

# Multi-stakeholder workshop: *Patient experience data in medicines development and regulatory decision- making*

21<sup>st</sup> September 2022, European Medicines Agency

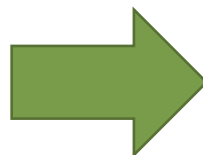
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# How patient engagement can contribute to the development and approval of medicines?

EURORDIS contributes to the engagement of patients in different EMA activities by involving and supporting its members in:

- EMA Scientific Committee members (i.e., COMP, PDCO, CAT, PRAC)
- Protocol Assistance/Scientific Advice
- Scientific Advice Groups (SAGs)
- Surveys, consultations
- Product information/Safety issues
- EMA stakeholders dialogue...



1. Patient Experience Evidence
2. Having a common regulatory framework and supportive processes
3. Opportunities in the upcoming pharmaceutical regulation
4. Strategic alignment

EURORDIS supports the work of the **rare disease patient representatives' members in the different EMA Scientific Committees** which represents **308 days/year approx.**

In addition, during **2021 EURORDIS helped EMA identifying and involving 34 patients in Protocol Assistance (90% of the dossiers requiring patient input).**

# 1. Patient Experience Evidence

- Patient Experience Evidence vs Patient Experience Data.
- Sources of evidence:
  - Quantitative: PROMs and PREMs.
  - Qualitative: interviews/videos/focus groups.
- Individual heterogeneity vs aggregated evidence.

## 2. Having a common regulatory framework and supportive processes

- Guidelines on the collection of patients outcomes and patient experience evidence for developers and patient organizations, and how it will be used in the assessments to enhance a common regulatory framework.
- Supportive processes:
  - EMA scientific advice to support the qualification of novel technologies as well as novel PROMs, PREMSs, etc.
  - Informal meetings with patient organizations to discuss the methodology and design of their surveys, ensuring that what is collected will be accepted for all the stakeholders.

### 3. Opportunities with the upcoming revision of the pharmaceutical regulation

- Include a new regulatory requirement: systemic inclusion of patients' experience in the submission of the marketing authorization application (e.g., US FDA's Patient-Focused Drug Development Guidance).
- Involve patients' representatives to provide their views at the marketing authorization level.

## 4. Strategic alignment

- Strategic and early alignment between EMA and HTA as well as payers.
- International regulatory cooperation, consider creating a specific cluster.
- Resources to support this process.

**Thank you for your  
attention!**