



# 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



## Understanding patient and stakeholder needs alongside the patient journey\*

Case from a Pharma Company (“Company”)

### PE project description

The Initiative had the goal to understand the needs along the patient journey and to co-develop a solution that helps patients managing their daily life covering the most important needs. As early as possible, stakeholders’ needs alongside the patient journey were collected and matched, so that the most important needs could be identified.

As a second step, based on the prioritized needs, a solution gets co-created and tested. The solution will get implemented after launch. The project itself got stopped due to prioritization of another digital health solution, which was more advanced. However the insights could be used for the other patient solution.

\*Update in Jan 2018: This project has been discontinued due to internal matters



## The assessment of the quality of PE practice

Steps towards the Initiative's solutions in a non-oncology disease setting:

1. Multi-stakeholder advisory board with patients, patient experts, nurses and physicians has been conducted by asking their challenges, needs and feelings alongside the patient journey (Please see details below).
2. Results from advisory board have been confirmed with market research and had been matched to internal strategy.
3. External landscape analysis has been conducted to check what solutions are available to patients with the disease.
4. Potential solutions with patients, patient group representatives, nurses and physicians, that were present in the first advisory board, have been co-created. Three ideas were defined to move forward.
5. The solutions have been tested with other patients and physicians and revised.
6. The solution is going to be implemented within test countries and in collaboration with local patient groups.

Session	Groups	Exercise	Outcome
<b>Working Session I</b> (75 min. plus 15 min. walkthrough during coffee break)	2 mixed groups (collaging is an individual task therefore group composition does not matter)	<b>Collage:</b> select picture and create a collage that reflects how you experience life with the disease (For HCP: create a collage that reflects how you feel about treating patients)	Emotional understanding of: <ul style="list-style-type: none"><li>• The patient's feelings, problems, needs, state of mind</li><li>• The HCP's feelings and emotions when treating patients</li></ul>
<b>Working Session II</b> (60 min. plus 10 min. per group for sharing of key points in plenary)	Separate groups (Patients/PAGs and HCPs)	<b>Needs along the patient journey</b>	<ul style="list-style-type: none"><li>• Talking through patient journey phases (awareness, diagnosis, treatment and adherence)</li><li>• Mapping of needs along the entire patient journey</li></ul>
<b>Working session III</b> (60 min.)	<b>Patient/ PAG group</b>  <b>HCP group</b>	<b>Focus - Life with the disease:</b> impact on social life and living with the disease and symptoms <b>Focus - Interacting with patients:</b> typical questions, concerns, dealing with patients' emotions	In-depth understanding of daily challenges, struggles and needs in focus areas that most relevant and actionable for Pharma Company (basis for solution development)
<b>Working session IV</b> (30 min. plus 10 min. per group for results sharing in plenary for III and IV)	Same groups continue from working session III	<b>Solution brainstorming</b> for focus areas: <b>information needs and other solution ideas</b>	<ul style="list-style-type: none"><li>• Longlist of ideas that addresses identified needs in the focus areas</li><li>• Voting and definition of 3-5 ideas</li></ul>





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

- |   |  |   |
|---|--|---|
| <ul style="list-style-type: none"> <li><input type="checkbox"/> <b>Research and discovery phase</b><br/>1) unmet medical needs identification, 2) disease understanding [patient experience of the disease], 3) drug discovery, non-clinical and candidate-identification phase</li> <li><input type="checkbox"/> <b>Pre-clinical phase</b> (including non-clinical, pre-clinical research, safety and efficacy tests)</li> </ul> | <ul style="list-style-type: none"> <li><input checked="" type="checkbox"/> <b>Clinical study phase 1</b></li> <li><input checked="" type="checkbox"/> <b>Clinical study phase 2</b></li> <li><input checked="" type="checkbox"/> <b>Clinical study phase 3</b></li> <li><input type="checkbox"/> <b>Health technology assessment</b></li> <li><input type="checkbox"/> <b>Regulatory review and approval or registration phase</b> (including submitting for market authorisation request and approval)</li> </ul> | <ul style="list-style-type: none"> <li><input checked="" type="checkbox"/> <b>Post-registration / -launch activities</b></li> <li><input checked="" type="checkbox"/> clinical study phase 4,</li> <li><input type="checkbox"/> drug safety monitoring and pharmacovigilance,</li> <li><input type="checkbox"/> Pricing and reimbursement</li> <li><input type="checkbox"/> real-world evidence generation,</li> <li><input type="checkbox"/> adherence,</li> <li><input type="checkbox"/> patient education,</li> <li><input type="checkbox"/> patient and carer support programmes,</li> <li><input type="checkbox"/> disease management,</li> <li><input type="checkbox"/> public health,</li> <li><input type="checkbox"/> marketing insights</li> <li><input type="checkbox"/> <b>Other</b></li> </ul> |
|---|--|---|

### Which stakeholders does this PE project involve?

- |   |  |   |
|---|--|---|
| <ul style="list-style-type: none"> <li><input checked="" type="checkbox"/> <b>Patients<sup>1</sup> and carers</b><br/>(including caregivers, and family members)</li> <li><input checked="" type="checkbox"/> <b>Patient advocates, patient organisations and associations</b></li> <li><input checked="" type="checkbox"/> <b>Healthcare professionals<sup>2</sup></b><br/>(including clinical investigators, general practitioners, specialists, pharmacists and nurses)</li> </ul> | <ul style="list-style-type: none"> <li><input type="checkbox"/> <b>Policymakers</b></li> <li><input type="checkbox"/> <b>Regulators</b></li> <li><input type="checkbox"/> <b>Payers</b></li> <li><input type="checkbox"/> <b>Health technology assessment organisations</b></li> </ul> | <ul style="list-style-type: none"> <li><input checked="" type="checkbox"/> <b>Pharmaceutical companies or industry</b><br/>(including medical devices and biotech companies)</li> <li><input type="checkbox"/> <b>Researchers</b><br/>(academic researchers and investigators)</li> <li><input type="checkbox"/> <b>Research funders</b></li> <li><input type="checkbox"/> <b>Other</b><br/>(for example, contract research organisations (CRO) and hospitals)</li> </ul> |
|---|--|---|

1. Patients a) just diagnosed and b) living with the disease for longer time from the 3 pilot countries

2. HCP who were considered as local and/or global TAEs



### 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 involved patients and patient representatives as well as other stakeholders from the start until the end of the project, by understanding the needs, co-creating the solution and by implementing it together.*

*Examples of the questions and topics planned for the advisory board to get discussions started.*

#### **1. Select pictures and create a collage that reflects how you experience life with MS or how you experience to treat MS patients:**

- *Why did you use this picture?*
- *Why is ... important to you?*
- *How do you deal with ...?*
- *What is the impact of ... on your daily life?*

#### **2. Patient Journey:**

- *What was it like for you to go through these steps?*
- *What were particular challenges you faced?*
- *Why were certain things difficult and what are the implications of that?*
- *What are things that went well?*
- *What support did you receive?*



## SECTION 2: The quality of patient engagement



### 1. Shared purpose

#### 3. Concerns/ Challenges:

- a. What are your (an MS patient's) biggest concerns when getting up in the morning?*
  - b. Where do you feel the biggest challenges regarding your social life being an MS patient?*
  - c. Where do you struggle most in daily life with the MS disease and the related symptoms?*
- *Contracts and track records are in place to meet Company's compliance and legal requirements.*
  - *After each of the meetings Company have assessed the quality of interaction with participants.*
  - *The final solution still needs to be implemented and needs to include metrics to measure the success. **[Edit Jan 2018: initiative has been disconnected]***



### 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.

- *In the workshops a way of verbal and nonverbal communication was used with (for example) pictures, as feelings may be better expressed with images.*
- *Very strong listening skills as well as moderators who are having the empathy to accommodate all stakeholders have been used.*
- *There was a follow up in 1:1 meetings to clarify all individual perspectives and to understand if anything in the group was missing .*
- *HCPs and patients were first split in the workshop. However, the participants wanted to listen to the other perspective, so they handle a joined meeting the following time.*
- *Participants' surveys helped to improve the project and the way of working with the stakeholders.*



### 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 most difficult task was the identification of the right participants\*. They were identified via their affiliates and patient group relations people. Qualitative market research was used to complete their findings.*

*\*1. Patients **a)** just diagnosed and **b)** living with the disease for longer time from the 3 pilot countries*

*2. HCPs who were considered as local and/or global TAEs*

*Company could match the outcomes of the advisory board and the market research very well.*





## SECTION 2: The quality of patient engagement



### 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.

*Follow up meetings 1:1 as well as updates via emails informed about the progress. Once the final implementation is done, the feedback loop will be ensured.*

*This PE project owners used feedback from participants to check if what they did was appropriate to achieve this criterion.*

*The feedback of the workshop participants was always very good and didn't have a lot of feedback for improvement, other than mixing the stakeholder groups more.*



## SECTION 2: The quality of patient 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.

*In this project, the focus was on questions and solutions for daily life. Therefore, no further skills or knowledge from the participants were necessary. The stakeholders were informed alongside the process of the questions and tasks.*

*Stakeholders did not look for clinical trial experience, knowledge of drug development. They selected participants on the basis of the following criteria:*

- 1. How long they were living with the disease*
- 2. Geographical scope*
- 3. Female and male ratio*



## SECTION 2: The quality of patient engagement



### 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.

*Contracts and track records are in place to meet Company's compliance and legal requirements. As per Company's guidance, it is mandatory to document any activity with external engagement, e.g. advisory boards. There is a need to work on the share best practice in house and get local approvals for the engagement.*

*Documents were shared with the workshop participants before, during and after the project. Before the meeting only the objective was shared. After the meetings the outcome and the next steps including the final testing were shared.*



## SECTION 2: The quality of patient 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 scope of the project as well as the role of each individual involved was always very clearly communicated via the contracts, as well as in the meetings. Whenever questions occurred, they were appropriately addressed.*





### Results, outcomes and collecting evidence

#### **PE practice led to the following measured outcomes:**

- Profound understanding of patient and stakeholders needs alongside the patient journey.
- Potential solutions that may impact the life of the patients.
- This Initiative showed Company that there is a very high unmet need in giving the power to patient to live their lives.
- With the co-created solution, which is still in iterations, Company believes that it can help patients to achieve their goal to be proactive in living a normal life.
- The process Company has chosen, is a very solid approach, however, final implementation seems to be more challenging than anticipated due to cost and compliance considerations.

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### Positive impact for specific medicines development phases

Improvement of self-empowerment and therefore also clinical care.

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### Direct or indirect positive impact for patients

Living a normal life.

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

Helping HCPs.



### Lessons Learned

The project was stopped, however the way of working to assess the needs along the patient journey and how to co-create solutions already has been used by other disease areas.

It was very difficult to find the right participants with the right profile and without compliance/ legal restrictions. However, once Company had identified the stakeholders, the biggest learning was, that a multi-stakeholder approach gives the best overview to the questions Company had providing the variety of different perspectives. This made it easier for Company to prioritize the key findings.

Involvement of the stakeholders alongside the project including the multi-stakeholder perspectives.

It's important to give enough time to plan and identify the participants.