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

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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!

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