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.

@The_Synergist
@PFMDwithPatient

#PEQualityGuidance
#PEQualityCriteria

www.PatientFocusedMedicine.org

PATIENT FOCUSED
MEDICINES DEVELOPMENT

Patients to work with researchers in the context of innovative therapy

Case from a Patient Organisation (“Organisation”)

PE project description

Recruitment of patients with Parkinson’s 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?

- ✓ **Research and discovery phase** 1) unmet medical needs identification, 2) disease understanding [patient experience of the disease], 3) drug discovery, non-clinical and candidate-identification phase
- ☐ **Pre-clinical phase** (including non-clinical, pre-clinical research, safety and efficacy tests)
- ☐ **Clinical study phase 1**
- ☐ **Clinical study phase 2**
- ☐ **Clinical study phase 3**
- ☐ **Health technology assessment**
- ☐ **Regulatory review and approval or registration phase** (including submitting for market authorisation request and approval)
- ☐ **Post-registration / -launch activities**
  - ☐ clinical study phase 4,
  - ☐ drug safety monitoring and pharmacovigilance,
  - ☐ Pricing and reimbursement
  - ☐ real-world evidence generation,
  - ☐ adherence,
  - ☐ patient education,
  - ☐ patient and carer support programmes,
  - ☐ disease management,
  - ☐ public health,
  - ☐ marketing insights
- ☐ **Other**

Which stakeholders does this PE project involve?

- ✓ **Patients and carers** (including caregivers, and family members)
- ✓ **Patient advocates, patient organisations and associations**
- ☐ **Healthcare professionals** (including clinical investigators, general practitioners , specialists, pharmacists and nurses)
- ☐ **Policymakers**
- ☐ **Regulators**
- ☐ **Payers**
- ☐ **Health technology assessment organisations**
- ☐ **Pharmaceutical companies or industry** (including medical devices and biotech companies)
- ✓ **Researchers** (academic researchers and investigators)
- ☐ **Research funders**
- ☐ **Other** (for example, contract research organisations (CRO) and hospitals)

1. Shared purpose

This refers to the project's aims and outcomes that all stakeholders taking part should agree on before starting the project. Consider putting in place processes to help facilitate discussions between all stakeholders to identify each other's values, expectations and objectives, and review and discuss priorities in the planning of the project. It can be valuable to enable stakeholders to exchange views openly to understand the scope and objectives of the project, acknowledging that some of their objectives may differ. All parties concerned should also have a shared written description of the common goals of the project.

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 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. The Organisation'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.

Stage 1: *Organisation 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 "Patient involvement in research training".*

1. Shared purpose

Stage 2: *Following the survey, Organisation 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. These interactions were evaluated by Organisation to ensure that all stakeholder needs and goals were met.*

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.

This work was completed as part of the Organisation's Research Involvement Award [program] which brings together and facilitates partnerships between people affected by Parkinson's and the research community. The Organisation'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 Organisation's research community.

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.

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 Organisation 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 Organisation measured. A diversity project will be launched this year to ensure that Organisation has a more diverse pool of patient contributors, but this is challenging for everyone.

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.

By outlining clear goals and shared purpose, each stakeholder group was aware of their responsibilities to the session and to each other. The Organisation'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, Organisation monitors this accountability and has 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

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.

Both the researchers and people affected by Parkinson's involved in the discussion group had completed Patient and Public Involvement training from Organisation. 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 Organisation if there were any questions related to the pre-read material before the session.

Organisation 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. Organisation worked with people affected by Parkinson's and the researchers to plan the session. They 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.

5. Capacity and capability for engagement

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

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.

Prior to recruiting people affected by Parkinson's for involvement, Organisation 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

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.

To ensure transparency, Organisation:

- *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*

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

1. Organisation's perspective

- Gaining informed consent from survey participants
- Have separate facilitators and note takers to capture all comments and information
- Organisation also changed its 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.

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 Organisation 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.