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Guide for researchers to address patient and public involvement (PPI) in clinical trials

1. Objective and scope

This guide should be read in conjunction with the **SCTO's PPI Fact Sheet**,[1] which explains the overall concept of **patient and public involvement (PPI)**, its benefits and challenges, and how it can be incorporated in clinical trials.

The objective of this guide is to **support you as a researcher** to identify opportunities within your clinical trial that can inspire effective and meaningful PPI when you start planning your project and apply for potential funding. This guide is not intended to serve as comprehensive guidance on how to perform PPI.

Patients can offer a unique perspective on research. Through their experience with a disease or condition, patients and patient representatives know best which aspects are most relevant to them. By sharing this specific knowledge, they can contribute to the **quality, feasibility, relevance, and credibility of clinical research**. Importantly, this can have an effect on the **recruitment and retention** and hence the success of the trial. From an ethical point of view, one can argue that patients should have an influence on research that affects them, in line with the motto "nothing about us without us".

There is no one-size-fits-all approach to PPI. The level of involvement and the methods you choose may differ depending on the characteristics of your clinical trial. **The points below are meant to serve as recommendations and as a starting point for your considerations on what fits best with your clinical trial.**

In section 7 "Potential pitfalls", you will find some concrete examples of insufficient PPI statements. Suggestions are provided on how they can be improved. It is important that you provide justified reasons when something is not feasible within your specific clinical trial.

Please note that for reasons of simplicity, the term "patient representatives" is used throughout this document, and it encompass patients, patient relatives, caregivers, patient organisations, patient experts, patient advocates, and the public at large.

[1] Available on the SCTO's website.

2. General considerations

A meaningful PPI strategy is not a box-ticking exercise. Therefore, make sure that your PPI strategy:
– involves patient representatives in the early planning phase of your clinical trial
– goes beyond a statement of intention, meaning it provides evidence for PPI involvement with concrete examples
– adds value to your clinical trial and clearly highlights its added value
– reflects on the impact that PPI had on the study design and the impact it will have during the trial.

3. Lay summary

When asked to provide a lay summary of your study concept, please make sure that someone who is not familiar with research can understand the general scope of your trial. Here are some recommendations to consider:[2]
– Use conversational language with everyday words.
– Avoid acronyms and abbreviations.
– Use lay terms or explain medical terms.
– Use short, informative headings.
– Use short, simple sentences with one message per sentence.
– Test if the summary works by reading it to a lay family member or friend.

4. Identification of research question(s) and study design

See points 1 and 2 in the annex's graphical overview of possibilities for PPI.

When planning your clinical trial, we recommend considering the following points for your strategy and resource planning:

Research question(s)
– Consult with local/national patient representatives when defining research question(s) to ensure the questions you want to answer are relevant for patients and reflect their needs, for example through online surveys, interviews, social media, and crowdsourcing platforms.
– Explain how and to what extent input from patient representatives has been integrated into your clinical trial.

Study endpoint(s)
– Consult with patient representatives regarding whether the endpoint(s) you propose is/are **meaningful from a patient perspective**, in other words with regard to general health improvement, improvement of the quality of life, etc.
– Highlight **how patients can benefit from your research**. Does your research have the potential to be a game-changer, addressing an unmet medical need?

Recruitment strategy
– Consult with patient representatives to discuss your **patient recruitment and retention strategy**.
– Highlight the input you received and how you integrated it in your overall recruitment strategy.
– If needed, train the team staff on how to interact with specific patient groups.

Resources for PPI activities
– Within your clinical trial budget, allocate sufficient resources to compensate patient representatives for their contributions.
– Make sure you have allocated enough research staff to take care of PPI activities, for example a PPI facilitator who is the primary point of contact for all patient representatives.

[2] Source: Presentation by Prof. DK Theo Raynor, University of Leeds, UK & Luto Research, at the SCTO Forum, Bern, January 2021; Communicating clinical trial results to patients and the general public: What to watch out for

– Consider that patient representatives **may need to be trained** prior to their involvement so they clearly know what is expected from them and understand the overall research concept.

Finding patient representatives (PPI contributors)
– Define **clear criteria** (age, gender, demographic characteristics, etc.) for the selection of your patient representatives. Consider expertise and diversity of expertise.
– Make sure the criteria you have defined are **not too selective** so that you are able to find suitable PPI contributors.
– Identify **patient organisations** that can help you identify patient representatives.
– Consider **advertising for patient representatives** via your website, social media channels, or any other relevant communication tools.
– Consider **approaching EUPATI Switzerland**[3] to help you find patient representatives that fulfil your criteria.

Data protection and sensitive data:
– Define how **sensitive data** (information that patient representatives might provide about their experience living with the disease, their health condition, etc. while being involved in your research project) will be handled. A special **consent form** signed by both parties could be useful.
– Prepare **clear instructions** on how patient representatives need to handle sensitive data they might have access to during their involvement in data collection or analysis.

5. Trial management, conduct, and data analysis

See points 3 and 4 in the annex's graphical overview of possibilities for PPI.

When planning the degree of patient representative involvement during your clinical trial, we recommend considering the following points:

– Clearly define the **role of patient representatives** within your clinical trial and your **expectations**.
– Clearly identify **how** and **when** patient representatives will be involved during your clinical trial, for example:
  – become a member of the safety board/steering committee/advisory board
  – support communication with study participants, for example to ensure new safety and patient information is communicated to them in an understandable way
  – provide input on the analysis of the data from a patient perspective.

6. Dissemination and evaluation

See points 5 and 6 in the annex's graphical overview of possibilities for PPI.

When planning the degree of patient representative involvement in the follow-up phase of your clinical trial, we recommend considering the following points:

– Clearly identify **how** and **when** patient representatives will be involved after your clinical trial ends (cite (1)), e.g.
  – support the communication of research results to the participants in the study and the public at large
  – support the publication of the research results, e.g. in scientific journals or via social media channels
  – help summarise research results in lay language for publication in research databases
  – participate in conferences where **patient representatives are integrated** e.g. in a special session for lay people or in a session co-organised with a patient organisation involving patients as speakers.
– Make sure you use an **appropriate form of communication** to reach the target group(s).
– Describe how you plan to evaluate PPI activities and their impact during and after the study.

[3] Visit EUPATI Switzerland's website for more information.

7. Potential pitfalls

This section provides examples[4] of insufficient PPI statements and suggestions on how to improve them.

"Patient organisations have been involved in the design of the study."

➔ It is important to explain **how this has been achieved** and what has changed or been improved in the design of the study as a result of patient involvement.

"Patient engagement/involvement is not applicable or relevant to the proposed project."

➔ It is important to explain **why PPI is not applicable.** No matter how complicated or technical the research is, there is **rarely an example in which patients cannot be involved at all**, for example they can be involved in the dissemination activities at the very minimum.

"We could not find a relevant organisation./A relevant organisation does not exist./The disease is too rare."

➔ You may consider contacting an umbrella organisation. EUPATI[5] may connect you with another European patient organisation.

"The applicants are in contact with patients and patient organisations so patients will be engaged/involved throughout the research project."

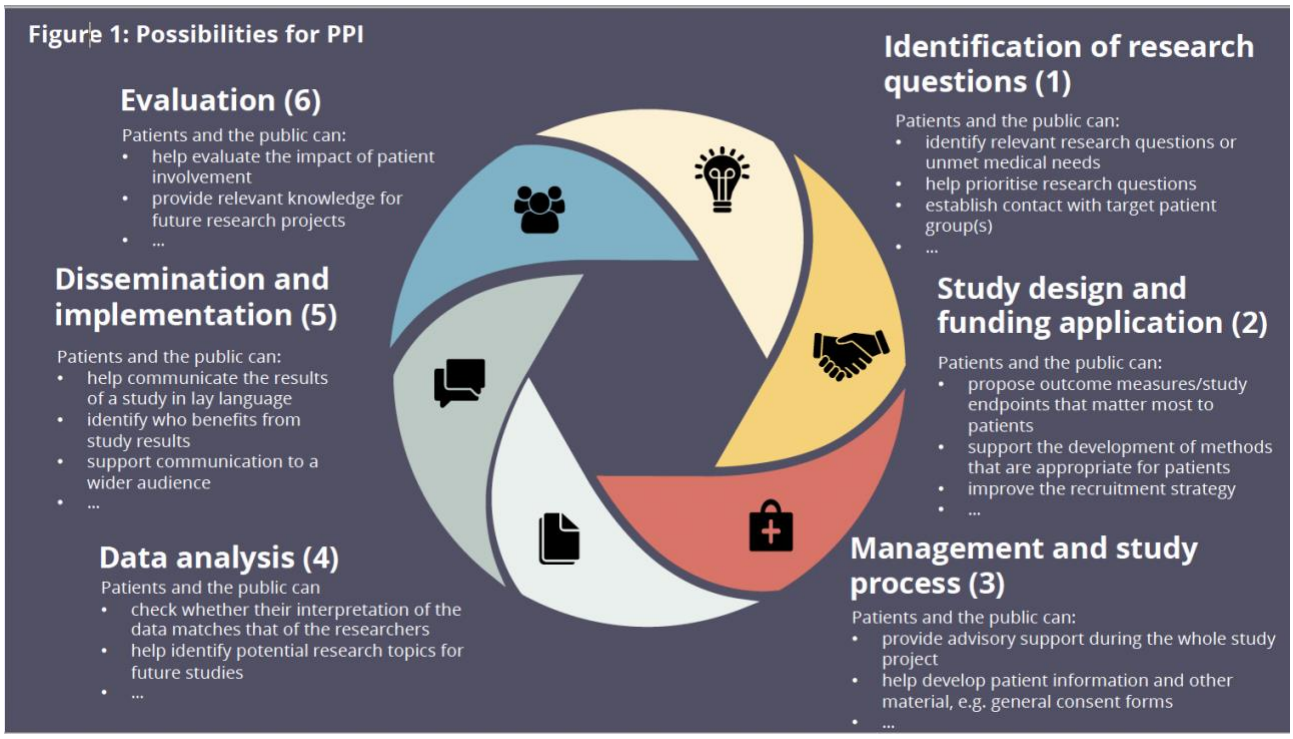
➔ Any **specific roles and responsibilities need to be discussed and agreed upon** between the researchers and the patient organisations (or patients), ideally before submitting the proposal. These prior agreements need to be detailed in the proposal. Generic statements are not useful to evaluators and need to be expanded to include the descriptions of the responsibilities of the different partners.

"Patient representatives will be invited to attend scientific meetings/conferences."

➔ Although inviting patients to hear about the results of the research should not be discouraged, it is important to think about:
- the specific role of patients who have had a meaningful role in the project in presenting the results and information in accessible language (i.e. for non-scientists)
- supporting patients to attend conferences (e.g. with fellowships or bursaries to cover travel expenses)
- having patients/a patient organisation take a role in the conference's programme, for example within the program committee, as a speaker, as a session moderator, or as panel member.

[4] See also Short Guide on Patient Partnerships in Rare Disease Research Projects, Patient Engagement in Biomedical Research Projects (accessed on 30 June 2021)
[5] Visit EUPATI's website for more information.

8. Annex



9. Further resources

(1) https://www.ejpraregiseases.org/wp-content/uploads/2021/03/SHORT-GUIDE-ON-PATIENT-PARTNERSHIPS-IN-RARE-DISEASE-RESEARCH-PROJECTS.pdf (accessed on 30.06.2021)

(2) https://www.rand.org/pubs/research_reports/RR2678.html (accessed on 30.06.2021)