The PFMD
Book of Good Practices

1st edition I 2018

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#PEQualityGuidance
#PEQualityCriteria

www.PatientFocusedMedicine.org

May 2018

Message from PFMD

Dear reader,

Patient Focused Medicines Development (PFMD) was established in 2015 out of a need that was expressed by many stakeholders in various roundtable discussions. This need was eventually translated into a dedicated organisation that aims to drive systematic patient engagement and involvement forward in the research, development and delivery of medicines.

Today we are very proud that you are reading the Book of Good Practices, as this too was born from a need expressed by many stakeholders. As patient engagement is becoming a norm instead of an exception or a one-off practice, there was a need to share knowledge about

- how other organisations have involved patients in their activities,
- what can be considered as high quality patient engagement, and
- how can different organisations reach the level of patient engagement that is both meaningful to patients but also to the research and development processes so that the output or outcomes will serve the end users better.

The PFMD Patient Engagement Quality Guidance, that was launched in 2018, introduces 7 Quality Criteria for good patient engagement that can be used to plan patient engagement activities, or to assess the level of patient engagement in ongoing or completed activities. Where the Patient Engagement Quality Guidance serves as a tool to help you to do patient engagement, the Book of Good Practices serves as a set of real life cases from a variety of organisations, that illustrate in detail how they have done it. These cases have been chosen from a big pool, assessed by an external group of reviewers and chosen to be included because they exemplify exceptionally well the 7 Quality Criteria. For detailed descriptions of the criteria and explanations for icons used, check the annexes at the end of the book.

The Book of Good Practices will be growing year by year with new cases. To contribute to this work, you can also submit your patient engagement experiences to the PFMD team.

We hope this book will inspire and help you in your patient engagement journey. We encourage you to explore all the tools at your disposal within PFMD and Synapse - the mapping and networking tool, and connect with us for more guidance if needed.

We'd like to extend our thanks to all the reviewers, all case owners and all readers for making the Book of Good Practices possible.

PFMD Team

PATIENT FOCUSED
MEDICINES DEVELOPMENT

gsk | ViiV Healthcare

Patient and community feedback on plain language summaries (PLS)

Organisation: Viiv Healthcare/ GSK

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Table of content

1. BASIC INFORMATION 05

2. THE QUALITY OF PATIENT ENGAGEMENT

- Shared purpose 07
- Respect and accessibility 08
- Representativeness of stakeholders 08
- Roles and responsibilities 09
- Capacity and capability for engagement 09
- Transparency in communication and documentation 09
- Continuity and sustainability 10

3. RESULTS AND OUTCOMES 11

4. LESSONS LEARNED 12

4. REFERENCES 12

Annex 1. How to read the Book of Good Practices 13

Annex 2. Descriptions of the Patient Engagement Quality Criteria 14

THE BOOK OF GOOD PRACTICE INITIATIVES ARE ALSO AVAILABLE IN SYNAPSE.

TO DOWNLOAD THE FULL BOGP, PLEASE VISIT:
https://involvement-mapping.patientfocusedmedicine.org/book-of-good-practices

PATIENT FOCUSED MEDICINES DEVELOPMENT

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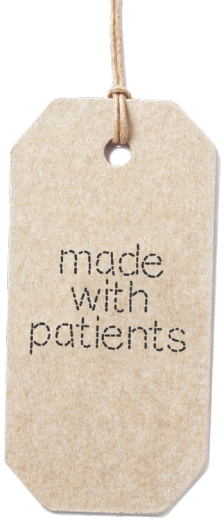

Patient and community feedback on plain language summaries (PLS)

Organisation: Viiv Healthcare/ GSK

Basic Information

Our company has been integrally involved in the cross-sectoral (industry/academia/regulators/patients/HCPs) initiatives coordinated by the TransCelerate and Harvard MRCT (Multi Regional Clinical Trials) Center, aimed to improve communication of study results to participants and the wider community through the development and dissemination of plain language summaries (PLSs). We also served on the Health Research Authority Task Force, a multi-stakeholder working group responsible for developing the EU Regulatory Guidance on Layperson Summaries (release August, 2017). These initiatives will address the requirements of the upcoming EU regulation (2018) to publish a PLS within 12 months after LSLV (last subject last visit) of a study and support efforts to provide study result to participants.

In 2014, we started their internal work to ensure good preparedness and smooth delivery of this initiative within the company, reach the PLS quality criteria in line with the requirements, and address their patients' expectation to have results communicated as early as possible after a study completion. This early work, well ahead of the applicability of the EU CT Regulatory requirement*, enabled us to understand the challenges and identify pragmatic solution to help inform external work in this area. We also wanted to ensure that the developed PLS would be understandable and clear for patients and the general public (all of whom are patients as well).

The following are two examples of feedback sprints authors conducted:

- Pilot PLS (COPD) reviewed by EUPATI trainees (2015) and
- 2 PLSs review (COPD study 113108 and study 115151 SLS) via Crowd sourcing Amazon Turk online platform (2017).

Detail on the methodology:

1. Summaries of Clinical Trial Results for Laypersons released June 2017
   https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-10/2017_01_26_summaries_of_ct_results_for_laypersons.pdf
2. Layperson Summaries of Clinical Trials: An Implementation Guide (Download here)
3. Clinical Data Transparency
   http://www.transceleratebiopharmainc.com/assets/clinical-data-transparency/

   * EU Regulation No 536/2014, see https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-1/reg_2014_536/reg_2014_536_en.pdf

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

Research and discovery phase | Pre-clinical phase | **Clinical study phase 2-3** | Health technology assessment | Regulatory review and approval or registration phase | **Post-registration/ -launch activities** clinical study phase 4 | Other

Which stakeholders does this PE project involve?

- **Patients and carers**
- Policymakers
- Health technology assessment organisations
- Research funders
- **Patient advocates, patient organisations and associations**
- Regulators
- **Pharmaceutical companies or industry**
- **Other**
- Healthcare professionals
- Payers
- Researchers

Other: Wider community: caregivers, media and treatment activists

The quality of patient engagement

1. Shared purpose

The Harvard MRCT workgroup was co-led by a patient advocate. The multi-stakeholder discussions and ultimately the guidance and toolkit released, represented a balanced and comprehensive approach based on significant input and discussions among the many stakeholders. This work then formed the basis of the HRA Task Force's guidance which was further vetted and refined specifically for alignment and clarity to enable compliance with the EU Clinical Trial Regulation as to Layperson/Plain Language Summaries (PLSs) shared with patients and community representatives to ensure good understanding of key study points and addressing all unclear stuff prior final PLS approval and publication.

The first activity in 2015 helped us refine formatting and content to improve clarity. This activity also provided further feedback as to the importance of providing clinical trial results in plain language from the patient perspective.

Pilot 2015: EUPATI interviews - Initial Patient feedback
(feedback on version 1 helped refine contact and improve clarity)

"It is essential and respectful to volunteers and patients who have taken part in a clinical trial."

"Surely it will build up good relationships between patients, the public, and researchers."

"A graph would be useful. I'm very visual."

A patient who spoke English very well as a second language misunderstood the distinction between "inflammation" and "infection".

"feeling of partnership [not] merely a patient to test on - a guinea pig"

"There are three really important sentences, but they are hard to search out [Maybe] if there were headings?"

The second activity in 2017 helped us to understand patients/wider community preferences and expectations from a communication on study results as well as common understanding of the pilot PLS:

"Really can't stress enough- this was incrediblywell compiled & easy to process. I started this under the impression that it would ultimately be mind-bendingly tedious given the subject matter, and was very pleasantly surprised to find the exact opposite. Thanks!"

"I enjoyed this study very much. I'm always interested in finding out whether the medicines big pharma companies churn out are worth the price or side effects."

"It was very interesting. Especially so since I may have the beginnings of COPD but have not yet been diagnosed."

"This is important if the manufacturer wants it to be read by this group [lay public] which apparently it does."

"I think it's a great idea because far too much information provided to patients (especially package inserts) is practically unreadable unless the patient has a medical background."

"As someone who has a relative who suffers from COPD this is a very interestingtrial to read."

"Thank you, this is important research because it is important that people understand this information and that is it not written in a way that scares people away..."

"It was understandable; plus, it was interestingto me since I have COPD."

"It's a shame it wasn't effective. Regardless, this is why drugs we so expensive by the time they get into the consumer market."

2. Respect and accessibility

At the pilot stage, we invited the EUPATI trainees (patients who completed the EUPATI course on R&D procedures and medicine development continuum) to have a preliminary feedback on the approach used. At the stage of the review via the Crowd-sourcing Amazon Turk online platform we assessed feedback on understandability, likeability and areas for improvement from patients and other stakeholders: advocates, media and treatment activists, caregivers and others (118 participants for the COPD Study 113108 and 115 for the SLS study 115151). We focused on the US geography, three age brackets, varying education levels and all had English as their primary language.

Overall, the PLS development process was highly appreciated by patients and community representatives; the response rates exceeded our expectations.

3. Representativeness of stakeholders

The feedback received on the developed PLS drafts has reflected the interests of several stakeholder categories: first and foremost – patients, caregivers, media and treatment activists, members of patient organisations and other community representatives. The only limitation was around the English-speaking audience in Europe (for the pilot stage) and in the US (for the Amazon Turk platform), as all drafts were written in English. We envision opportunities to obtain feedback on PLS in other therapy areas, clinical trial phases and on discrete issues (e.g. whether a particular graph or chart is understandable etc.)

The diversity of views/opinions was reflected in the following statements from participants regarding possible PLS improvement:

"I would like to see a little more explanation about a few points such as pulse wave velocity and the m/sec value."

"Improving it would make it longer more complicated, and then it would lose its ease of use. I'd leave this way."

"No, the document seems to provide the minimum amount of information while still serving is intended purpose which appears to me to be the goal."

"If the studywas a success, not a success or if it needed more data to be determined successful or not successful. It showed results but never uite stated that I saw if it was worth it."

"I thought the paper could be longer and more involved but then it would be complex and not as readable to the average person."

"Add a glossary to explain terms used in the summary."

"I would like to see the results for smokers vs. non-smokers"

"It would have been better if the medicines tested were useful."

4. Roles and responsibilities

All participants were instructed prior to PLSs review and accepted the terms and conditions (at the pilot stage and prior starting the Amazon questionnaire). Participants from Amazon Turk crowdsourcing were included if they were rated as high-quality responders within the Amazon Turk environment. Results for Amazon Turk were assessed across 3 discrete age ranges to get acceptable representation as well as for two different PLS (one we internally rated as "easier" to understand and the other "harder").

Internally, questionnaire was developed to assess the understanding of and how well the PLS are received. We assessed understanding by asking responders to provide 3-4 main points from the PLS in open response and then scored as to whether or not the statement was accurate. Thus, we allowed for individual differences in what stood out as important to each but could nonetheless measure if accurate or not. We found that respondents were 94-96% accurate on the 2 PLS selected for feedback (one was simpler and one more complex). We also measured whether there was anything they liked or disliked about PLS and if so, what, and whether there was anything confusing and if so, what.

Feedback received was assessed for quality of responses and completeness of questionnaire and found to be acceptable by internal team for the intended purpose.

5. Capacity and capability for engagement

Having in mind the key target audiences for PLSs as study participants (patients, including expert patients) and community representatives (caregivers, advocates and activists) we asked them to review the prepared drafts and provide feedback on wording/terminology, visibility, format, understandability and the ways data presented.

PLS development/review projects have made us understand that patient and general public input is needed to assess [the reader's] understandability. Extrapolation and repeated assessment in different populations will likely be needed to assess how well-received the PLS are for a given population. We strive for understandability at a 12-year old reading level and use health literacy and numeracy principles but in addition, stakeholder feedback is critical to ensure we are achieving a quality PLS that is fit for purpose.

6. Transparency in communication and documentation

The received feedback was documented and presented internally. As we take part in the cross-sectoral initiative as an industry representative, including at the Harvard MRCT, the findings were presented and discussed with broader stakeholder groups (including patient and patient representatives such as at the EFGCP-EFPIA Workshop, Brussels on May 2. 2017). This case study was also presented at the PFMD Task Force meeting on May 9, 2017.

Due to the tight timelines for developing and disseminating PLS, we would not recommend obtaining individual feedback before dissemination. However, obtaining feedback generally and on specific issues and

incorporating lessons learned and best practices moving forward is quite important. Crowdsourcing is useful for feedback from a general audience. Feedback from study participants is also important, keeping in mind that perspectives can differ by therapeutic area, geography, phase of study, age of participants, etc.

Written materials, documents and records are available internally and/or externally.

7. Continuity and sustainability

We have developed a standard operating procedure (SOP) for development, translation and distribution of PLS with particular consideration of a mechanism for getting feedback from patients (study participants) and the wider community on developed PLS to improve their quality.

The PLS development strategy as an essential part of our commitment to R&D transparency and disclosure of study results has been substantiated and approved for the period of 2018-2021.

Results and outcomes

Building in quality by strategic writing and review process which help establish roles and responsibilities as well as actual process for writing and reviewing by each involved stakeholder. This well-defined writing and review (process) provided clarity around who is reviewing for plain language, who for scientific accuracy and who for technical review, for example. Often there is a need to go back and forth between plain language and subject matter experts to assure that accuracy remains, however, subject matter experts are often not adept at writing in plain language.

Another learning has been the benefit of our internal work in developing a PLS template and detailed instructions so that we can hand to our selected external vendor now in 2018 and help develop their capability and understanding of what we want.

Rollout plans are to post PLS to GSK Study Register along with translation to local languages. We are scaling up this capability and will expand beyond the EU CT Regulatory requirements for PLS.

Positive impact for specific medicines development phases

Although this case study reflected the post-phase IIIb experience, PLS may be reviewed after any phase study/ clinical development milestone (of course, with the biggest consideration of phase II-IV interventional studies) and we are currently seeking feedback from study participants. This approach contributes to our values:

- Be focused on the patient;
- Respect for people;
- Act with integrity;
- Operate with transparency

Direct or indirect positive impact for patients

- Explore and/or pilot co-creation of PLS to ensure clarity and consistent understanding by several groups of patients and community representatives;
- Awareness of study results as early as possible;
- Satisfaction of study participants (if selected as PLS reviewers) making them proud of any to have contributed to the study and any knowledge gained as a result.

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

- PLS team within the company: lessons learned and areas for improvement;
- Cross-sectoral project teams (other companies, (in the development of an Implementation Guide and Recommendations for Drafting Non-Promotional Layperson Summaries – both through the R&D Organisation, regulators contributed input toward an aligned PLS guidance from FDA, EFPIA/industry associations (Reflections paper), HCPs and investigators);
- Public health benefits: increase transparency and openness within healthcare systems. The public (as patients) may feel empowered with increased knowledge and understanding of clinical trials and are better able to discuss their condition. Also, they may develop greater trust in the drug development process.

Lessons learned

After concluding the Amazon Turk second sprint, the assessments are the following:

- There is a right direction;
- This Amazon Turk system offers a fast way to see if specific portions of a PLS are understandable;
- Great method for quick check where needed;
- Could be used to test for specific issues (e.g. are more graphics/wording/glossary preferable?)
- Is there benefit in considering a head-to-head comparison with scientific summary or publication abstract?) or clarity/confusion of part of a PLS as an adjunct?
- Significant expertise has been gained, which can help with vendor selection and onboarding

Methodology, involvement of patients and the general public for feedback.

To seek feedback from study participants as a specific target audience.

References

1. https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-1/reg_2014_536/reg_2014_536_en.pdf
2. https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-10/2017_01_26_summaries_of_ct_results_for_laypersons.pdf
3. http://www.transceleratebiopharmainc.com/wp-content/uploads/2017/02/Implementation-Recommendations_20Jan17_Final.docx
4. http://www.transceleratebiopharmainc.com/assets/clinical-data-transparency/

Annex 1: How to read the Book of Good Practices

The Book of Good Practices cases are all structured in the same way as the Patient Engagement Quality Guidance. You will find that each case has a basic description, followed by icons to show in which phases of medicines continuum they fit in and which stakeholders they have involved in their work (see description of icons below). In section 2 these cases will describe how they reached each of the 7 Quality Criteria. You will see from the wheel in the beginning, which of the Quality Criteria they exemplified in (judged by an external group of reviewers). Finally, you will find the results and outcomes of each case and the lessons learned.

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

Research and discovery phase | Pre-clinical phase | Clinical study phase 1-3 | Health technology assessment | Regulatory review and approval or registration phase | Post-registration/ -launch activities | Other

- ✔ **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 1-3)**
- ✔ **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 | **Policymakers** | **Health technology assessment organisations** | **Research funders**

Patient advocates, patient organisations and associations | **Regulators** | **Pharmaceutical companies or industry** | **Other**

Healthcare professionals | **Payers** | **Researchers**

- ✔ **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)

Annex 2: Descriptions of the Patient Engagement Quality Criteria

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.

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.

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.

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.

Annex 2: Descriptions of the Patient Engagement Quality Criteria

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.

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.

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.