Follow-up on strengthening patient-centric development

9th Industry Stakeholder Platform on Research and Development Support

Presented by Juan Garcia Burgos on 5 December 2022
Head of Public and Stakeholders Engagement Department

An agency of the European Union

Feedback from the multi-stakeholder workshop

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

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.

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**

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**

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**

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**

Key outcomes

Challenges and Actions - Need for resources and technical expertise

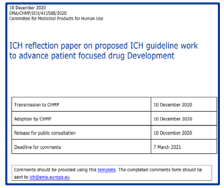
- ✓ As part of the overall strategic plan to advance PED generation, the Agency will look into different **options to increase capacity and adequate training**
- ✓ This includes **training in areas relating to digital data** included in the Big Data work plan 2022-2025, specifically in the recommendation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

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**

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

Acknowledgments

- Nathalie Bere
- Rosa Gonzalez-Quevedo
- Melanie Carr

Update on the pilot seeking early patient input in Marketing Authorisation Application (MAA) review

Background

- Patients are involved at various timepoints along the medicines' regulatory lifecycle at EMA – the value of including their perspectives is well acknowledged
- Requests for patient input often come at a late stage of the evaluation (e.g. SAG/expert meeting, oral explanation)
- We explored how current practices could be enhanced to minimise missed opportunities
- Proposal for pilot to foster earlier dialogue with patient organisations
- To complement other engagement methodologies and facilitate further interactions as the procedures progress, if needed
- Pilot started January 2021

Pilot methodology: early contact with patient organisations

- Relevant patient organisations contacted at start of orphan MAA's by EMA.
- Patient organisations invited to share key aspects from their perspectives of living with the condition, so CHMP can be aware from the beginning.
- Patient organisations given 3-4 weeks to respond (in advance of first AR).
- Information from patient organisations shared with (Co-)Rapporteurs (and the company for transparency) - Rapps decide if information provides added value, is useful for assessing the dossier, and if merits being included in AR.
- To assess contribution and value of patient input received during pilot, short questionnaire sent to (co-) Rapporteurs (and to patient organisations) for feedback.

Pilot outcome

- Pilot ran from January 2021 to May 2022 (17 months).
- **37** MAAs included
- Wide range of therapeutic areas included (orphan status)
- Range of information received from patients:
  - Individual testimonies, survey results, websites, links to articles
  - A few paragraphs to several pages in length.

Among 17 patients surveyed, pain and fatigue are the most frequent symptoms

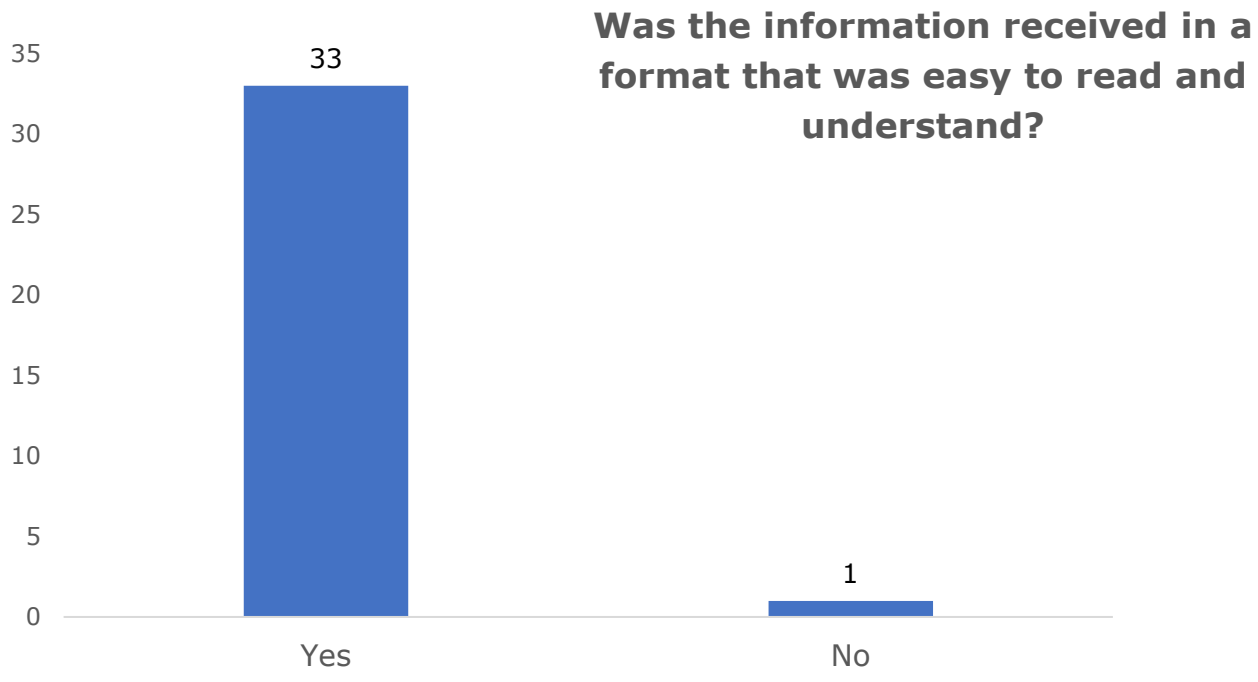
Crises and complications can turn their lives upside down at any time - fear of the onset of a crisis is the patients obsession

Patients often use off-label medicines

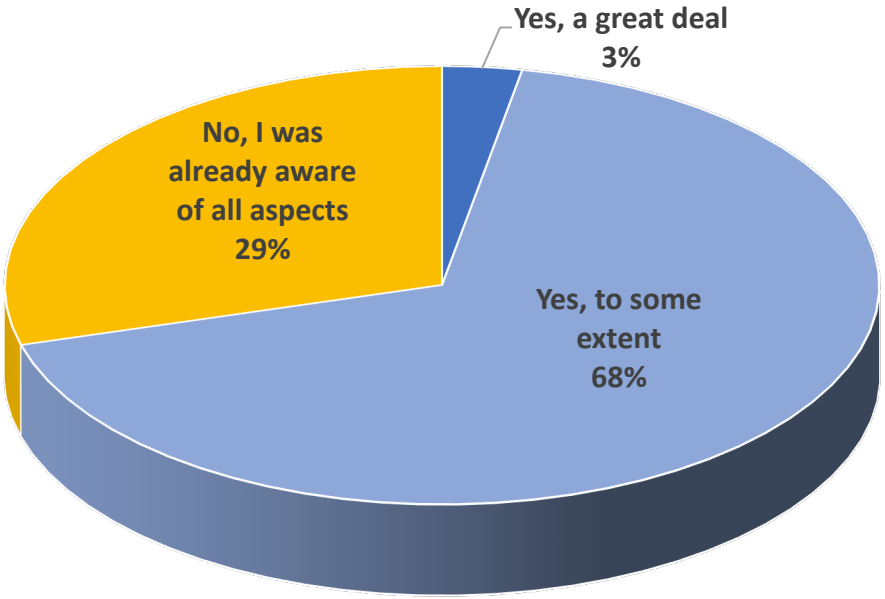
Parents often reluctant to accept an aggressive treatment when they see their child suffering with side effects

Quality of life affects so many aspects of daily life

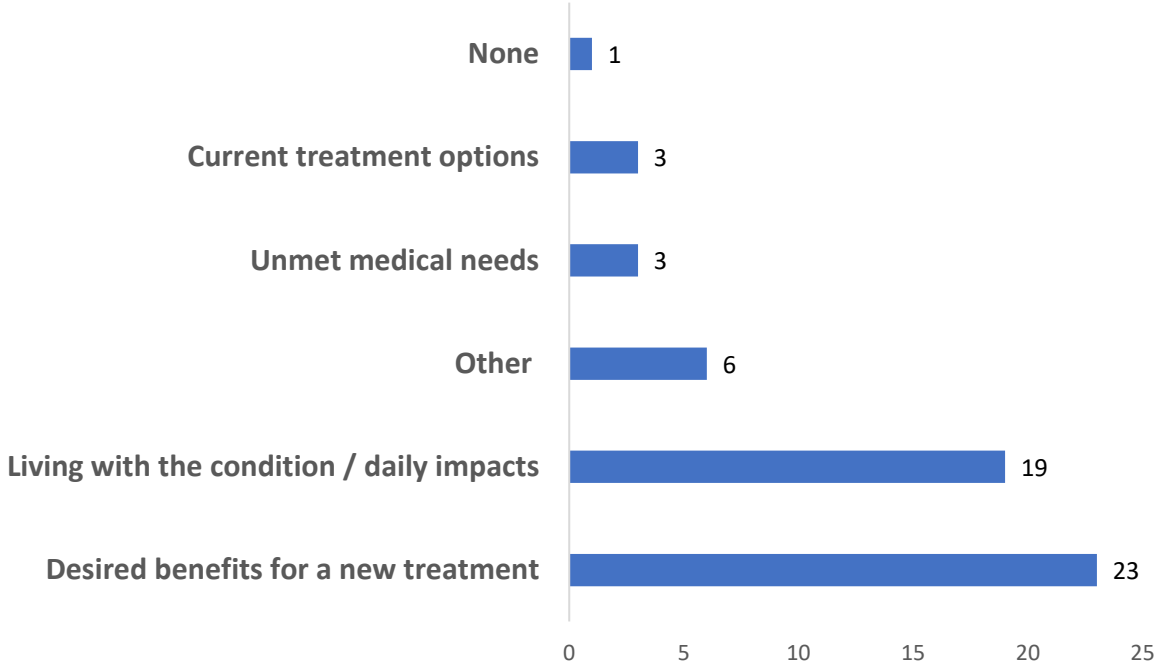
A treatment-naive patient is ready to accept more side effects than a patient switching between products



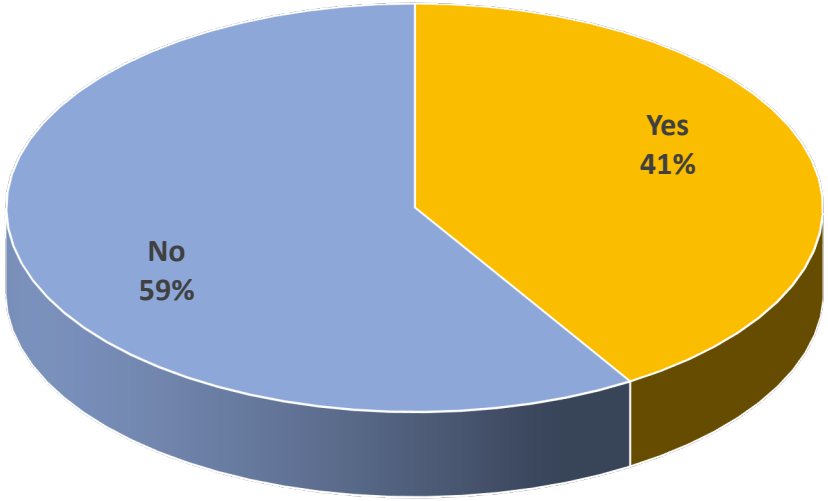
Did the information from the patient organisation highlight aspects that you were not already aware of?



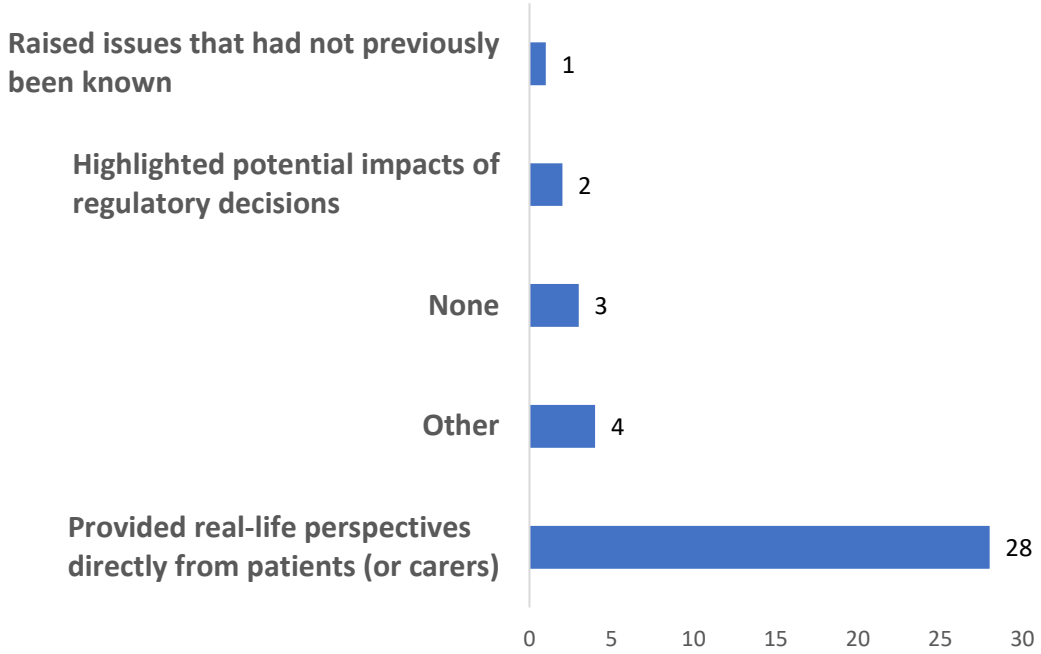
Which aspect(s) of the patient information were most useful / insightful?



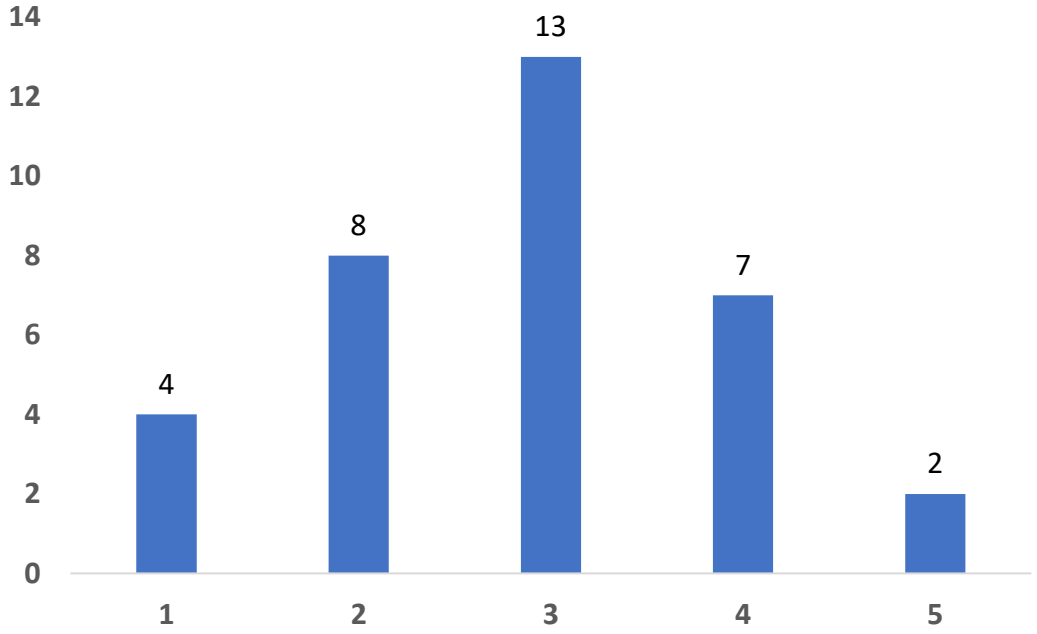
Did any of the patient information contribute to the development / content of first assessment report?



What was the added value of the information received from the patient organisation (if any)?



Overall, on a scale of 1 to 5, how useful did you find the information received from the patient organisation (5 being highest)



Pilot outcome summary

- Good number of cases; 37 procedures over 17 months (2 months no OD)
- Overall feedback received from (Co-)Rapporteurs is positive and reflects usefulness and benefit
- 41% of cases contributed to the development of the first assessment report
- Information from patients related to *daily impacts*, *treatment options*, *perspectives and perceptions of adverse effects*, *what constitutes important improvements and desired benefits for new treatments* have proven to be insightful / helpful
- Initial feedback from patient organisations is positive; increases trust in the work of the CHMP, also helps with contacting relevant patients later in the assessment if requested by EMA

Way forward

- Pilot results were presented to all CHMP - further positive feedback was received.
- It was agreed to establish contacting patient organisations at start of new MAAs for orphans as a regular procedure, and also include products with new indications where no therapies currently exist.
- We will also explore reaching out to healthcare professionals in same manner.
- Work will be undertaken to define best way to reflect this input systematically in the CHMP AR, with possible improvement to AR template.
- This is an important milestone in providing an additional methodology to capture and integrate patient experience data within medicines assessments at EMA and avoid missed opportunities to include their voice from the beginning of new MAA evaluations.

Acknowledgments

- Nathalie Bere

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
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Highlights of additional comments received in survey responses

We would like to express our gratitude and support the further involvement of patients in the decision-making process. Assessors must consider and balance between statistical significance and clinical relevance. Often, we cannot see things from the patient's point of view - potential new drugs should be able to meet the expectations of those to whom they are intended

Daily impact, current treatment options and desired benefits for new treatment were very useful to gain insight on the disease and its consequences.

Sometimes it can be unclear to assessors how important improvements in certain disease parameters are. What seems to us to be of less important can make a significant difference to patients in their daily functioning. That input will always be appreciated.

Preferably, input from patient organisations is asked at time of pre-submission meeting, so input is available at start of procedure and can be taken into account easier than at a later stage

It would be helpful if the input would be more targeted to the current dossier.

The information was not easily readable and consisted of unorganized paragraphs.