PATIENT FOCUSED
MEDICINES DEVELOPMENT

BoGP
2nd edition

The PFMD
Book of Good Practices

2nd edition I 2019

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#PEQualityGuidance
#PEQualityCriteria

www.PatientFocusedMedicine.org
October 2019

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

NIHR | Sheffield Biomedical Research Centre

Consulting a patient and carer group on the design and delivery of a proof of concept drug repurposing trial in Parkinson's Disease

Organisation: Sheffield Biomedical Research Centre

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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

NIHR | Sheffield Biomedical Research Centre

Consulting a patient and carer panel on the design and delivery of a proof of concept drug repurposing trial in Parkinson's Disease

Organisation: Sheffield Biomedical Research Centre

Basic Information

Background

To prepare to take a licensed drug into clinical trial for use in Parkinson's disease for the first time, the Principal Investigator on the study and Clinical Trials Manager from our organisation involved a representative panel of patients and carers from an early stage to help design the study protocol and develop trial documents. The Parkinson's UK research interest group for Yorkshire and the Humber are a panel of patients and carers who regularly meet in the region and work with researchers to help to shape their research. The group is organised and funded through a PFMD member, Parkinson's UK. The regional administrator for the group was contacted and a first meeting arranged at the Sheffield Institute for Translational Neuroscience (SITraN).

Initiative Team

- Professor Oliver Bandmann, Principal Investigator, The University of Sheffield
- Mrs Sarah Moll, Clinical Trials Manager for the NIHR Sheffield Biomedical Research Centre
- Parkinson's UK Research Interest Group (Yorkshire and Humber)

Initiative

The patient and carer members were sent draft documents relating to the proposed study in advance of meeting with the Principal Investigator and Clinical Trial Manager. The background, aims and proposed methodology were clearly explained in the face-to-face meeting and all aspects of the trial discussed with the group. Written feedback from individual members of the research interest group on the development of the protocol was obtained through email follow up and patient advice was incorporated into the documents sent for ethical review.

In brief, the background to the study highlighted that work within SITraN to conduct first drug screen in human Parkinson's Disease patient tissue had identified Ursodeoxycholic Acid (UDCA) as a potent mitochondrial rescue drug. UDCA is a bile acid that naturally occurs within the body and is marketed under multiple trade names for use in a type of liver disease and other conditions. The rationale for repurposing UDCA to slow down neurodegeneration in Parkinson's disease was explained and a range of methods to monitor progression discussed. The advice of the Parkinson's UK regional research interest group members was sought on a number of components of the study and these were discussed in detail to give the study the best chance of success in recruitment and retention of participants and their adherence to study requirements.

Patient input on the feasibility of the administration of the medication, patient visit schedule, recruitment strategy and the wearability and usability of a Parkinson's Kinetograph (a fitbit-like device) for home monitoring of motor activity helped to shape the final study protocol which received ethical approval.

The impact of their involvement was fed back to the research interest group by sending the updated study documents with changes that resulted from their involvement and they continue to be involved and updated at the trial progresses.

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

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

The quality of patient engagement

1. Shared purpose

What did you do to achieve this criterion?

The Parkinson's UK Patient and Public Involvement (PPI) programme makes the co-developed aims of regional research interest groups clear to both their patient and carer members and researchers who are service users of the groups. What all parties can expect from involvement is clearly laid out in information provided online and through contact with Parkinson's UK PPI co-ordinators. Meetings and presentations on research given by researchers, working with researchers to help shape their research, reviewing and making decisions about research projects are regular activities performed by the research interest groups. The researcher's request to meet with the Yorkshire and Humber group for consultation on a study design was approved for support by a Parkinson's UK group administrator after reviewing the drafted proposal.

A regional PPI coordinator acted as a liaison to help organize the activity and make mutually agreed arrangements. Draft documents and a series of questions in relation to them were sent to the patient and carer members in advance of an arranged meeting so they were aware of the topic to be discussed.

What is your stated "shared purpose"?

To involve patients and carers in developing a design for a proof of concept study. In particular, advice from representative patients is sought on the feasibility of the study design for participants in order to give the study the best chance of success in recruitment, retention and delivery on research objectives.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

The overarching purpose of the research interest group is agreed by members on joining and by researchers in advance of engaging with the group. The specifics of the activity were agreed by email and in person through the first meeting chaired by a Parkinson's UK PPI co-ordinator.

Have you reviewed the shared purpose and its understanding among stakeholders?

Yes. The shared purpose was revisited by a follow-up email 4 months later, prior to submitting the study documents for ethical approval and further useful feedback was obtained.

At what time points?

It will continue to be reviewed through further updates with the research interest group as the research progresses. The group meet roughly every 2-3 months to discuss a variety of research projects throughout Yorkshire and The Humber. The Principal Investigator and Clinical Trial Manager plan to update the group at a frequency of perhaps once per year or at key stages in the development of the research. The shared purpose of the interaction will evolve as things progress; for instance agreeing to meet to disseminate the research findings and have patient input in how to disseminate the findings rather than designing the research.

2. Respect and accessibility

How have you addressed respect and accessibility in this project?

This work was completed ensuring that the needs of people affected by Parkinson's were considered at every stage. The provision of information and time given for written feedback was scheduled in accordance with a co-agreed timeline. Parkinson's UK (PDUK) provide a check and balance by coordinating patient engagement with researchers and ensure that interactions are mutually respectful in line with their INVOLVE-informed guidance for PPI. PDUK actively advertise for membership to research interest groups and highlight all opportunities for involvement in clear and simple language on their online platforms and in all promotional material. They also discretionarily review research documents from researchers asking for PPI support before approving the involvement. Involvement was sought from both people with Parkinson's and people affected by Parkinson's in other ways (family members, carers, partners, friends of people with Parkinson's) through the mixed research interest group.

Conducting some of the activity via email enabled more accessible follow-up, though travel budget and refreshments for their research interest group members was provided by the PDUK.

How have you assessed with stakeholders that they acknowledge mutual respect, and that access to engagement has been optimised?

Parkinson's UK publish guidance for PPI and also provided a co-ordinator who ensured that the location, timing and format of meetings and email exchanges were acceptable to all stakeholders.

3. Representativeness of stakeholders

How have you ensured broad, competent, diverse representation of stakeholders?

A diverse representation of patients was ideal for this project to develop a feasible study protocol for a trial with fairly broad inclusion criteria in terms of age (18-75) and sex (any). As part of the study protocol involved taking medication at home and wearing a fit-bit like movement sensor, to monitor daily activities outside of the clinic, a representational mix of patients who might be working or retired, have children in the household and other lifestyle differences was desirable.

There was a good mixture in terms of male:female ratio, lifestyle, years since diagnosis and experience of research participation from both patients with Parkinson's disease and from family members in the research interest group. This representation arose through chance by the self-selected regular group membership of patients however, rather than being specifically selected for the activity, in line with PDUK recruitment for PPI. The active membership of the Yorkshire and Humber Parkinson's research interest group is typically at minimum 6-10 people who attend meetings regularly. A full group of 12 members attended the meeting for this project.

How did you check that the representation of stakeholders in your project supported achieving project outcomes?

Some patients from the research interest group volunteered their age and time since diagnosis in the group discussion. Although a reasonably representative mix of patients were assembled for the project, the group was also asked to consider the feasibility of aspects of the study from the perspective of other, potentially older or younger participants with different lifestyles.

4. Roles and responsibilities

What did you do to achieve clarity and communication as well as regular check-points on roles and responsibilities?

By outlining clear goals for the project and it's shared purpose, each stakeholder was aware of their responsibilities to the session and to each other. PDUK provide an overarching framework for PPI through their research support network that is in line with INVOLVE guidance. The PDUK PPI coordinator for the Yorkshire and Humber research interest group was defined as the go-to person in organizing the project. Mrs Sarah Moll as the future trial coordinator and point of contact for public inquiries concerning the trial was defined as the primary person to direct any questions about the practicalities of the proposed research study to.

A process was put in place through the PPI co-ordinator and Sarah Moll to:

- Follow up with all stakeholders
- Feedback to all stakeholders
- Give further support if required

How did you check that all participants understood what their roles and responsibilities are, and what is expected of them?

The role of research interest group members and the responsibilities of the researchers and members are published in PDUK research support policy and PPI guidance for researchers. The types of questions that would be posed by the researchers were communicated to the research interest group along with the study document drafts ahead of an arranged face-to-face meeting. The deadline for returning written feedback was agreed upon at the meeting.

At what frequency have you checked this in?

This was confirmed at the face-to-face meeting and in subsequent follow-up emails.

5. Capacity and capability for engagement

What did you do to support building the required capacity and capability for engagement?

The research interest group members had prior experience of reviewing and commenting on research proposals and clinical trial protocols and study documents. Parkinson's UK offers training support for their research interest group members, and members can also access free training courses through the Sheffield Teaching Hospitals Clinical Research and Innovation Office. To facilitate discussion at the face-to-face meeting, the research interest group members were given access to the draft protocol and patient facing documents as well as a questionnaire about the proposed protocol 2 weeks before attending and were given the opportunity to contact the research team with any questions before the session. Prior to the documents being sent out, they were evaluated internally by our organizations PPI lead to ensure clarity for lay readers.

The research team ensured that everyone was appropriately prepared to take part in this session prior to attendance. The project team worked with the Parkinsons UK PPI co-ordinator to plan the format of the session. Time was planned to be spent explaining the research and answering questions to ensure a full understanding of the subject. At the start of the face-to-face meeting, the Principal Investigator gave an

overview of the background research and proposed clinical trial. This was then followed by a Q&A session which was given extra time if needed.

How did you check that all stakeholders have what they need to contribute effectively and meaningfully?

This was confirmed through discussion in the face-to-face meeting and through email contact.

6. Transparency in communication and documentation

What did you do to achieve and implement processes for timely communication and updated documentation throughout the project?

Documentation shared between all stakeholders before meeting face-to-face comprised a drafted summary of the study, patient facing documents and questions to consider in relation to these as well as the research team contact details. We worked with Parkinson's UK to agree a timeline for sending out the study related literature before the face-to-face meeting and for receiving further written feedback afterwards. Further communication was agreed at the face-to-face meeting including an immediate follow up email detailing the next steps with regard to providing written feedback. The research team circulated via email the updated pre-final study protocol and documents after collating the comments and answers to 17 questions on the study regimen prior to submission for research ethics committee approval 4 months after the face-to-face meeting.

How did you validate that your communication and documentation plans were useful and appropriately implemented?

Timescales were agreed in advance with the research interest group members and to ensure that these were still reasonable and workable for them, confirmed in person at the face-to-face meeting. Following circulation of the updated study documents, further suggestions were received via email in response and incorporated into the final protocol submitted for research ethics committee approval. The excellent email responsiveness from the research interest group members is indicative that the communication and documentation plans were useful and well implemented.

7. Continuity and sustainability

What did you do to achieve this criterion?

Parkinson's researchers in the UK are very fortunate to have a well organised patient research support network available through Parkinson's UK. The regional research interest group is sustained through Parkinson's UK and continued involvement is encouraged and supported through the charity's PPI programme. At the time of writing, the PI and clinical trial manager have followed up with email exchanges at 2 timepoints following the initial face-to-face meeting to continue involvement in preparing documents for ethics committee approval. The co-designed study is now near to opening for recruitment and has a planned trial period of 30 months. In line with the availability of the group as a regional Patient Engagement resource and in response to key developments in the course of the research, a plan is in place to update the group on the study through both email and face-to-face communications.

How did you gather feedback on what you have done?

Through email follow up. Updates were sent out to the group regarding the outcomes of involvement including where suggestions were implemented and how the project has been shaped as a result of involvement.

How did you check that your planning to secure continuity and sustainability was appropriate also for the stakeholders you've involved in the project?

Through discussion at the first face-to-face meeting, the research interest group members, study PI and clinical trial manager agreed that continued involvement was of interest to all parties. The sustainability of the research interest group is thanks to Parkinson's UK. The Parkinson's UK PPI co-ordinator agreed to arrange follow up meetings in addition to the planned follow up email communication on the project.

Results and outcomes

Many useful comments on the development of the study protocol came through open discussion at the first face-to-face meeting. These were captured in minutes by the Clinical Trials Manager and collated with the requested written feedback from the group members. Changes were incorporated into the study protocol as a result of the patient engagement including wearing the home monitor 'fit-bit-like' movement sensor only for one week at the start and end of the study rather than throughout the assessment period and conducting agreed weekly phone calls from the research team to study participants to aide compliance.

Positive impact for specific medicines development phases

The project likely decreased the time to study registration through co-developed modifications to the study protocol and by adding the patient voice to the documents submitted for ethical and regulatory review. It is likely also that the refinements to study design and patient facing literature will impact positively on patient recruitment, retention and adherence to protocol.

Direct or indirect positive impact for patients

- Empowerment for patients/public who are involved
- Increased awareness of relevant clinical programmes and recruitment procedures.
- Patient voice embedded in decision making

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

The project led to:

- Better understanding of patient perspective in the acceptability of study procedures and expectations
- Smoother process through ethical approval with patient co-designed protocol and study documents

Once the trial opens to recruitment, it is hoped that the patient input into the protocol will lead to maximum recruitment and retention of participants on the project and a reduced burden on the participating patients.

Lessons learned

The Principal Investigator and clinical trial manager found the discussion at the face-to-face meeting very useful. To enable all participating members of the research team to be fully engaged in the discussion, deploying a separate administrative assistant to minute the meeting would be helpful to capture all comments and information. This is enabled by the new recruitment of an administrator to our organization.

The introduction segment of the session was very beneficial in making sure that patient volunteers understood everything and felt free to ask any questions as the project continued. Their comprehension of all aspects of the proposed study was key to co-developing the protocol. This reinforced the importance of building relationships with patients so as to facilitate a frank and open discussion of research. Continued involvement will facilitate relationship building and gives the opportunity for patient input into all stages of the research cycle.

The guidance and support for PPI provided by Parkinson's UK was excellent in planning and executing this project. The provision and maintenance of the research support network makes patient engagement activities extremely time and cost effective for researchers. Parkinson's UK were instrumental in the set-up and continuity of this PE activity.

NIHR | Sheffield Biomedical Research Centre

@SheffieldBRC

About the Sheffield Biomedical Research Centre

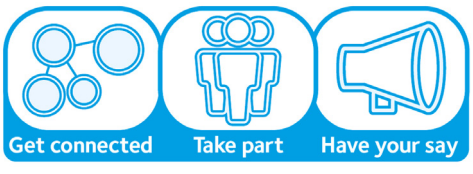
NIHR Sheffield Biomedical Research Centre (BRC) is a Translational Neuroscience research partnership between the University of Sheffield and Sheffield Teaching Hospitals NHS Foundation Trust. Our mission is to improve the treatment and care of patients living with chronic neurologic disorders by pulling through advances in neuroscience into clinical evaluation.

http://sheffieldbrc.nihr.ac.uk/

About the team

The **UP study** is led by Professor Oliver Bandmann, Professor of Movement Disorders at the Sheffield Institute for Translational Neuroscience (SITraN), University of Sheffield. Prof Bandmann, Mrs Sarah Moll, Clinical Trial Manager for the NIHR Sheffield BRC and the Parkinson's UK **Research Interest Group for Yorkshire and Humber** made up the project team.

The Parkinson's UK Research Support Network (RSN) brings together people driven to help find a cure and better treatments for Parkinson's. Anyone can join the Network to get connected with the latest research news, events and opportunities by email. There are numerous ways for people to get involved, from helping to shape, steer and take part in research, to helping share research news and findings with the wider Parkinson's community. Parkinson's UK Research Interest Groups (RIGs) are made up of RSN members, people affected by Parkinson's and researchers. They support us by increasing awareness and understanding of research at a regional level.



PARKINSON'S UK
CHANGE ATTITUDES.
FIND A CURE.
JOIN US.

Annex 1: How to read the Book of Good Practices

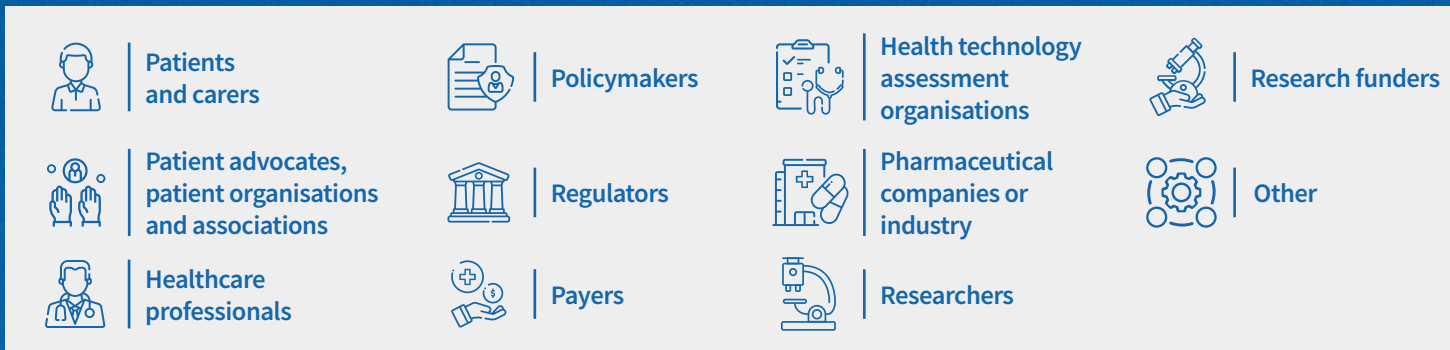
The Book of Good Practices cases are all structured in the same way as the Patient Engagement Quality Guidance. You will find that each case has a basic description, followed by icons to show in which phases of medicines continuum they fit in and which stakeholders they have involved in their work (see 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** (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.