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.

New internal tools and processes
Case from a Pharma Company ("Company")

PE project description

Company modified and developed new tools to include direct patient perspectives and drive patient centered decisions:

- **Patient Insights Playbook and Integrated Patient Journey Template**

  Patients from US, UK, Australia, Taiwan and Korea provided feedback on the PE Playbook. This occurred via in depth interviews. Some modifications have been made and further modifications will be made, especially to terminology, and also to ensure that interactions are mutually positive.

- **Updated Target Product Profile (TPP): includes direct patient insights**

  To validate insights from patients, various qualitative and quantitative approaches have been utilized, depending on the project. For example, during a patient advisory board meeting, patients consistently expressed and described the impact of fatigue on their daily lives. Fatigue is well documented in the literature, however hearing directly from patients influenced the R&D team to take action and study fatigue.

Although the Target Product Profile (TPP) is proprietary, it includes primary and secondary endpoints, patient reported outcomes, patient benefits, safety, tolerability, and dosing/presentation. Insights should be provided directly by patients based on descriptions of disease and treatment burden and unmet needs (Please see Appendix 1). For representativeness, it is important to specify at the beginning what is the target population.

- Customer Value patient insight into formulation/supply chain
- Patient Voice Plans in R&D: patient insight into compound and clinical strategies and protocol design
- Internal Compliance Guidebook: best practices on how to implement patient engagement

Practical examples: modifying a device design based on patient insights, resulting in more correct dosing of medication, re-ordering tests in a clinical trial, reducing length of visits in a clinical trial, decision to measure fatigue and sleep patterns based on patient insights, working with patients to develop a new symptom diary, and involving patient groups in development of web site content. These examples are from multiple disease areas within the Company.

The assessment of the quality of PE practice

Driving change via Patient Engagement Dashboard
- **Objective:** drive behaviour to incorporate direct patient voice early and understanding barriers.

Activation: Company is measuring the amount of organizational change.
Enablers: Company is seeking to understand roadblocks to organizational change.
Sustainability: Company is measuring the impact of these Patient Engagement activities.

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

"These tools and processes span the drug development life cycle"

✔ **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.

Company's Patient Engagement Mission Statement: "We will systematically interact with patients, obtaining direct and inferred insights, so we can effectively act on their perspectives early".

In one example, the Patient Reported Outcomes team worked with patients to understand their perspectives, ultimately resulting in the development of a new tool to measure what matters to patients, as reported directly by patients. Direct patient interviews were used to develop the questionnaire, and patients participated in cognitive interviews to ensure that the questionnaire's instructions, questions, recall periods and response criteria were clear and easily understood. Validity and reliability were assessed in an observational study.

Patient Reported Outcomes team worked with patient groups to create and validate the mission statement.

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.

Existing tools and processes have been updated to include Patients as an additional stakeholder with equal respect in Company's decision making (Target Product Profile, Customer Value/Formulation Decisions and Integrated Patient Journey). Patient Reported Outcomes team also created a Patient Voice Plans for R&D at a compound level, to strategically assess and determine what were the best ways to engage with patients in Company's clinical plans. Patient Reported Outcomes team knows that there is not a one size fits all methodology for patient engagement so they recognize that the unique needs of all stakeholders should be taken into account upfront.

In working with patients on the PE Playbook, Patient Reported Outcomes team worked with facilitators who are based in the local countries and understand the local cultures, and a discussion guide was used.

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.

The Patient Voice Plans, at a compound level, will strategically assess and determine which sample groups should be involved in the project. The Internal Compliance Guidebook and the Patient Insights Playbook also provide guidance and recommendations on selecting representative groups.

One example in France: a patient group provided input into a trial protocol, changes were made, and the group was notified about which changes were implemented and some which were not. Later, Company decided not to further develop the medicine and the same patient group was notified.

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.

In collaboration with patient groups, a video was produced to help patients manage a new diagnosis with IBD. This was a truly collaborative effort, with roles and responsibilities outlined at the beginning, which were modified as the project was underway based on real time learnings. In the UK, the operating company has partnered with a patient organization for a three year period to be measured by the patient group against a set of criteria evaluating the level of patient centricity. A baseline has been identified, and plans have been agreed upon.

Patients helped develop the Patient Engagement Mission, Patient Engagement Playbook, and have provided insights into Target Product Profiles as well as other tools. Patients provided feedback and insights through various interactions including advisory boards, interviews with patients and patient groups, focus groups, surveys, market research and other means. Patients also helped one R&D team develop a new survey to measure symptoms for a disease. The process began with members of an online patient community providing feedback on a draft which was then further modified, and further tested.

Elements of study designs modified include reordering tasks/tests and reducing length of visits. A new Director, Patient Insights has joined the team and is currently conducting studies in over 13 countries with more than 1,300 patients across all 11 disease areas. The purpose is to gather patient feedback on protocols in order to make modifications before protocols are finalized.

4. Roles and responsibilities

A recent example of modifying study design was reordering tasks based on patient feedback. The team originally planned to start with more difficult tasks and end with simpler tasks as a "reward". However, based on direct patient insights, a decision was made to start with simpler tasks and build up to more difficult, so that patients could build confidence. This change will apply to multiple studies for the same disease area.

In both cases (IBD project and patient centric culture partnership), contracts were mutually agreed upon and entered into up front – before the start of the projects - and modified as appropriate along the way. One lesson learned is collaborating on a project slowed down timelines, but it was the right thing to do.

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.

The Patient Voice Plans, at a compound level, will strategically assess and determine what support is needed for meaningful engagement in the project(s). The Patient Insights Playbook also provide guidance and recommendations meaningful collaborations.

Training and tool kits have been developed for internal personnel, and external moderators are selected based on experience working with patients. For a patient advisory board recently put in place for a 1.5 – 2 year period, patients went through an application process. The application was written in a manner appropriate for lay persons, and mutual expectations are being set for engagement throughout the project.

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.

Within the Company, tools and processes have been standardized to ensure a harmonized approach to patient engagement. Anonymized project specific insights are stored and shared so that they can be leveraged in the future. The dashboard can be found in Appendix 1.

A new internal system was developed and put in place to store market research and other appropriate documents so that patient insights can be referred to and acted on regularly. Patients and patient groups have provided consent and privacy rules and agreements have been followed. Before a project begins, materials are produced which are clear and written in lay language.

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.

Company has policies on patient engagement, inclusive of transparency (Global, Regional and National regulations).

Company's policies are set according to global/local laws and regulations and are independently monitored and audited for business adherence to the set policies.

Results, outcomes and collecting evidence

Outcomes and results include, but are not limited to:

1. **Product formulation/design** - modification of a drug/device combination product in development based on patient insights, resulting in more correct dosing

2. **Modification of multiple clinical trials** based on direct patient insights, including reversing the order of tests/tasks (protocol modification), providing transportation to the site, and reducing length of visits. In one clinical trial, there were no dropouts for 7 months.

3. **Developing medicines that better meet needs:** decision to modify a target product profile to pursue a biomarker or faster acting medicine, based on feedback from a patient advisory board.

4. **Measuring what matters to patients:** decision to measure fatigue and sleep patterns, working with patients to develop a new symptom diary.

5. **Co-developing disease educational materials.** These examples are from multiple disease areas within the Company.

Results, outcomes and collecting evidence

- Better recruitment and retention in clinical trials
- Better correct use leads to better outcomes
- A more aligned product to patient's needs and wants

Direct or indirect positive impact for patients

- A better experience in Clinical Trials
- Better correct use of a product
- Products that better align to their needs and wants

Lessons Learned

Learning is that patient engagement requires time and commitment. It is important to have the support of senior leaders, to have tools and expectations and to embed patient engagement into the various departments, such as R&D, supply chain, commercial etc.

It is also helpful to hold employees accountable by measuring progress over time. The Dashboard has been in place for 1.5 years. Significant progress has been made and there is still more to do. To operationalize patient engagement, it is important to get support from all functions of Company to embed patient engagement as a way of working.

It was found helpful to start with "service models" whereby a dedicated resource or department in Company initially conducts the engagements – this can accelerate progress. It was also learned that budget constraints and time can be limiting factors for patient engagement, but as long as everyone is asking what does the patient want and need and as long as patients are involved directly in development of solutions, ultimately needs should be better met and research should be accelerated.

Appendix 1

Dashboard Objectives:

- **Drive Organisation's behavior** to incorporate direct patient voice early in drug development
- **Understand barriers** to incorporating patient voice

  - High Value Compound
  - Timing: Early, approx. ph 2
  - Patient Role: in selecting, paying adhering

Patient Engagement Dashboard

Disease Area Strategy:
- Is an Integrated Patient Journey (IPJ) complete?
- Is there direct patient input into disease area strategy?

Compound Strategy
- Are Patient and Self Pay sections of the Target Product Profile (TPP) complete, with insights derived directly from patients?
- Did patients provide direct input into compound indication/s and end points?
- Have patients provided input into product formulation / delivery per Customer Value Framework?
- Does the compound have a pre-approval patient access strategy?

Clinical Development Plan:
- Did patients provide direct input into the clinical development plan prior to the start of pivotal?
- Are protocol designs and operational strategies informed by direct
- patient insights?
- Are the Patient Reported Outcomes needed for evidence at launch incorporated into the pivotal?

Integrated Patient Journey - Template

Key terms	Phase 5	Phase 6
Rational Title	Remission or Relapse	
Emotional Title	Waiting for the ball to drop	
Age & Time in Phase	Months	
Essence of the Phase	Attempting to return to life without cancer. Challenging as faelents are unsure of what to do with many feeling stuck in a holding pattern	
Pt/Caregiver's Emotional & Psycho-Social Experience	Patient: Optimistic that they will remain cancer free, though an underlying feeling of anxiety as patients worry about it coming back. When it does return. patients feel devastated Caregiver: supporting the patient has needed. attempting to return to their normal routine	
Pt/Caregiver's Physical & Medical Experience	Patient feels better than they have in the past with CT scans every 3 - 6 months reminding them of their cancer	
Moments of Meaning (includes unmet needs)	Physically, patients feel better in remission, emotionally it can be a tough time with many unsure how to move forward	
Stakeholder Experience	Patient, caregiver, oncologist, radiologist	
Business Leverage Point	Provide / direct patients and caregivers to support groups to help them emotionally	
Facts and Data	Even in the NCCN guidebook, remission or relapse is not discussed Treatment is discussed as cure or controlling	
Knowledge Gaps	How can patients / GGs best deal with remission? Are there things that they can do to help them find their 'new normal'?	

Appendix 1

Introduction | Purpose of Patient Engagement (PE) Playbook

Chapter 01 | Foundational Understanding of Patient Engagement

Chapter 02 | Engagement Mapping

Chapter 03 | Best Practices for Strategic Excellence

Chapter 04 | Tools, Templates, and Cross Functional Application

Chapter 05 I In Patients' Own Words

Chapter 06 I Glossary

Patient Engagement defined:

Patient engagement is integrating the voice of the patient throughout the lifecycle of the disease area and item (i.e. drug, device, diagnostics, solutions) development and commercialization.

It is defined as a purposeful dialogue with patients for improving lives and outcomes. It involves:

- Systematically Listening, Understanding, Co-Activating and Collaborating with patients directly or indirectly in one-way or two-way participation.

- Activating patient insights by informing Company's product, service and solution development early on, and by creating meaningful value proposition through innovative ideas that are empowering for the patients and their care community.