



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 FOCUSED
MEDICINES DEVELOPMENT



Online tool to inform, educate and explore patients' needs

Case from a Pharma Company (“Company”)

PE project description

The Online Tool, created for patients as well as their relatives to inform, educate and explore people's needs. The Online Tool, first in navigating patients and caregivers through the complex HC system for diseases, initially metastatic breast cancer, stroke and kidney cancer, is patient-led and developed in concept, content and design, leveraging synergies across therapeutic areas in co-creation with external partners.

In a pilot project, a digital service platform that offers quality-reviewed information and patient pathways for people living with illness and their families was developed. With a clear structure with questions, answers, and links, those affected should quickly find the information they need. What's unique about this: Through the development of personal “patient pathways” patients also get individual help in dealing with their disease. The patient pathway helps them outline and understand their options and what they can do to stabilize or improve their health. Thus the platform may help the people affected, as well as their families and other members of their support communities, to cope better with their new situation and their own emotions, to deal with the plethora of information, and to make necessary decisions with a sense of confidence and empowerment.

The central impulse for the creation of this internet portal was the idea of a board of patient experts to develop a new information offering for patients with a severe chronic disease, which would offer them support and comfort on their path and would help them deal with their new situation, their emotions, and the plethora of information, as well as empower them to make important decisions and to cope better with the disease in their lives.

The Online Tool is based on patient, physician and the Pharma Company's (hereafter, referred to as Company) expertise:

The experts are represented for metastatic breast cancer (14 experts), stroke (8 experts) and kidney cancer (6 experts) according to their professional background. In both categories, Company's colleagues are involved and participate at eye level with the external specialists that support the project.

Users find information through more than 250 questions and answers that include links to medical associations, treatment guidelines or journals to make external knowledge available for the users. The Online Tool provides links and short descriptions of more than 50 patient organizations and other platforms that offer information and help.

Information is collected by the people using it to help them navigate their care including and not limited to appointments, family and patient resources, medicines, professional and social issues.

All content goes through a multi-level review process before publication. Checks are performed on the factual correctness of the information, the relevance and currency of the content, and whether the selection and presentation of the topics is suitable for the patient audience. Experts who provide different specialist competencies, such as in the medical, legal, and editorial fields, are involved in the different review processes. Patients also ensure that the patient orientation of the topics and content is guaranteed. Medical specialists check the accuracy of the health topics, while communication experts ensure that the text is easy to read and flows well.

The protection of personal privacy and private data is important to Company. Company captures, processes, and uses personal data in compliance with the German data protection laws and its data protection declaration.



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

“Medicinal product agnostic, generally intended for newly diagnosed people with illness with potential utility at other times in the journey with that illness”.

- 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** - Care journey (disease/ care management)

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 concept for the Online Tool originated from patients attending a regularly occurring Company-Patient-Dialogue in Germany with patient advocacy groups. Discussions were continued among a smaller group of patient representatives, IT experts, health care professionals, and Company colleagues to co-create the Online Tool.

The working group confirmed the appropriateness and progress at regularly occurring update discussions.



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.

The program was shaped from the start with guidance from patients and in an iterative manner through its development. Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.



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.

Patient advocates and healthcare professionals involved in the project interact on a regular basis with a diverse group of patients within Germany and strived to integrate those diverse experiences into the project. However, authors recognize that individual patient experts cannot be fully representative of all patients who may use the Tool.



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.

The point of contact for the patients involved was a skilled and dedicated Patient Relations Manager (well-versed in Company's, EU and Country regulations for interactions with patients) who has developed and nurtured relationships with patients and patient organizations involved.

Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.



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 patients involved had the requisite expertise as established advocates and included people with health communications expertise as well as experience as patients.

The selection of the team included patients from the start as well as Company colleagues to ensure the alignment of capabilities with the goals of 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.

A transparent communication plan was part of the design and release of the Tool to the public. In addition the quality process is outlined below:

- *Concept: Co-Development of Concept, Topics and Content together with Patient Organisation*
- *Editorial: Defined quality criteria*
- *Review I: Check by patients and caregivers*
- *Review II: Check by medical and legal external experts*

At a glance: How was the content for this website created?

| Conception | Editorial Team | Review I: Patients | Review II - Scientific experts |
|--|---|---|--|
| <i>Selection and concept of topics and content in collaboration with experts in patient work</i> | <i>Creation and editorial review of content based on defined quality criteria</i> | <i>Review of the content by external patients and relatives</i> | <i>Review of the content by medical experts on the scientific advisory board</i> |

Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.



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.

The plan included launch in two illness areas with the intent if successful for expansion beyond those areas and in additional geographies beyond Germany. The team including patient experts has been involved in those discussions. A third illness area was launched and more are planned.

Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.



Positive impact for specific medicines development phases

Company’s “patients first” approach across the medicines development phases includes creating new alliances and partnerships among stakeholders. Responsiveness to suggestions meeting a need identified through the Patient-Company-Dialogue is but one way Company is demonstrating this commitment.

Authors are working with other country and global teams to implement this in other countries and adapt it in ways patients feel it will be useful for them.

Direct or indirect positive impact for patients

The Online Tool may help people identify their needs and assist them with navigation through the healthcare system as well as a better understanding of patient health condition and expectations. Metrics below about initial reach and average session duration are encouraging.

Total Number of users (launch May 8th until 30th April 2018)

| <i>Users total</i> | <i>Session Duration</i> | <i>New Visitors</i> | <i>Returning Visitors</i> |
|--------------------|-------------------------|---------------------|---------------------------|
| <i>221,000</i> | <i>00:00:59</i> | <i>219,600</i> | <i>17,400</i> |

Updated total number of users 221,000 as of May 2018

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

The impact for non-patient stakeholders is the experience of co-creating a tool with patients as experts on the same team. This has not been the traditional approach in development of healthcare tools for patients.



Lessons Learned

1. A “Patient First” approach is key: the patient need was at the core of the project and a group of patient experts were involved in developing the program.
2. Strong sponsorship and involvement of sponsors along the journey: sponsorship at senior organizational levels, with involvement throughout the project and at key strategic points.
3. Plan carefully with the flexibility to be responsive to potential challenges: create a project plan, look at all the tasks and dependencies early and get input from all external and internal experts along the way.