PATIENT FOCUSED
MEDICINES DEVELOPMENT

made
with
patients

The PFMD
Book of Good Practices

All cases selected for the Book of Good Practices have been anonymised for the sake of quality control of the assessment. The Book of Good Practices showcases patient engagement projects that are exemplary in one or more PE Quality Criteria or overall show high and meaningful ways to engage and involve patients and other stakeholders in the medicines research and development continuum. The language and content reflects the views of project owners, only minimal alterations have been made to the text by PFMD to provide more clarity (when it was needed). For more information, please contact the PFMD team.

Patient Pathway Mapping
Case from a Patient Organisation ("Organisation")

PE project description

Provide data on unmet need and therapeutic burden of patient and care partner through participatory techniques, pathway mapping, life impact and emotional journey across disease states.

The assessment of the quality of PE project

The methodology of the PE practice:

1. Qualitative semi-structured interviews utilizing a conversational, narrative, ethnographic approach to allow patients and care partners to piece together and share their stories and unmet needs in a meaningful way.

2. Participatory techniques providing opportunity for co-production of patient/care partner pathway mapping. Example domains include:

- Emotional journey
- Impact of condition on main aspects of life
- Challenges and barriers faced
- Triggers and behaviors
- Therapeutic burden
- Relationship with healthcare team
- Decision points and clinical trial experience

Which phases of research, medicines development, lifecycle or disease management does this PE project cover?

✔ **Research and discovery phase** 1) unmet medical needs identification, 2) disease understanding [patient experience of the disease], 3) drug discovery, non-clinical and candidate-identification phase

☐ **Pre-clinical phase** (including non-clinical, pre-clinical research, safety and efficacy tests)

☐ **Clinical study phase 1**

☐ **Clinical study phase 2**

☐ **Clinical study phase 3**

☐ **Health technology assessment**

☐ **Regulatory review and approval or registration phase** (including submitting for market authorisation request and approval)

☐ **Post-registration / -launch activities**

☐ clinical study phase 4,

☐ drug safety monitoring and pharmacovigilance,

☐ Pricing and reimbursement

☐ real-world evidence generation,

☐ adherence,

☐ patient education,

☐ patient and carer support programmes,

☐ disease management,

☐ public health,

☐ marketing insights

☐ **Other**

Which stakeholders does this PE project involve?

✔ **Patients and carers** (including caregivers, and family members)

✔ **Patient advocates, patient organisations and associations**

✔ **Healthcare professionals** (including clinical investigators, general practitioners , specialists, pharmacists and nurses)

☐ **Policymakers**

☐ **Regulators**

☐ **Payers**

☐ **Health technology assessment organisations**

✔ **Pharmaceutical companies or industry** (including medical devices and biotech companies)

✔ **Researchers** (academic researchers and investigators)

☐ **Research funders**

✔ **Other** (for example, contract research organisations (CRO) and hospitals)

1. Shared purpose

This refers to the project's aims and outcomes that all stakeholders taking part should agree on before starting the project. Consider putting in place processes to help facilitate discussions between all stakeholders to identify each other's values, expectations and objectives, and review and discuss priorities in the planning of the project. It can be valuable to enable stakeholders to exchange views openly to understand the scope and objectives of the project, acknowledging that some of their objectives may differ. All parties concerned should also have a shared written description of the common goals of the project.

The initiative was created to ensure that patients and caregivers have an opportunity to voice unmet needs and therapeutic burden outside of specific interview style opportunity. Pathway mapping was conducted utilizing 1:1 (patient), 1:2 (patient:caregiver) and 1:many (group of patients) approaches.

Group and 1:1 feedback was obtained from patients and caregivers to ensure they felt their specific needs were being addressed.

2. Respect and accessibility

This refers to (1) respecting each other, and respectful interactions within the project to be established among partners, and (2) openness to and inclusion of individuals and communities (to the project) without discrimination. Considerations to ensure good conditions to implement the project should be made from the beginning. For example:

- simplification of wording

- budget and payment considerations

- cultural adaptations to procedures

- practicalities such as meeting timing, location and format

- accessibility of project materials

- written co-developed rules of conduct

Accessibility to participate may be facilitated by enabling multiple ways to involve stakeholders who could benefit from and/ or contribute to the project. For example, patients with cognitive impairment might need more time to go through project material, or need printed versions rather than electronic documents or PDFs for easier reading.

All patients and caregivers received coaching during this [project] to ensure they were comfortable interacting with various stakeholders. In addition, they created a process to provide patients, who were not comfortable participating in a group setting and those with limited mobility, to also participate. Patients were compensated for their time.

Benchmarking of fair market reimbursement was conducted. Frequent 1:1 touchpoints.

3. Representativeness of stakeholders

> This refers to the mix of people you involve, which should reflect the needs of the project, and the interests of those who may benefit from project outputs (for example, target population). Consider diversity in expertise, experience, demographics, and other relevant criteria for inclusion. When selecting PE stakeholders, patients, attention will be given to awareness of the diversity required to achieve visible representative voice.

Different age groups, stages of disease, ethnicities and geographies were incorporated into the selection of Organisation's patient/caregiver partners using Organisation's database, which consists of ~17,000 patients and caregivers representing various patient and caregiver segments across disease states.

This PE project owners used documentation of demographics (to ensure fair balanced) and virtual opportunity to check if what they did was appropriate to achieve this criterion.

4. Roles and responsibilities

This refers to the need for clearly agreed, and ideally co-created roles and responsibilities, in writing, addressing that all aspects of project needs will be established upfront and revisited regularly.

Patients and caregivers were clear on their role in patient pathway mapping and how the data would be used to determine trends in unmet need. Patients and caregivers were also provided direction on how their participation [in this project] would be used with feedback loops built in throughout.

Commitment documents were developed with patients and caregivers. A co-designed scorecard was used during the assessment.

5. Capacity and capability for engagement

This refers to (1) capacity as having relevant and dedicated resources from all stakeholders (for example, providing a dedicated point of contact by the sponsor and having allocated sufficient time by all stakeholders to allow genuine engagement); and (2) capabilities for all stakeholders to enable meaningful engagement. (For example, the level of knowledge, expertise and training stakeholders might need to deliver PE activities throughout the project).

Consider supporting stakeholders to build the required capacity and capabilities for this project in different forms of training both with sponsor organisations and with each stakeholder (for example, helping to understand the context, processes, involved terminology etc.).

Both capacity and capability building are intended to facilitate participation and lower barriers to collaborate. Stakeholders can be given access to learning resources and given dedicated support (if needed). Capability needs may vary depending on the project needs, but also e.g. personal circumstances of PE representatives.

All patients and caregivers received coaching during this [project] to ensure they were comfortable interacting with various stakeholders. Not all patients/caregivers that were interviewed were used in [this project], thus comfort level and boundaries were determined in advance.

Each patient/caregiver completed an assessment on their engagement preferences.

6. Transparency in communication and documentation

This refers to the establishment of communications plan and ongoing project documentation that can be shared with stakeholders. Communication among stakeholders must be open, honest and complete. In addition, adequate up-to-date documentation must facilitate communication with all stakeholders throughout the project. Consider proactively and openly sharing progress updates throughout the project externally. In addition, communicating outcomes of the project to all stakeholders and how their contribution was of value to the success of the project is critical.

Some interviews were recorded and notes taken throughout. Feedback was captured in [the project's] scorecards.

This PE project owners used co-designed scorecard to check if what they did was appropriate to achieve this criterion.

7. Continuity and sustainability

This refers to the smooth progression of the project, as well as efforts to maintain ongoing relationship with stakeholders. Consideration should be given for the role of stakeholders beyond a single project. When starting the project, consider including in your project plan the actions needed for maintaining expected flow of the project from beginning to end. Create a plan to nurture relationships with your partners and stakeholders involved during the project, and when needed and requested, beyond the project as well. For all stakeholders successful planning and personal and organisational resilience should be anticipated.

All patients and caregivers that participated were kept abreast of development and continuous improvement of the initiative through electronic updates, even as Organisation rotated other patients and caregivers through the project.

This PE project owners used various communication channels to check if what they did was appropriate to achieve this criterion.

Results, outcomes and collecting evidence

PE practice led to the following (measured) outcomes:

- 1,800 patients/1,200 caregivers
- Short term and long term unmet needs were identified
- Trends in burden of therapy were identified
- Co-design of potential research questions
- Patient/caregiver unmet needs-traditionally from KOL perspective
- 5 innovations evaluated during [this project] utilizing 300 patients and caregivers

Improvement in understanding unmet needs and therapeutic burden.

Evolution from KOL perspective of patient/caregiver burden.

Positive impact for specific medicines development phases

- Co-design of research question
- Opportunity for non-traditional voices to be heard
- Quantified patient stories through various tools

Direct or indirect positive impact for patients

- Opportunity for patients and caregiver perspectives to be heard and dimensions of life affected to be measured
- Opportunity to understand how others are also affected through simplified

Direct or indirect positive impact for stakeholders involved in the project (other than patients)

- Informed understanding from patient/caregiver perspective
- Validation against EBM