



PATIENT FOCUSED
MEDICINES DEVELOPMENT



The PFMD Book of Good Practices

1st edition | 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

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



Supporting patient involvement in the development of new treatments

Organisation: Parkinson's UK

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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>

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Supporting patient involvement in the development of new treatments

Organisation: Parkinson's UK

Basic Information

Recruitment of patients with Parkinson's UK to work with a research team: stem cell therapy; exploring the level of understanding/benefit vs. risk.



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



Which stakeholders does this PE project involve?





The quality of patient engagement

1. Shared purpose



Values and common purpose include:

1. Exploring the level of understanding of stem cell therapy among people affected by Parkinson's and attitudes towards stem cell therapy as a potential future treatment in Parkinson's
2. Understanding minimum benefit vs risk when considering stem cell therapy treatment
3. Recruiting people affected by Parkinson's to work as part of a research team in future work

By outlining clear goals and shared purpose, each stakeholder group was aware of their responsibilities to the session and to each other. Our Research Involvement team worked with stem cell therapy research team to develop objectives and goals for involvement. These were then communicated with people affected by Parkinson's prior to the focus group meeting.

Stage 1: We worked with a UK based University research team and people affected by Parkinson's to create a survey to consult a large patient population about attitudes to stem cell therapy. The 548 survey participants self-selected from a group of over 3000 people affected by Parkinson's interested in research. The discussion group of 16 participants were selected as they had completed the "Patient Involvement in research training".

Stage 2: Following the survey, we organised a discussion session between a group of 16 people affected by Parkinson's and the stem cell research team at the University. The purpose of the session was to explore and understand the survey responses in greater depth.

Stage 3: The attendees at the discussion session received feedback about the survey and discussion session and were then invited to work as part of the research team to further develop this work and to apply for funding. We evaluated these interactions to ensure that all stakeholder's needs and goals were met.

2. Respect and accessibility



This work was completed as part of Parkinson's UK's Research Involvement Awards which brings together and facilitates partnerships between people affected by Parkinson's and the research community. Parkinson's UK's research involvement team act as the broker/neutral party, ensuring that the needs of people affected by Parkinson's are considered at every stage, that information and opportunities are timely and appropriate and that conversations and output are always respectful and reciprocal.

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). Offering several involvement opportunities (the survey and the meeting) enabled some of the barriers to involvement to be addressed and ensured that the involvement opportunity was open to people across our research community.



3. Representativeness of stakeholders

It was important as part of this project to ensure that there was representation in both the survey and the discussion group related to:

- Age
- Sex
- Years since diagnosis
- People with Parkinson's and carers

This was achieved but it is a continuing challenge for us to ensure that ethnicity and socioeconomic status are also appropriately represented.

The 548 survey participants self-selected from a group of over 3000 people affected by Parkinson's interested in research. The group of 16 participants were selected as they had completed the training. There was a good selection in terms of male/female, years from diagnosis and experience of the condition and research. But in terms of ethnic and economic/social diversity this wasn't something we measured. A diversity project will be launched this year to ensure that we have a more diverse pool of patient contributors, but this is challenging for everyone.



4. Roles and responsibilities

By outlining clear goals and shared purpose, each stakeholder group was aware of their responsibilities to the session and to each other. Parkinson's UK's Research Involvement team worked with stem cell therapy research team to develop objectives and goals for involvement. These were then communicated with people affected by Parkinson's prior to the focus group meeting.

As part of the Research Involvement Award, we monitor this accountability and have processes in place to:

- Follow up with all stakeholders
- Feedback to all stakeholders
- Evaluate stakeholder experience
- Give further support for partnership working if required



5. Capacity and capability for engagement

Both the researchers and people affected by Parkinson's involved in the discussion group had completed our Patient and Public Involvement training. This training ensured that all stakeholders understood the importance of partnership working, the goals, as well as fully understanding their respective roles.

People affected by Parkinson's were also given access to information on stem cell therapy to read before attending the discussion session and given an opportunity to contact Parkinson's UK if there were any questions related to the pre-read material before the session.



Parkinson's UK delivered training to all stakeholders involved (the lead researcher and the people affected by Parkinson's) to ensure that everyone was appropriately prepared to take part in this session. We worked with people affected by Parkinson's and the researchers to plan the session. We spent lots of time explaining the research and answering questions to ensure a full understanding of the subject and evaluated the session from both the researcher and patient perspective.

As part of the session, the lead researcher introduced stem cell therapy to begin the session - in case the attendees had not had the opportunity to read the document or had not understood it. This was then followed by a Q&A session which was given extra time if needed.

6. Transparency in communication and documentation



Prior to recruiting people affected by Parkinson's for involvement, Parkinson's UK worked with the stem cell therapy research team to develop an Involvement Plan for the project to ensure involvement was well planned. Other documentation shared between all stakeholders included:

- Pre-read information on stem cell therapy as well as detailed information on the agenda and access to facility and expenses claims form and policy
- The slides to make notes
- An immediate follow up email detailing next steps
- An evaluation of experience
- An intermediate follow up document with interim findings
- An invitation to join the research team to further develop the project
- Further documentation will include long term follow up (6-12 months)

7. Continuity and sustainability



To ensure transparency, we:

- Provided updates/feedback on the outcome(s) of involvement including where suggestions were implemented and how the project has been shaped as a result of involvement
- Evaluated patient and researcher experience and feedback
- Offered in depth follow up (immediate, mid-term, long-term)
- Recommend that further partnership working results in patients becoming part of the research team, co-applicants and co-authors



Results and outcomes

1. Parkinson's UK's perspective

- Gaining informed consent from survey participants
- Have separate facilitators and note takers to capture all comments and information
- We also changed our processes in terms of conditions of support to a signed agreement from researchers about feeding back to patient contributors as well as having a more robust plan/timelines in place for things like feedback.

2. From a researchers' perspective - the researchers had originally wanted people affected by Parkinson's to comment on the more methodological aspects of their work (lab based research) but at the discussion session the patient contributors were reluctant to do that. More preparation and support needs to be given for patient contributors to contribute this way and for researchers to ask the right questions.

Conducting a consultation (survey) to shape and inform the discussion group was an excellent way to gain a wider patient perspective along with the complementary real-life stories and in depth answers.

Positive impact for specific medicines development phases

- Understanding unmet medical need
- Understanding benefit vs risk
- Informing clinical trial development/design

Direct or indirect positive impact for patients

- Increased awareness of stem cell therapy as a treatment for Parkinson's (past trials/research, current research and future/potential trials)
- Influencing stem cell therapy trial development, making them more relevant and likely to succeed
- Increasing likelihood of new treatment for Parkinson's

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

- Better understanding of patient perspective, acceptability and expectations
- More likely to develop relevant future work
- Publication opportunity as unique area of work
- Increased chance of funding
- Empowerment for patients/public who are involved



Lessons learned

Learnings from the logistics of discussion group:

- Difficult to get enough disability parking close enough to venue
- Difficult to give good directions as the campus/venue was complicated
- Most of the group did not read the pre-read documents, but as we went through an introduction, this did not significantly affect the day
- It would have been good to have additional members of staff to help with escorting patients to room/venue
- Discussions were rich but the time consuming and resource intensive nature of using recording was not fully appreciated – it may be worth paying for a scribe to attend future sessions

Authors are currently experiencing some questions around being able to use the survey responses in a publication. Advise to get consent to publish as a specific survey question.

About Parkinson's UK

Parkinson's UK is the largest charity funder of Parkinson's research in Europe. Our vision - our ultimate ambition - is to find a cure and improve life for everyone affected by Parkinson's.

Parkinson's UK influence and fund groundbreaking research aimed at finding better treatments and advancing understanding of Parkinson's and its causes. Everything we do is driven by people affected by Parkinson's. We are an international leader in research, driving progress through collaboration with organisations from around the globe and people affected by Parkinson's to accelerate progress towards new and better treatments.

Alongside our research activities, we provide expert information and support to people with Parkinson's, their families and carers to help people manage the condition and live life to the full. We also raise awareness of Parkinson's, change perceptions of the condition and work in partnership to drive better services.



DR NATASHA RATCLIFFE

Research Involvement Manager, Parkinson's UK

Natasha is Research Involvement Manager at Parkinson's UK, leading the charity's patient and public involvement programme and supporting researchers across academia and industry to work with people affected by Parkinson's to improve research outcomes. Natasha consults on methods and approaches to involvement and facilitates partnership working to help ensure that the expertise of people affected by Parkinson's is incorporated meaningfully throughout

all stages of the research process. She also works closely with other charities and external organisations, contributing to national and global initiatives to help drive forward the field of patient and public involvement in research.



DR EMMA LANE B.SC. PH.D. FHEA

Senior Lecturer in Pharmacology

Director of Postgraduate Research Studies School of Pharmacy and Pharmaceutical Sciences Cardiff University

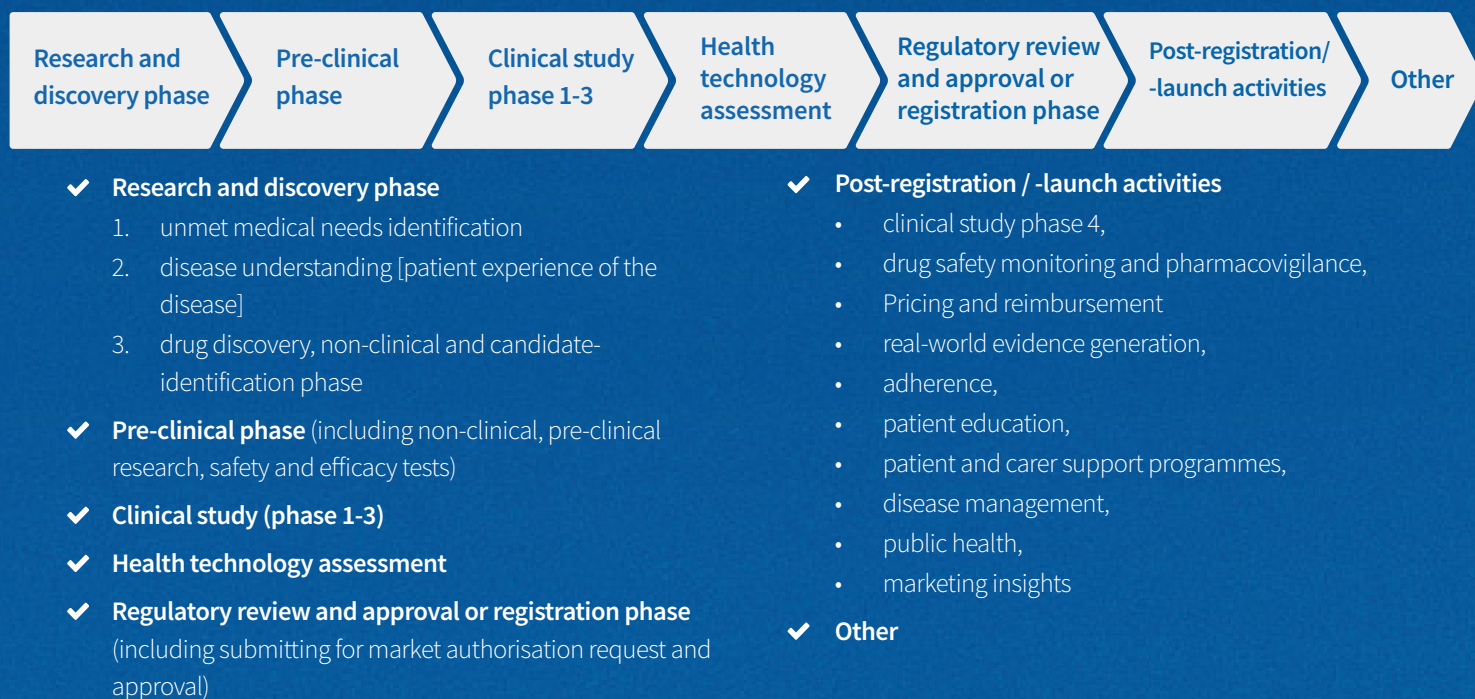
Completing a PhD in Pharmacology at King's College London in 2004 focused on Parkinson's disease, I then worked with world leaders in the field of cell transplantation and L-DOPA induced dyskinesia at Lund University, Sweden,

to understand the generation of side effects of cell transplantation as a treatment for Parkinson's disease. I then moved to Cardiff University, first as a post-doctoral researcher and subsequently securing a position as a Lecturer in Pharmacology in the School of Pharmacy and Pharmaceutical Sciences. Since then my group has continued to focus on laboratory based research into cell and gene based treatment for Parkinson's disease but with a growing parallel interest in understanding and involving the patient voice in both preclinical and clinical research through Public Patient Involvement and engagement activities. This has led to ongoing work in leading a PPI group supporting and guiding studies in South Wales on surgical interventions for neurological disorders BRAINInvolve.

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?



Which stakeholders does this PE project involve?



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.