The PFMD
Book of Good Practices

1st edition I 2018

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#PEQualityGuidance
#PEQualityCriteria

www.PatientFocusedMedicine.org

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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

Sant Joan de Déu Research Foundation

Kids Barcelona - Young Person's Advisory Group

Organisation: Sant Joan de Deu Children's Hospital

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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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Basic Information

Background

Huge experiences, more than 20 years, have demonstrated the benefits to involve adult patients in the field of research and clinical trials. Their contributions are positive for the projects in terms of return on investment (reducing time and costs) but above all in terms of return on engagement (quality of life). Advocacy of adult patients or their representatives cover all the different stages of clinical research (from the definition of priorities to communicate the outcomes of a project).

In the case of children the scenario is different. The number or experiences are limited, which are focused basically in the information addressed to young patients involved in clinical trials (patient information sheet and assent document).

Young persons' advisory groups (YPAGs) are groups of young people interested in the field of research and clinical trials, in science and to become a young advocate. Previous to the participation in any type of research project they receive suitable training to ensure that they have the right knowledge and skills.

The Young Persons' Advisory Group acts as a scientific council of teenagers founded to improve clinical trials: the YPAG of Sant Joan de Deu Children's research hospital in Spain is connected with the Clinical Research Unit. The team was created in January, 2015. The training programme lasted six months and it included content and skills about the four topics in which the hospital is performing projects: biomedicine, research, clinical trials and innovation.

After the training program, the team became the Youth Scientific Council of the Hospital. It means that those responsible for the different projects can ask for their consultation and advice with the aim to improve their projects and ensure patient centricity.

The members of the Young Persons' Advisory Group have regular monthly meetings led by two facilitators of the team: the coordinator of the Clinical Trials Unit and the coordinator of the Patient Engagement in Research Area.

The methodology of every session follows a systematic process addressed to:

- Educate and empower the young people in the specific topic of the project for which their help is requested. For example, improve the language, content and format of the assent document.
- Dynamic interactivity to collect feedback aiming to improve the project by using the best methods to collect the information. For example, focus groups, questionnaires or surveys, personal interviews, etc.

The content and methods of the sessions are designed specifically for each project. In the first part of the session the principal investigator of the project is the expert responsible to educate the young people on the topic of the session. In the second part of the session, the facilitators perform the practical activity to facilitate the process to discuss delivery and contribution to the project.

In the last three years the Young Persons' Advisory Group team has been involved in several projects. Below are some examples:

- **Survey to know children and teenagers' opinion about drugs and involvement in medical research.** The Paediatric Committee (PDCO) is the European Medicines Agency's (EMA) scientific committee, where the national agencies are taking part to ensure the safety, efficiency and adequacy of medicine specifically for the children population. PDCO prepared an easy survey with eleven questions targeting children and teenagers between 10 and 18 years old, with the goal of knowing their opinion about drugs and their involvement in the medical research (clinical trials with medication). Answers had to be provided from March until May 2015. The goal of this survey was to obtain information about preferences of this population related with pharmaceutical dosage and types, as well as the difficulties they may face when consuming these medications. In addition, the intention was to know their opinion about their possible involvement in research studies with drugs. The Young Persons' Advisory Group members, in collaboration with their educational institutions, collected more than 500 answers in Spain. Currently, analysed information is in publication phase (scientific publication) with the other data collected across other European countries
- **European regulation about clinical trials.** In 2016, a consultation about ethical aspects of the European Paediatric Clinical Trials Regulation was opened. Young Persons' Advisory Group members represented by iCAN (International Children's Advisory Network) Youth Committee joined the public consultation period to review and suggest improvements to the legislation text.
- **Feedback about the clinical trials studies addressed to pediatric patients.** The members of the Young Persons' Advisory Group reviewed drafts of clinical trials submitted by the pharmaceutical industry, in particular about the treatment of:
  - Flu
  - Cystic fibrosis

  The questions that they commented on and that allowed the improvement of the initial study draft proposal were: palatability, frequency of medical follow-ups and number of medical tests, quality of life data, formulation preferences, information for patients and families, etc. At the moment, in collaboration with other groups, the Group is planning to write a "white paper" that will allow to unify working procedures with the pharmaceutical industry and regulators, with the aim of standardising ways of collaboration.

- **Launch of the European YPAG Network (eYPAGnet).** In May of 2017, the eYPAGnet achieved the recognition of EnprEMA and was officially launched. The specific European regulatory environment of paediatric clinical trials and the international and multicentre methodology to perform the studies, encouraged the creation of this network. During the next three years, the SJD Children's Hospital is going to be the coordinator of the network.

  The goals of eYPAGnet are:

  - Improve the capacity of collaboration with the different agents, who participate in the research process and development of innovative drugs
  - Gather a variety of experience related with different pathologies
  - Promote the planning and development of clinical research initiatives for children at the European level
  - Consolidate the curriculum of capacity-building and empowerment training programs for young patients
  - Promote and lead the creation of new groups
  - Empower the selection of professional careers in the scope of science, among the youth
- **Collaboration with the Ethics Working Group of EnprEMA (European Network of Paediatric Research of EMA).** Partly harmonized Informed Consent/Assent template - document for paediatric CTs, was prepared during 2016. The document template was reviewed by the members of Eypagnet about the usefulness and understandable text contents, and it will be finalized after the careful review against the new "Pediatric Ethics Guideline" together with comments of the experts from the European Academy of Paediatrics (EAP). The template will be made publicly available on Enpr-EMA website.
- **Framework for involvement of patients in the activities of EMA.** Young Persons' Advisory Group was consulted by the EMA in the process to design the "Principles of involvement of patients in the activities of EMA". In the upcoming months authors are going to help in the process of its implementation.

Initiative update 2019

Kids Barcelona is already involved in several European Projects focused in the young people engagement in clinical research with the aim to represent this group of patients. Currently we are participating in the PARADIGM and the Connect4Children project, both of them granted by the Innovative Medicine Initiative (IMI). On the other hand, the collaboration with the other young persons advisory groups (YPAG) around Europe is growing up thanks to our involvement as coordinators of the The European Young Person's Advocacy Group (eYPAGnet).

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

Which stakeholders does this PE project involve?

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

Other: CRO

The quality of patient engagement

1. Shared purpose

- Planning meetings with the stakeholders (researchers, pharma company, regulator, etc.) who request the scientific advice of the YPAG.
- A meeting to evaluate the feedback of the YPAG in terms of suggestions and improvements of the assessed project.
- Regular meetings with the most active researchers and clinicians involved in the clinical trials performed in the Hospital.
- Participation in events to introduce what is a YPAG and to inform about the expertise of Young Persons' Advisory Group.
- Report the outcomes of the feedback session with the YPAGs will be sent to the stakeholders and this will analyse in qualitative way the results of the patients' involvement
- Maintain at least one annual meeting with the researchers and clinicians involved in the clinical trials performed in our Hospital
- Participation in at least three events per year to introduce Young Persons' Advisory Group team
- In every session, the members of the YPAG know in advance the topic, the project and the objectives of the activity. The methodology always has a win-win focus; on one hand, the training of the young people, and on the other hand their contribution to different projects (clinical trials, innovation, etc). The first YPAG in the world was created in 2006 in Liverpool, and there is a very close collaboration. Their experience was very useful to grow the team (in Spain).

2. Respect and accessibility

- Involvement of paediatric patients and healthy young people. In the case of patients, they are suffering from different types of diseases in order to ensure the diversity of their feedback based on their experience as patients. Their experience in participating in a clinical trial is an important criterion when selected as a member of the group. The number of young people involved in the YPAG is stable during a year, and they can belong to the group at least until 18 years. The longer they are involved, the more expertise and capabilities they can offer to different projects through contributing with their "scientific advice".
- Offer the support that patients with special needs can have to be involved in the Young Persons' Advisory Group: transportation, accessibility, dietary restrictions, etc.
- Inform all the members of the group and their families about the special needs that one member of the group can have. E.g. immunosuppressed.
- To have at least 50% patients as members of the YPAG and not more than 2 young people with the same disease (e.g. cancer)
- To have members with different ages in the range between 12 to 18 years old.
- To have boys and girls in an equal distribution.

3. Representativeness of stakeholders

- Young patients are being recruited with the involvement of researchers and clinicians that perform clinical trials in the Hospital.
- Experience as patient participant in a clinical trial will be considered in the recruitment process of patients.
- Scientific interest will be a mandatory feature for all the candidates.
- Good level of English is also mandatory to ensure the involvement of the members of the YPAG in international projects.
- The minimum age to be considered in the recruitment process will be 12 years old. At this age in the country where the work is taking place you have to sign the assent document.
- They have members that represent different socioeconomic backgrounds and diseases. Also healthy young people are involved to ensure that they can't have the bias of the disease and ensure that the general feedback that the team can provided is not connected with an specific condition. Related to the young patients they have members with chronic diseases (e.g. diabetes) or patients with complex illness conditions (e.g. cancer).
- They have experience to work with different stakeholders, but for privacy reasons they can not share names or data. Basically they are: pharma companies, research centers that perform non-commercial clinical trials, regulators and ethics committees.
- Annually, a recruitment process is set up, the previous criteria will be considered and also the result of an individual interview with the facilitators of the YPAG.

4. Roles and responsibilities

- The YPAG has two coordinators with experience in the field of clinical trials and education. They are the facilitators of the feedback sessions and the liaison with the different stakeholders. Any request of the stakeholders is going to be addressed to them by email or phone. A common email account was created in order to facilitate the interaction between the two facilitators and the stakeholders.
- The members of the YPAG have also internal responsibilities in the group: president, secretary, education committee, means of communication committee, etc. (they are elected for 3 years).
- Facilitate interactions with the stakeholders.
- Provide always feedback in "real" projects (protocols, assent documents, etc).
- Stable involvement of the facilitators/coordinator at all the time, with independence of the project in which the team is involved. In any case, the role of professionals with expertise in the field of clinical trials and education will be needed.
- Annual approval of the internal role and responsibilities of the young people involved in the YPAG. The minimum roles will be: president, secretary, coordinator of the education committee and coordinator of the means of communication committee.
- Participate in meetings with stakeholders to introduce the methodology of patients' involvement that is behind the YPAGs.
- Use official documents and projects to provide scientific feedback.

5. Capacity and capability for engagement

- A common training process will be offered to all the new members of the YPAG. The training programme will be focused in four areas of expertise: biomedicine, clinical research, innovation and clinical trials. These four fields are related to the different research projects in what the members of the YPAG can be involved.
- During the scientific advice sessions a stakeholder representative involved in the project (researcher, pharma company, regulator, etc.) is going to participate in the activity. This is to ensure that the content, terminology and goals of the project are understandable for the members of the YPAG.
- Annually they perform a survey with the members of their team to know the evaluation of the training program and the projects in which they were involved. The biggest benefit for them is to know that they are helping other sick kids. Second, the access to a rigorous and innovative scientific knowledge, and last but not least, the development of personal/professional skills. The older members of the group help with the training process of the new members.
- A test is mandatory at the end of every training module to measure if the young people have achieved the educational goals.
- Specific training content is going to be offered to the young advocates related to the project in what they will be involved. This is going to be offered by the stakeholder representative.

6. Transparency in communication and documentation

- A private agreement will be signed by the parties (stakeholder and the SJD Children's Hospital) to ensure that the rules about documentation and confidentiality are aligned. When involving minors this is mandatory to ensure that the ethical principles are respected.
- The members of the YPAG sign an agreement as volunteers of the project. In this document the rules about confidentiality, transparency and ethics are detailed.
- With the agreement of the stakeholders they are going to spread the word about the main outcomes of the different projects in what the young people have been involved.
- Signing an agreement is mandatory to start any type of project collaboration by all stakeholders.
- Signing an agreement as a volunteer of the YPAG will be mandatory to all the members of the YPAG.
- The dissemination of the projects in which the members of the YPAG have been involved is going to be done on the website of Young Persons' Advisory Group.

7. Continuity and sustainability

- In the private agreement with the stakeholders the economic value of the scientific advice provided by the YPAG is detailed. It can be translated into an economic income to ensure the sustainability of the group or in an in-kind contribution to improve the skills and capabilities of the young members (e.g. develop a new educational resource).
- All the projects and good practices are going to be collected in the annual report of the YPAG.
- A detailed mapping database of stakeholders will be updated.
- All the relationship with the stakeholders will be detailed in the private agreement.
- The annual report is going to be sent to all the stakeholders involved in the year's project and to the contacts of the mapping stakeholders database.
- Quarterly update of the database to include new contacts.

Results and outcomes

Positive impact for specific medicines development phases

- Identification of young patients' preferences about medicines that can impact directly in the adherence to the treatment (E.g. taste, formulation, doses, etc).
- Suggestions related to protocol design
- Improvement of the information related to Patient Reported Outcome Measures (PROMs)

Lessons learned

At this moment, we don't have standardized templates to be used in all the studies they are involved in. Currently, the limited experience with young patients advocacy requires a collective work with all the stakeholders to standardise procedures. For this reason authors are leading the European YPAG network (eYPAGnet). One of the activities that they will perform next year is the development of a guideline about the young patients involvement in drug development. They hope to have common templates and tools to be used across Europe.

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About Sant Joan de Déu Research Foundation

Sant Joan de Déu Research Foundation was created in 2002 to provide a framework for the research activity which is carried out in the biomedical and social spheres at Sant Joan de Déu Maternal and Children's Hospital in Esplugues, at Sant Joan de Déu Healthcare Park in Sant Boi de Llobregat and in others.

Picture: Kids Barcelona coordinators Begonya Nafria and Joana Claverol

We approach our research as a participative and interdisciplinary process in which the interaction between our healthcare professionals and society is essential. Patients and families are the heart of our work and they are engaged to be involved in our initiatives to ensure its patient centricity.

The research is organised around our Institut de Recerca Sant Joan de Déu and covers 7 areas of knowledge, primarily within the fields of maternal and children's health and mental health. We also have research groups working in other spheres.

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.