the European Medicines Agency.
Reproduction is permitted provided the source is acknowledged.

The presenter does not have any conflict of interests.



Classified as public by the European Medicines Agency

# Outline

- Patient Experience Data in the EU:
  - Importance and State of play in the EU
- Multi-stakeholder dialogue to progress on Patient Experience Data
- Key outcomes of workshop:
  - Common understanding of Patient Experience Data in the EU
  - Current methodologies
  - Challenges and Actions



Classified as public by the European Medicines Agency

# Why is Patient Experience Data (PED) important?

- Patients are ultimately the **end-users of medicines**
- Patients are experts **of their disease/ condition and treatments**
- PED helps ensure **more patient-relevant outcomes**
- National healthcare systems are faced with challenges to reimburse innovative treatments and may need to make **informed choices based on robust evidence**

  including **patient centric - RWE generated pre- and post-marketing**
- Users of medicines become instrumental in helping to **optimise medicines development and regulatory decision-making**



Classified as public by the European Medicines Agency

# State of play

- Although there has been much progress in the EU in recent years, **PED are still not systematically included** in all aspects of medicines development and regulation
- Reinforcing patient relevance in evidence generation is **a key priority** in the EMA's **Network Strategy** and the **Regulatory Science Strategy**
- Patient experience data is also relevant in the context of **the implementation of the new Health Technology Assessment (HTA) regulation**, thus in **value assessments** that inform subsequent decisions by payers
- **Guidance work ongoing at ICH for global harmonisation** of Patient Experience Data (PED)



Classified as public by the European Medicines Agency

# Multi-stakeholder dialogue to progress on PED

## EMA workshop on 21 September 2022


### Workshop objectives

- **Common understanding on PED definition** in the EU, including patient engagement, patient preferences and patient reported outcomes.
- **Current methods for collecting and incorporating patient data** into medicines development and regulatory assessments
- How **direct patient data collection from real-world healthcare** can be leveraged and used
- **Priorities** to enhance the collection and use of patient experience data

Classified as public by the European Medicines Agency

# Key outcomes

## Common understanding of Patient Experience Data in the EU

- **Patient Experience Data (PED)** are **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
- PED includes:
  - **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.



Classified as public by the European Medicines Agency

# Key outcomes

## Common understanding of Patient Experience Data in the EU

- **For EU regulators**, PED does not only involve quantitative sources of evidence (e.g., PROs, PREMs) **but also qualitative sources** (i.e., any information obtained as part of patient engagement activities that reflect the wider perspective of patients' experience e.g., outcome of focus groups)

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



Classified as public by the European Medicines Agency

# Key outcomes

## Current methodologies

- Collection methods and analyses must be **fit for purpose and produce reliable data**

- **Quantitative and qualitative** methodologies have been developed for collecting and analysing PED, including **methodologies based on engagement**, but **more is needed**



Classified as public by the European Medicines Agency

# Challenges and Actions - Alignment among decision-makers needed

- ✓ Most challenges can be addressed through **effective research design, collaboration and dialogue between different stakeholders and early patient involvement**
- ✓ Agreed to continue **multilateral stakeholder cooperation** to obtain the best regulatory outcomes, and to explore **additional engagement opportunities** (e.g. focus groups) for key topics
- ✓ Key to ensure from early stages that, in addition for regulatory purposes, **PED needs to be useful for other decision makers such as health technology assessments**



Classified as public by the European Medicines Agency

# Challenges and Actions - Need for regulatory guidance

- ✓ **The Agency will elaborate a position paper** (reflection paper) to provide advice on the best EU approach to generate and collect PED
- ✓ This will help provide **clarity on the process and support mechanisms at EMA, while further collaboration at ICH level continues**



Classified as public by the European Medicines Agency

# Challenges and Actions - Need for further transparency on decision-making

- ✓ EU regulators will explore how to **better reflect in the assessment report the way PED is assessed** as well as the rationale for acceptance/exclusion for Benefit/Risk decision making in the AR
- ✓ Further consideration should be paid to the way **PED is reflected in the product information**
- ✓ For orphans, **PED is also important for discussing significant benefit** at time of reviewing the maintenance of the status at time of marketing authorisation application, and it can also be explored how to best reflect PED in the orphan maintenance assessment report
- ✓ Stakeholders will also look at **how to increase transparency using modern channels**

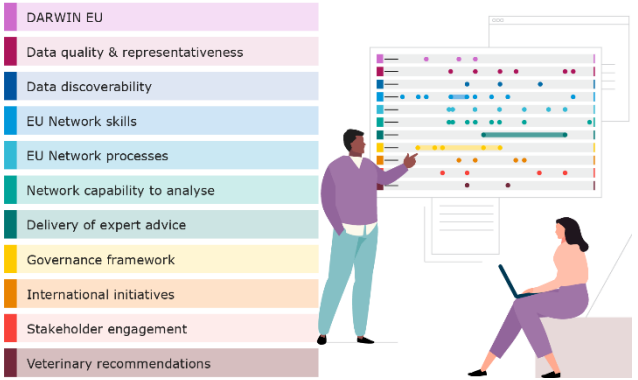


Classified as public by the European Medicines Agency

# Challenges and Actions - Digitalisation of patient-generated health data

- ✓ **Integration with ongoing activities in the European Health Data Space and the Big Data work plan 2022-2025** are ongoing, and the patient voice will continue to be gathered and used in these forums.

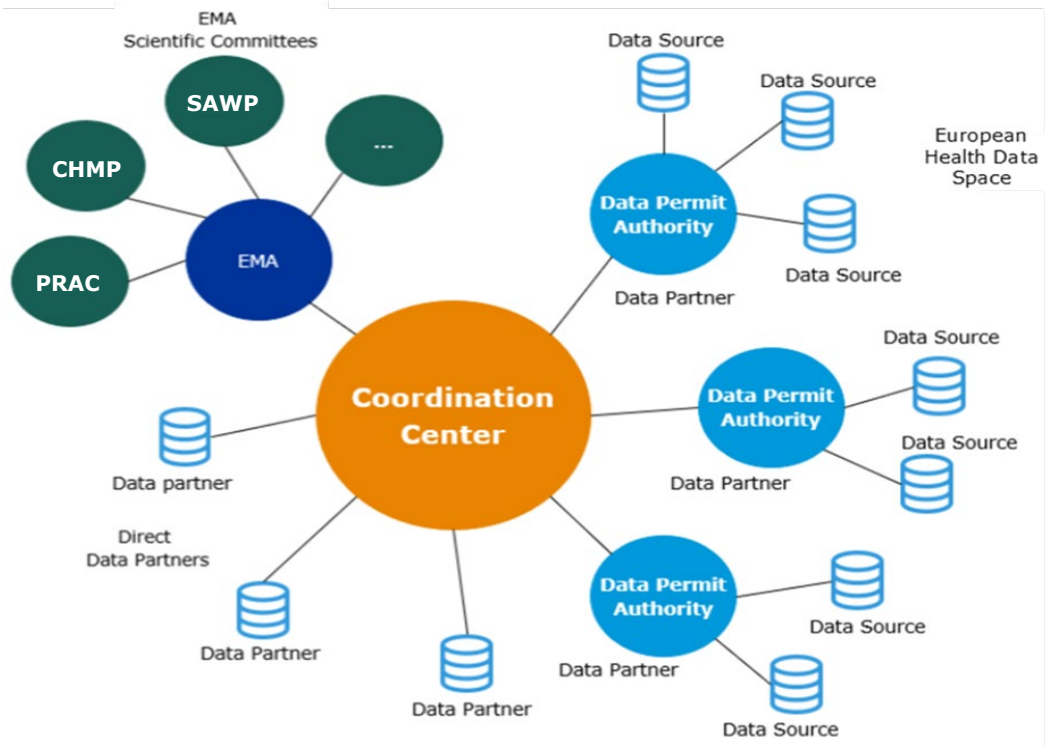
- ✓ In particular, recommendation 3 on data discoverability includes a **review on the utility of eHealth data in Q4 2023**






Classified as public by the European Medicines Agency

# DARWIN EU® will explore how to capture and generate analyses of PED




- ✓ **PED such as PROs may be held and analysed by DARWIN**
- ✓ **Patient representatives involved** in Big Data Steering Group / DARWIN discussions
- ✓ Work on **Big Data and PED closely coordinated** at EMA



Classified as public by the European Medicines Agency

# Key outcomes

## Challenges and Actions - Need for resources and technical expertise

- ✓ As part of the overall strategic plan to advance PED generation, the Agency will look into different **options to increase capacity and adequate training**
- ✓ This includes **training in areas relating to digital data** included in the Big Data work plan 2022-2025, specifically in the recommendation 4 on strengthening the EU Network skills by offering training modules to patients, healthcare professionals & academics in Q4 2023



Classified as public by the European Medicines Agency

# Key outcomes

## Challenges and Actions - Overall strategy on Patient Experience Data

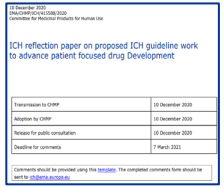
- ✓ Encourage **collaboration and dialogue between different stakeholders** and early involvement of patients.
- ✓ EMA recognized as optimal to facilitate stakeholder discussion and progress in this area
- ✓ On the basis of the workshop's outcomes, **EMA will enable discussions within the Network on current status, next steps and how to monitor progress**



Classified as public by the European Medicines Agency

# Conclusions






- **Reinforcing patient relevance in evidence generation** is a key priority for the Network
- **Guidance work ongoing at ICH for global harmonisation**
- **EU Multi-stakeholder approach** for defining robust and meaningful Patient Experience Data for regulatory decision-making
- EMA to work on a **reflection paper on the best EU approach to generate and collect PED**
- Robust methodology needed to capture and analyse what matters most to patients, to optimise **medicines development, regulatory decision- making and HTA assessments**
- **New digital tools for clinical data generation and DARWIN** offer wide opportunities for optimising clinical data generation and analysis to 2030



Classified as public by the European Medicines Agency

# Acknowledgments


- Nathalie Bere
- Juan Garcia Burgos
- Melanie Carr




Classified as public by the European Medicines Agency

# Thank you for your attention

## Further information

Contact me at rosa.gonzalez-quevedo@ema.europa.eu

**Official address** Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
**Address for visits and deliveries** Refer to www.ema.europa.eu/how-to-find-us
**Send us a question** Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

Follow us on **@EMA_News**

Classified as public by the European Medicines Agency