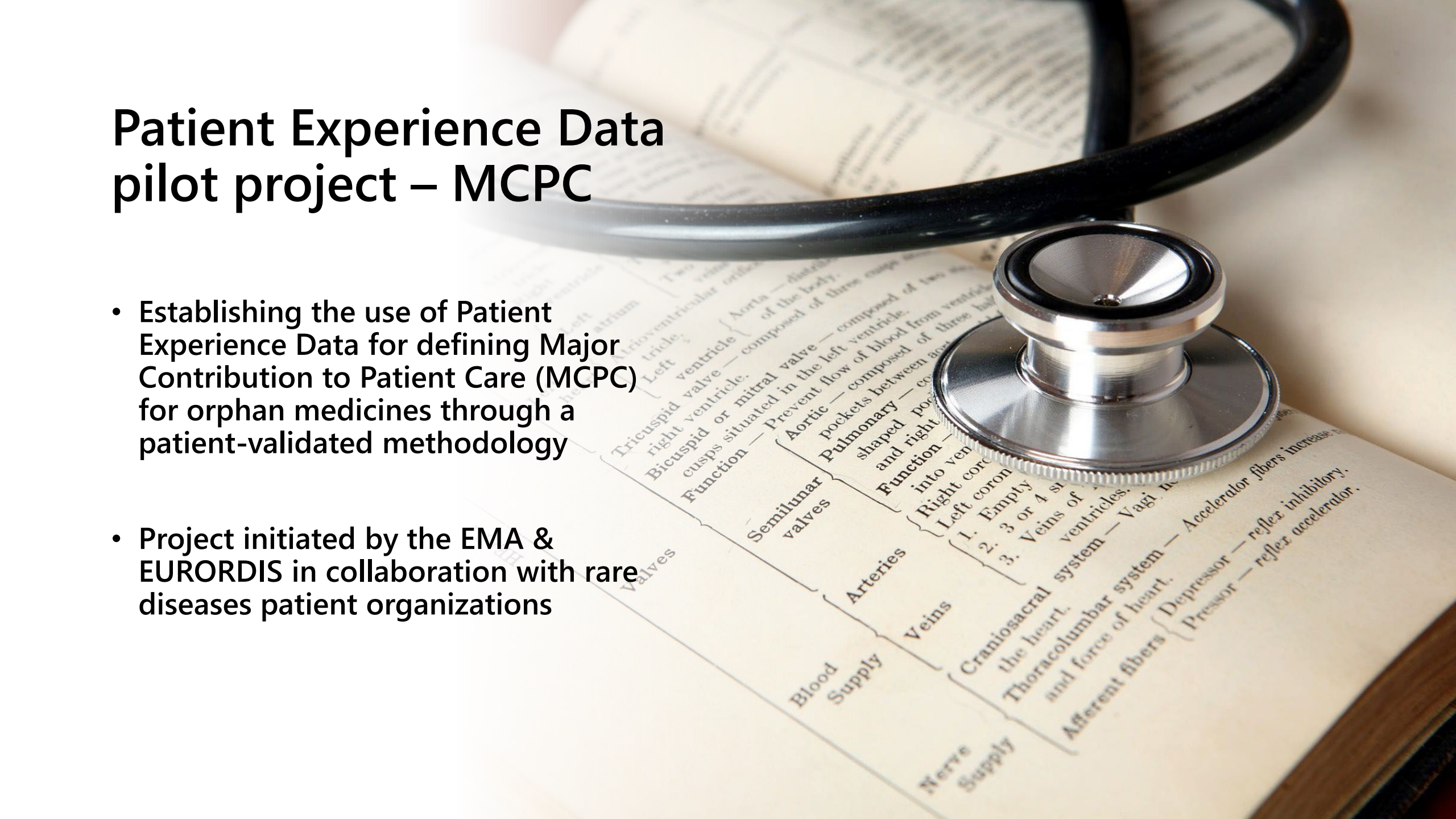
Patient Experience Data pilot project – MCPC

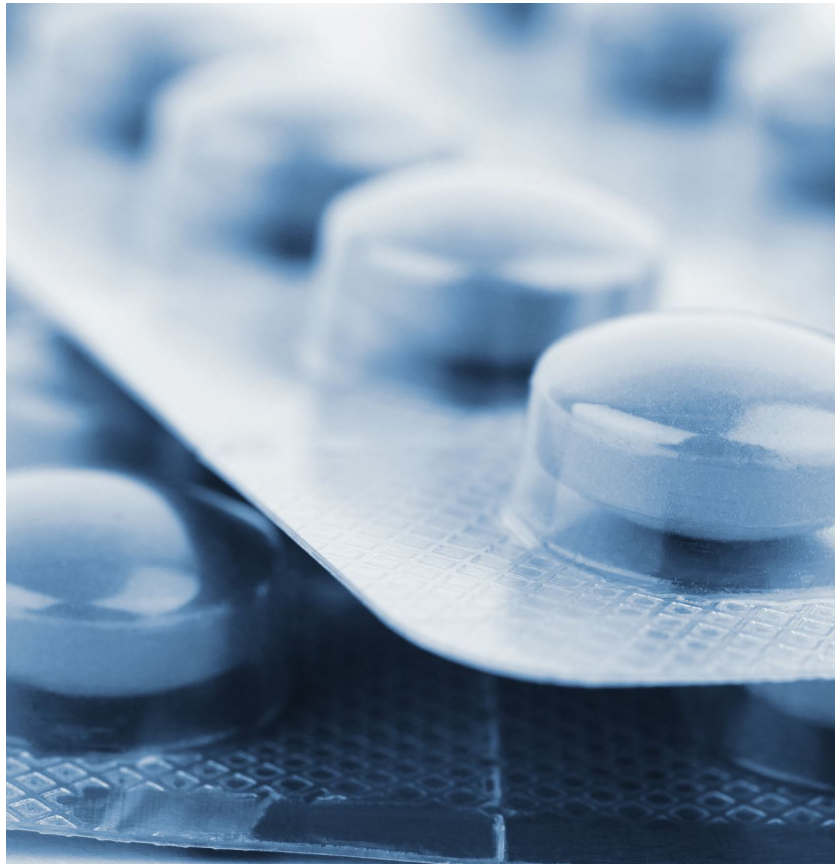
- Establishing the use of Patient Experience Data for defining Major Contribution to Patient Care (MCPC) for orphan medicines through a patient-validated methodology

- Project initiated by the EMA & EURORDIS in collaboration with rare diseases patient organizations



Criteria for Orphan Designation

- The condition is life-threatening or debilitating
- Prevalence of the condition is less 5/10.000 in European Union
- The existence of other methods for the diagnosis, prevention or treatment of the condition; if applicable, a justification of the medicine's significant benefit based on:
  - Clinically Relevant Advantage (CRA) based on improved efficacy or safety.
  - Major Contribution to Patient Care (MCPC) based on equivalent efficacy/safety and benefit/risk balance.



Major Contribution to Patient Care

Aim: 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)**
- **Challenges identified:**
  - **Lack of standards for PROMs codification**
  - **Lack of patient organization culture for capturing data**
  - **Absence of an established methodology to initiate the process**

Project proposal

EMA Regulatory Science Strategy 2025 – Patient Experience Data

Follow-up COMP Project on MCPC

Pilot project will aim to:
Propose a methodology for rare diseases patient organizations to capture and use patients' relevant information for enhanced patient care and explore how this can support regulatory decision making by the COMP

Pilot Methodology

- Use artificial intelligence to facilitate the process of identifying major contributions to patient care
- Listing major problems, needs and preferences perceived by patients living with a rare disease.
- Validated by patient organizations, including additional elements and ranking of issues.
- Data structuring into a simple validated list, easily usable format.

Example

This is a list of PROMs generated by GPT-4 using this prompt:

"You have to make a list of Patient Reported Outcomes for a rare disease. The disease is Dravet syndrome. The list must have specific items that can be evaluated by patients. You have to get the list from the comorbidities and symptoms of the patients. You have to be elements that contribute as a major contributor to patient care. The list has to have 10 items."

- Frequency of Seizures. How many seizures have you had in the past week?
- Intensity of Seizures. On a scale of 1 to 10, how would you rate the intensity of your seizures this week?
- Sleep Quality. How many hours of uninterrupted sleep have you had each night this week?
- Mood and Behavior. On a scale of 1 to 10, how would you rate your overall mood over the past week?
- Have you experienced episodes of irritability or aggression this week? If so, how many?
- Level of Fatigue or Energy. On a scale of 1 to 10, how would you rate your energy levels in the past few days?
- Cognitive Development and Motor Skills. Have you noticed any new difficulties in speech, memory, or motor skills this week?
- Feeding Difficulties. Have you experienced difficulties swallowing or eating in the past few days?
- Pain or Physical Discomfort. On a scale of 1 to 10, how much pain or physical discomfort have you experienced in the past week?
- Ability to Socialize. How many times have you socially interacted with friends or family members this week?
- On a scale of 1 to 10, how would you rate your ability to socialize this week?
- Impact on Family Life. How much do you believe your condition has affected your family's life this week, on a scale of 1 to 10?
- As a caregiver, how would you rate your level of stress related to caring for the patient this week on a scale of 1 to 10?

Patients' organizations role

Point of Contact: EURORDIS will act as the main point of contact between the project and the patient organizations. They will facilitate communication and ensure that patient organizations are actively involved in the validation process.

Knowledge Provision: Patient organisations, with its extensive knowledge and experience in the field of rare diseases, will provide insights into patient organization needs. Their input will be invaluable in shaping the project to ensure that it addresses the real-world challenges faced by patients.

Feedback Collection: EURORDIS will be responsible for disseminating the tool into patient organizations and promoting the use of the tool.

Representation and Advocacy: Patients organisations will represent the interests and voices of patients and patient organizations in the project, ensuring that the patient perspective is at the forefront.

Benefits

- Identifying and validating major issues faced by patients with rare diseases - quickly and efficiently.
- Informed regulatory-decision making – better quality data.
- Paving the way for more patient-centric regulations.
- Faster access to treatments, and more empowered patient advocacy.

Next steps

- Fine-tuning agreements.
- Present the project to the COMP.
- Present the project at the HMA/EMA Big Data Stakeholder Forum.
- Meet with the EMA rare diseases eligible patient organizations.
- Initiate the implementation.

Thank you for your attention!

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