Session 3: Patient Experience Data (PED)- Realising the potential of PED in EU medicine regulation

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Current challenges

- **Lack of Standards:**
  - The absence of harmonized standards for capturing and reporting PED results in fragmented and inconsistent data.
  - Difficulties in meeting regulatory requirements, due to data inconsistencies.
- **Methodological Shortcomings:**
  - No established methodology for the process of capturing PED, leading to ad-hoc and inconsistent data collection.
  - This inconsistency fails to effectively enhance patient care.
- **Cultural Gaps in Patient Organizations:**
  - Limited culture within patient organizations for structured, systematic data capture.
  - Challenges include lack of awareness, inadequate training, and resistance to technological adoption.

Major Contribution to Patient Care

- **Aims**: incentivise development of potential improved medicinal products that can bring meaningful advantages for patients.
- **What could represent a MCPC?**
  - Ease of self-administration: e.g.
    - Allows ambulatory treatment over hospital, or
    - is significantly more convenient and reduces treatment burden OR
  - Significantly improved adherence due to new pharmaceutical form (e.g. modified release formulation) in case there is
    - Documented difficulties with existing form (should be documented in *reviewed publications, patients' registries or therapeutic guidelines*) and data showing better clinical outcome in patients with new form (may include better QoL)

Our proposal: Collaborare

- The aim to identify and share Major Contribution to Patient Care (MCPC) elements for each rare disease.
- Simple web tool designed to transform the way patient experience data is collected from patient organizations.
- Driving towards deeper understanding and innovative solutions and therefore support regulatory decision-making.

https://collaborare.azurewebsites.net/

The workflow

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Identify and contact the specific patient organizations for the condition

Collaborare Platform

A list of MCPC items is created using an AI language model to facilitate the process of identification of the key elements

Patient organization

The representative(s) of the patient organization will login into the tool to contribute

Edit the list – adding/removing items

Review the correct wording of the item

Rank the items

Validate the final list

Deliverables

The list of MCPC items can be consulted using the platform.

The same platform can be used to review and modify this list

A structured format is available in a public repository for open access

The final list to be used to in regulatory submissions to support assessments.

THANK ALL YOU FOR YOUR ATTENTION!

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