



PATIENT FOCUSED
MEDICINES DEVELOPMENT



The PFMD Book of Good Practices

2nd edition | 2019

MESSAGE FROM PFMD

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The PFMD Team

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THE BOOK OF GOOD PRACTICES IS AVAILABLE AT:

<https://involvement-mapping.patientfocusedmedicine.org/book-of-good-practices>

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PATIENT FOCUSED
MEDICINES DEVELOPMENT



ENVISION PHARMA
GROUP

made
with
patients

Patient involvement in preparing clinical research peer-reviewed publications or results summaries: A systematic review

Organisation: Envision Pharma

The PFMD
Book of Good Practices

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Patient involvement in preparing clinical research peer-reviewed publications or results summaries: A systematic review

Organisation:
Envision Pharma



Basic Information

PE project description

Short summary that describes: the background and need for the project; the project objectives including anticipated benefit and/or expected outcomes; the methodology used/proposed and the project itself; stakeholders involved (including level or type of involvement as relevant).

- **Background and need for project**

Patient involvement is being encouraged throughout the development lifecycle of new medicines and devices. Many stakeholders (eg, patients, carers, regulators, payers, drug and device companies) have welcomed patient involvement as an important and fundamental change in the development lifecycle, and have promoted the potential benefits that meaningful, transparent, and ethical interactions with patients could bring. As with any change, however, research should be conducted to ensure the potential benefits and harms of patient involvement are understood, and that evidence-based best practices can be identified.

Compared with research on patient involvement in the clinical trial process, there appears to have been relatively limited research on patient involvement in peer-reviewed publication process.

Publications can affect patient care and we and others have shown that patients are engaging with the

peer-reviewed literature. Consistent with this interest from patients, medical journals are striving to facilitate greater patient involvement in the peer-reviewed publication ecosystem (e.g. as authors, peer-reviewers, readers). The extent of published evidence on patient involvement in peer-reviewed publications, however, is not known.

In addition to sharing clinical trial results through the peer-reviewed publications, results can also be shared through clinical trial results summaries. The forthcoming regulatory requirement in Europe to provide plain language clinical trial results summaries has driven strong interest in this method of results sharing. The extent of published evidence on patient involvement in clinical trial results summaries, however, is not known.

This systematic literature review is directed toward audiences who want to know the size and quality of the evidence base that exists to guide patient involvement in peer-reviewed publications and clinical trial results summaries.

- **Objectives and anticipated benefit/outcomes**

Our primary objective is to quantify the number of peer-reviewed publications that investigated the effect of patient involvement on preparing peer-reviewed publications.

Our secondary objectives are to:

- a. Quantify the number of peer-reviewed publications that investigated the effect of patient involvement on preparing regulatory-standard clinical trial results summaries.
- b. Evaluate the quality of the evidence reported in the eligible publications.
- c. Describe the number and the background (e.g. patient experts, clinical trial participants, patient advocacy group members) of patients contributing to the preparation of the publications or results summaries.
- d. Categorise the type of patient involvement (e.g. as authors, as non-author contributors).
- e. Describe the number and type of patient involvement outcomes assessed (e.g. benefits, harms, best practice recommendations, other).

By conducting this world-first systematic review, we will be able to raise awareness of the size and quality of the evidence base that exists to guide best practice for involving patients in preparing peer-reviewed publications and clinical research results summaries. This robust review will allow us to share recommendations for maximising the benefits and minimising the harms of involving patients in publications and results summaries.

- **Methodology**

This systematic review was registered in the PROSPERO database (PROSPERO 2018 CRD42018084452), conducted according to a pre-specified protocol, and will be reported in compliance with best-practice reporting guidelines for systematic reviews (PRISMA guidelines) and research involving patients (GRIPP2 guidelines). To minimise the risk of research waste, we searched (5 June 2017) the PROSPERO database to ensure we were not duplicating a planned or ongoing systematic review. We also registered our review on SYNAPSE, the Patient Focused Medicines Development (PFMD) repository for patient engagement initiatives

<https://involvement-mapping.patientfocusedmedicine.org/initiatives/first-systematic-literature-review-planned-and-conducted-with-patient-experts-on-patient-involvement-in-preparing-clinical-trial>

Within this study, ‘patient’ was defined in broad terms, based on an existing definition [PFMD/National Health Council, 2017] and input from our patient partners. For this research, ‘patient’ refers to “people having or at risk of having medical condition(s), whether or not they currently receive medicines or vaccines to prevent or treat a disease” as well as “the family and those voluntarily caring for those with the medical condition(s), patient advocates, and patient groups.”

Further details on the methodology can be found on the PROSPERO record.

- **Stakeholders involved**

To co-create this systematic review, our research and publication team involved multiple stakeholders as equal partners. Stakeholders represented patients, publication professionals, academic researchers, medical journal editors, and medical affairs staff.

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



Other: Peer-reviewed publications are generated throughout the medicines development lifecycle; they are used in submissions to regulatory and health technology assessment organisations. Clinical research results summaries are generated throughout clinical trial phases.

Which stakeholders does this initiative involve?



Other: Medical journal editor; Medical affairs service provider

The quality of patient engagement

1. Shared purpose



What did you do to achieve this criterion?

The shared purpose of this project was agreed to:

1. Verbally - at the author candidate calls and during the author kick-off meeting
2. In writing – every author signed an official [Authorship Agreement](#) that outlined the shared purpose of the project.

What is your stated “shared purpose”?

To conduct the first systematic literature review on patient involvement in preparing clinical research peer-reviewed publications or results summaries.

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?

All stakeholders had to sign the [Authorship Agreement](#), which was written in plain language to enhance understanding by all authors – patient authors and non-patient authors. By signing the Agreement, stakeholders had to confirm they understood the purpose of the project, the expected contributions they would need to make, and what rules would need to be followed to ensure the quality of the project would lead to a successful outcome.

Opportunities to influence the original plan were provided during development of the protocol, at authorship meetings, and during the development of presentations and the publication of the results.

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

Yes, at multiple time points.

- Before the project started (e.g. during the author candidate calls and author kick-off meeting).
- During the project (e.g. at authorship calls).
- After the project (e.g. the [Patient Authorship Experience Tool](#)); this tool will be completed by all authors at the end of the project. We developed this tool based on the PFMD Patient Engagement Quality Guidance Tool and incorporated patient and non-patient author feedback as part of a co-creation process.

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

To help ensure respect and accessibility in this publication project, we:

- Prepared a plain language [Authorship Agreement](#)
- Prepared a plain language summary of the [Good Publication Practice 3 guidelines](#)

- Clarified payment considerations in the [Authorship Agreement](#)
- Provided practical ways to meet as authors and gain feedback from authors (e.g. set up webinars, provided instructions for joining, setting – where possible – ‘generous deadlines’ for author review and feedback cycles)
- Providing electronic copies of documents to all authors
- Ensured all authors were aware of the rules of conduct when working together as co-authors on a publication (e.g. via the Authorship Agreement, [GPP3 plain language summary](#))

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

At authorship meetings, we proactively asked patient authors (and non-patient authors) for feedback and, if they were unable to attend a meeting, they were invited to share their feedback via other means (e.g. a 1-to-1 call, via email).

Where possible, efforts were also made to meet and engage with patient authors face to face (e.g. at conferences) to help build rapport and respect.

Testament to the respect shown to patient authors, they were also invited to present at conferences (i.e. demonstrating respect for the unique and valuable insights patient authors could bring, not just to this project, but to other projects).

The [Patient Authorship Experience Tool](#) that we developed for this project (to be completed by patient and non-patient authors on project completion) specifically asks about respect and accessibility.

3. Representativeness of stakeholders



A number of authors on the publication are employees of Envision Pharma Group. Patient diversity is one of the core principles (see below) that Envision employees must respect when partnering with patients, as has been done in this project.

Our Principles for Partnering with Patients*

4. Substantial involvement

We will seek to involve patients substantially (eg, early, often, usefully). We will avoid tokenistic involvement.

3. Ethical involvement

We will build trust with patients by complying with relevant guidelines and regulations.

2. Patient diversity

We recognise that patients differ (eg, in health literacy, culture, demography) and that our partnerships should reflect that diversity.

1. Respectful partnership

We respect the unique and valuable insights of our patient partners and their right to contribute to, and benefit from, robust and relevant evidence. We respect our pharmaceutical partners who strive to enhance patient outcomes. We are a trusted partner to patients and clients.



5. Leveraging technology

We will develop and use Envision's technology solutions to promote, document, and track effective and ethical involvement.

6. Co-learning

We will build trust with patients by complying with relevant guidelines and regulations.

7. Co-creating

We will work WITH patients to create content and solutions that are trusted and valued.

8. Evidence-based enhancement

We will contribute to and support evidence-based best practices to enhance patient involvement.

We involved authors from Europe, North America, and the Asia Pacific region. Our authorship team comprised female and male authors, with a range of ages and ethnic backgrounds. All authors were well educated.

The patient authors we sought to partner with had to be representative of the patients most likely to be interested in the topic of this systematic review i.e. informed and empowered patients who may be interested in authoring peer-reviewed publications or clinical research results summaries (see schematic below).

We recognise that these patients, who are leaders in their field and recognised for their expertise in empowering patients, do not represent the whole spectrum of patients. However, the patient authors on this project do represent the patients most likely to be interested in and benefit from this project. In the years to come, as more patients become informed and empowered partners in the publication ecosystem, a broader outreach strategy could be used.

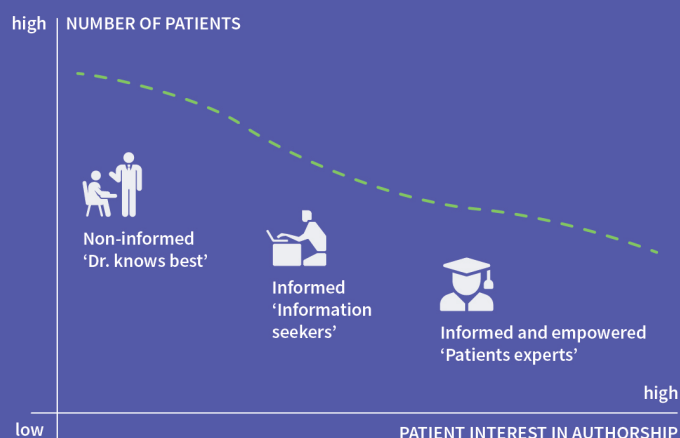
Patients as authors

Patient diversity and representativeness

Diversity: Which patients may be most interested in authoring peer-reviewed publications?

Representativeness: Are there patients represented on your publication authorship team?

Consensus is lacking in terms of a model that captures patients diversity. Models such as Amstein's Ladder of Citizen Participation or the IAP2 Public Participation Spectrum, have been criticised. No model has been co-created with patients to capture patients diversity in the publication ecosystem.



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

The [Patient Authorship Experience Tool](#) that we developed for this project (to be completed by patient and non-patient authors on project completion) specifically asks about the representation of stakeholders.

4. Roles and responsibilities



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

Before the project

The roles and responsibilities of all authors (patient and non-patient authors) were outlined in written

documents before the project started:

1. Written [Authorship Agreement](#)
2. Plain language summary of [Good Publication Practice guidelines](#)

During the project

We also had regular checkpoints (e.g. author calls) during the project so that responsibilities (e.g. providing feedback on documents) could be clarified and reinforced. Communication was encouraged during the calls and at any time between calls (eg, 1-to-1 calls, emails) if any author required further information / explanation.

After the project

The [Patient Authorship Experience Tool](#) that we developed for this project (to be completed by patient and non-patient authors on project completion) specifically asks about roles and responsibilities, and transparency in communication and documentation.

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

All authors (patient and non-patient authors) had to review and sign the [Authorship Agreement](#) to document that they understood their roles and responsibilities.

At what frequency have you checked this in?

As noted above, checks on understanding roles and responsibilities and what was expected of all authors (patient and non-patient authors) were built into this project ie, before, during, and after the project.

5. Capacity and capability for engagement



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

All authors on this project had to have the capacity and the capability to meet authorship criteria. We had to select author candidates who we believed would:

- Dedicate the time required to participate as an author and
- Have relevant expertise to make unique and valuable contributions as authors

Meeting authorship criteria is a key principle of Good Publication Practice and is a requirement to publish in respected peer-reviewed medical journals. We had to make sure, before starting this project, that the patient authors (and indeed non-patient authors) could meet the authorship criteria. It is unethical to have 'guest authors' (i.e. individuals who are named as authors, but do not have meet authorship criteria).

The capacity and capability requirements for this project are reflected in the authorship criteria. These criteria were outlined for all authors in the [Authorship Agreement](#) and in the plain language summary of the [Good Publication Practice guidelines](#).

To help build additional capabilities among the authors, we provided examples and information on some of the recent innovations in publishing (e.g. QR codes to video clips, translated language audio/print summary; infographics; protocol registration repositories). We included a number of these innovative features in a research [poster presentation](#) (extract below) that we co-created with patient authors on this project.



Patient Involvement in Preparing Clinical Research Peer-Reviewed Publications or Results Summaries: A Systematic Review

25

Karen L. Woolley^{1,2}, Anne Clare Wadsworth³, Kawaldeep Sehmi⁴, Beverley Yamamoto^{1,2}, Richard Stephens⁵, Lauri Arnstein⁶, Rachel Jones⁷, Arabella Sargent⁸, Thomas Gegany⁹

¹Proctor & Kee, Envision Pharma Group, Tokyo, Japan; ²University of Queensland, Brisbane, Queensland, Australia; ³University of the Sunshine Coast, Maroochydore DC, Queensland, Australia; ⁴Aligent EU - Envision Pharma Group, Wilmington, United Kingdom; ⁵International Alliance of Patients' Organizations, London, United Kingdom; ⁶Osaka University, Osaka, Japan; ⁷Neurology Angliandema Japan (Registered NPO), Hongo, Japan; ⁸MAE (Registered Charity), Switzerland; ⁹Consumer Liaison Group, National Cancer Research Institute, London, United Kingdom; ¹⁰Research Involvement and Engagement, London, United Kingdom; ¹¹Evidence - Envision Pharma Group, London, United Kingdom; ¹²Consultant in Patient Engagement, Petanini Health, Wilmington, United Kingdom; ¹³Curo - Envision Pharma Group, London, United Kingdom; ¹⁴Engage - Envision Pharma Group, Southport, United States of America



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Abstract

Objective

Although patient involvement in results reporting is being encouraged, relevant evidence must be assessed before developing best practice guidelines. Our objective was to conduct the first systematic literature review on the effects of patient involvement on results reporting.

Research design and methods

Patient experts and publication professionals co-created a PRISMA-P protocol (PROSPERO registration submitted). Using MeSH terms and OVID, we searched (10/09/2017) MEDLINE, EMBASE and Cochrane databases (all languages; 01/01/2015-10/09/2017) and secondary sources. Eligible articles had to report on the effects of having patients author or contribute to clinical research peer-reviewed publications or summaries. The primary outcome was the number of articles investigating patient authorship or contribution to peer-reviewed publications. For included articles, we assessed bias risk (Newcastle-Ottawa Scale).

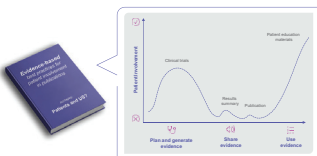
Results

Of the 105 database articles retrieved, 24 duplicates were removed. Title/abstract screening excluded 62 articles. From full-text screening of 99 articles, we could include 2. Both focused on the effects of patient involvement for preparing peer-reviewed publications. Evidence quality for each article was poor/fair (0 randomised controlled trials). Reported benefits of patient involvement included meeting funder requirements, critical and unique contributions, new research ideas, improved reporting, patient empowerment and new skill development (patients and researchers). Reported harms included the need for additional time, training, resources and budget.

Conclusions

This systematic review identified a major evidence gap that must be addressed to guide best practices for patient involvement in results reporting. Patients, sponsors and publication professionals could co-create a research priority list and use emerging evidence to draft interim guidelines for ethical and meaningful involvement of patients in results reporting.

Introduction



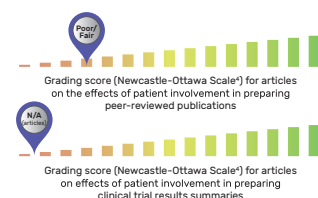
Results

Published evidence on patient involvement in results reporting is limited

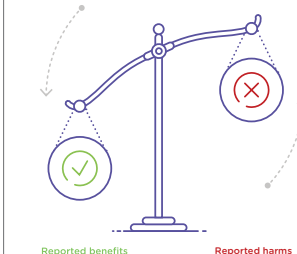
Quantity of evidence is low



Quality of evidence is low



Evidence on benefits and harms of patient involvement in publications



Patient involvement... in the voice of the patient author

“Despite the potentially daunting presence of experienced academics and clinicians, I felt my contribution at project meetings was always valued and clearly highlighted in the minutes. The chair actively ensured that I was included in any discussion and this gave me reassurance.”

Patient involvement... in the voice of the academic author

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

During author calls and informal discussions (before and during the project) we checked that all stakeholders had the time and information needed to make substantial contributions as authors.

The [Patient Authorship Experience Tool](#) that we developed for this project (to be completed by patient and non-patient authors on project completion) specifically asks about capacity and capability for engagement.

6. Transparency in communication and documentation



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

- To facilitate timely delivery and storage, electronic copies of materials for this project (e.g. outlines, drafts, meeting agendas and minutes) were circulated to all authors.
- Authorship meetings were held several times a year and contact with authors was made between meetings, as required.
- A dedicated team was identified to schedule author meetings, prepare agendas, prepare and circulate the minutes.
- A dedicated and secure file directory was established to store all documents related to this project.
- A publication plan was prepared that highlighted timelines and potential conferences and journals to present and publish the results from this project. Patient authors were specifically asked to nominate conferences most relevant to their stakeholder groups where they might want to present the results.

- Whenever possible, ‘generous deadlines’ were provided and key dates were clearly highlighted in meeting minutes and cover emails.
- Given the involvement of professional medical writers in this project and the commitment to plain language principles, information was communicated clearly and concisely.
- While some authors were bilingual, all authors were comfortable communicating in English so all communication was in English.
- In terms of complying with international guidelines for external communications, all authors were aware that peer-reviewed journals require disclosure of author names and any financial or nonfinancial competing interests. We recognise that this requirement may deter some patients from being involved as authors in publications, but full disclosure is typically required.

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

The [Patient Authorship Experience Tool](#) that we developed for this project specifically asks about transparency in communication and documentation.

7. Continuity and sustainability



What did you do to achieve this criterion?

To ensure continuity of the project and relationships from the beginning to the end, a publication plan was developed. This plan identified key milestones for presenting and publishing our research and these ‘external communication points’ helped build a sense of teamwork and a focus on delivering high-quality output.

To help share the learnings from this project, we have committed to presenting and publishing our research.

We have included a specific section in the publication of this project of ‘lessons learned’ that we hope will help other researchers as they conduct further studies on patient involvement in publications.

We are also consulting with our patient authors as to their interest in presenting at conferences, after this project concludes, to help inspire (if not challenge) research funders and researchers to involve patients as authors.

In terms of sustainability, we are also striving to provide practical support and training to help more patients become authors (eg, working with EUPATI to prepare a publications module for their curriculum; providing publication training for patient advocates at medical conferences).

How did you gather feedback on what you have done?

We have asked our patient authors about their interest in presenting at conferences to share their experience and ‘lessons learned’. The response from our patient authors has been positive and they have already presented at a number of meetings (e.g. a Forum in London; publication conferences in Japan and the US). We see this sharing of information as an important component of continuing our relationship with patient authors, from whom we have learned so much. We want others to learn from patient authors as well!

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

The [Patient Authorship Experience Tool](#) that we developed for this project (to be completed by patient and non-patient authors on project completion) specifically asks about transparency in communication and documentation.

Results and outcomes

This project has been successful, in terms of

1. Involving patients as co-researchers and co-authors of this systematic review
2. Using a robust method (systematic review) to collect and analyse evidence to guide best practices for patient involvement in reporting clinical research results. This has allowed us to identify:
 - a. Potential benefits and harms of involving patients as authors
 - b. Evidence-based best practice recommendations for involving patients as authors
3. Identifying challenges for conducting research in this area and proposing solutions

The first stage of the systematic review has been completed. The results have been presented at an international conference focused on ethical and effective publication practices (International Society for Medical Publication Professionals – 14th Annual Meeting; April 30-May 2, National Harbor, Maryland, USA; poster presentation attached). Patients were involved in all stages of preparing the presentation and co-authored the research abstract and presentation.

The second stage of the systematic review is nearing completion. All data have been collected and the target journal has been selected. Manuscript preparation is underway and will be submitted this year (2019).

Evidence based on our experience of this project will be collected via the [Patient Experience Authorship Tool](#). This survey instrument is being completed by patient and non-patient authors. The results will provide useful information on:

- What our team did well to facilitate patient authorship success
- Where we could improve the experience for patient and non-patient authors
- The utility and validity of a practical tool that is based on the PFMD Patient Engagement Quality Guidance tool, but focused on the publication element of medicines development.

Positive impact for specific medicines development phases

Publications are a key element of any successful medicines development program. They are relevant to during the research and discovery phase, the clinical trial phase, the registration and reimbursement phase, and the post-registration phase. By having patients involved in publications, medicines development may:

- Better – patient-authored publications could help identify, prioritise, and publicise unmet needs most relevant to patients. As one of our patient co-authors stressed to our project team, the Discussion section of the manuscript is where research priorities are described (e.g. areas for further research). If patients are not involved in publications, then opportunities to include patient-prioritised research ideas in the peer-reviewed literature (read by key stakeholders) are being lost.
- Faster – patient co-authors may help ensure authors submit manuscripts to the most appropriate target journal. Doing so would avoid delays in manuscript rejections and re-submissions. Non-patient authors can be tempted to submit manuscripts to high-impact journals because being published in these

Patient involvement in publications is still a very new and evolving area and we do not know if patient involvement in publications would have any material effect on the cost of medicines development. This is an area for future research. The additional time and costs of involving patient authors (e.g. patient author training, development of plain language documents/tools) may be offset by better and faster medicines development.

Direct or indirect positive impact for patients

Positive effects for patients from this project include:

- Providing patients with access to robust evidence proving that patients can be involved as authors on peer-reviewed publications. This evidence can be used to counter the argument that ‘patients can’t be involved in publications because they can’t meet authorship criteria’.
- Providing patient authors and non-patient authors with evidence-based best practice recommendations to facilitate successful involvement of patient in publications.
- Confidence that patients can provide unique and valuable contributions to communicating clinical research results.
- Helping patients set and communicate priorities for research and have these priorities embedded in the peer-reviewed literature.
- Development of new skills (e.g. planning and preparing publications, use of innovative communication tools, such as infographics, QR codes, video abstracts etc.)
- Development of new relationships (e.g. trusted and mutually respectful relationships with co-authors)

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

Non-patient (eg, academic) authors can benefit from this project, because they can:

- Gain a better appreciation of the unique and valuable contributions patient authors can make to publications.
- Demonstrate to employers, funders, patients, and the public that they have involved patients as true partners ie, authorship requires regular, substantial, and valuable contributions by all authors, including patient authors (this cannot be tokenistic involvement or the publication could be retracted for misconduct e.g. if patients were ‘guest authors’).
- Target a broader range of journals for their publication – more journals (e.g. The BMJ, Research Involvement and Engagement) are actively looking to publish robust research with patients involved as co-researchers and co-authors.
- Attract greater attention to their research by broader stakeholder groups (e.g. patients, patient advocacy organisations, the public, the media, etc.) by preparing plain language summaries of their publications with their patient co-authors and having patient co-authors raise awareness of their research (e.g. via their networks, which would extend and complement traditional academic networks). We have shown that patients may raise awareness of published research more than healthcare professionals.

Who engages with patient-centered, peer-reviewed publications? Tweeting of JAMA Patient Pages

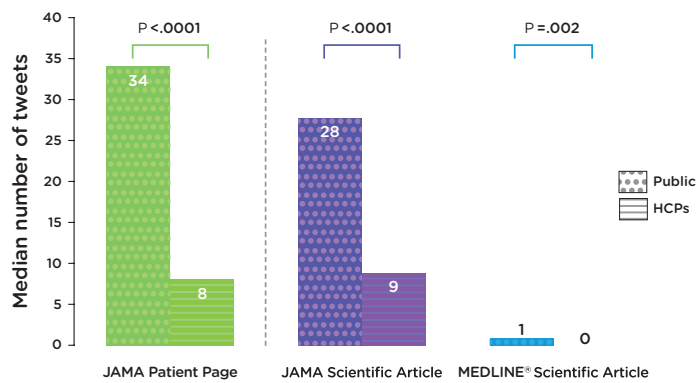
Karen L. Woolley PhD^{a, b, c, d}, Elise Magatova PhD^a, Yukiko Homma MPharm^e,
Emma A. Platt PharmD^f, Paul Lane PhD^g

^a ProScribe Pty Ltd; ^b Adjunct Professor, University of Queensland, Australia; ^c Adjunct Professor, University Sunshine Coast, Australia;
^d Government Director, Sunshine Coast Hospital and Health Service; ^e ProScribe K.K.;
^f Excel Scientific - Envision Pharma Group; ^g Consulting Solutions - Envision Pharma Group

Results

Public outperforms HCPs

The public tweets more than HCPs about JAMA Patient Pages
AND about related scientific articles



Lessons learned

We have learned, directly through experience and indirectly through a systematic review of the literature, that patients can provide unique and valuable insights as authors on peer-reviewed publications. **We now have clear evidence and experience to challenge the belief, held by some, that ‘patients can’t meet authorship criteria’** and can, therefore, be excluded from the publication ecosystem. This is neither true, nor desirable. We and others can now use evidence to challenge this barrier to patient involvement.

We have also learned that practical tools and plain language documents can and should be prepared to facilitate ethical and effective involvement of patients in publications. Developing these tools and documents has taken time, effort, and resources, but they can be re-used and shared to benefit others.

Given the success of this project, we will be following the same processes and using the same tools for future publication work with patient co-authors. Where possible, however, we would look to improve on what we did and the feedback from the [Patient Authorship Experience Tool](#) will be most helpful in this regard. We can use this feedback to help us share ‘lessons learned’ not just with our team, but with the broader community (e.g. patients who are interested in authorship, non-patient authors who are interested in partnering with patients as co-authors).

We have already incorporated a number of the lessons that we have learned through this project into a publications training session for patient advocates (European Hematology Association, Stockholm, June 14 2018). This was the first publications training session for these patient advocates and the insights and lessons shared were very well received (patient advocate tweeted about the value of this publications training (Twitter output below). We have now been invited to prepare the first dedicated publications module for patient advocates being trained through the European Patients Academy (EUPATI).

During this project, we have also learned that publication professionals (e.g. Certified Medical Publication Professionals) are ideally positioned to help smooth the way for involving patients in publications. This was a somewhat fortuitous finding based on the fact that a number of authors happened to be Certified Medical Publication Professionals. To gain this certification, they are tested on their knowledge of ethical and effective publication practices. Publication professionals have to know the ethical guidelines that govern publication planning and preparation. As we found through the systematic review and our experience, patient authors benefit from having a trusted ‘go to’ person on the publication team.



The publication professional can be the ‘go to’ person, supporting and mentoring patient authors as they gain experience in publication planning and preparation. In a promising sign of support for patient authors, when publication professionals were asked should patients become more involved in publications, the answer was a resounding yes (see the ‘hands up’ vote in the pictures below from the 2018 meetings of the

International Society for Medical Publication Professionals; top panel, US meeting; bottom panel, EU meeting). We have already started to share our lessons and learnings with publication professionals and the broader community by starting a hashtag (#GPP4) on Twitter. We hope that the fourth version of the [Good Publication Practice guidelines](#) can include a section on patient involvement in publications.



One challenge that we experienced, but one this is not easily overcome, is having sufficient time to work on this project. As authors, we are completing this project as volunteers, which can require working after hours and on weekends. We recognise that this situation would not be tolerable for all research teams and authors. We welcome further discussion about this issue to help ensure patient authors and their non-patient co-authors can conduct and publish research during working hours (e.g. have dedicated and protected time to work on publications). The voice of the patient in publications is too important to allow it to become muffled or muted by practical issues.



made
with
patients

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

The PFMD Book of Good Practices

2nd edition | 2019





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



Basic Information

PE project description

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.

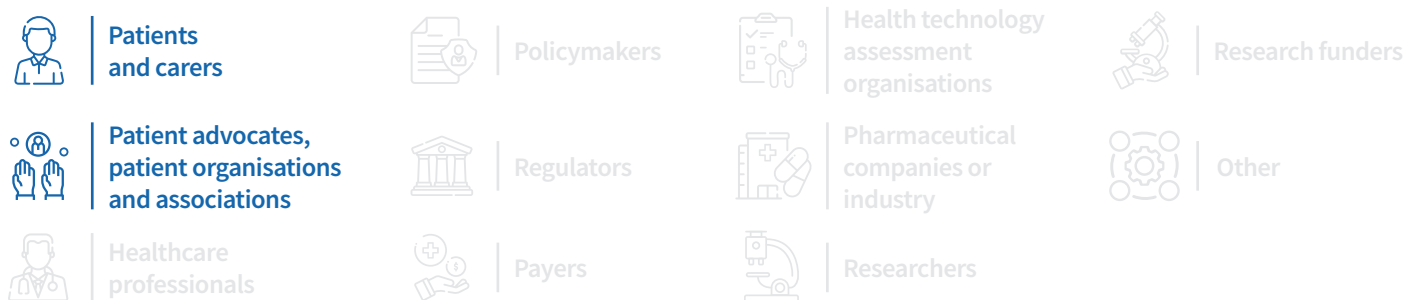
Patient input on the feasibility of the administration of the medication, patient visit schedule, recruitment strategy and the wearability and usability of a Parkinson's Kinetograph (a fitbit-like device) for home monitoring of motor activity helped to shape the final study protocol which received ethical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



Which stakeholders does this PE project involve?





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 its 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.



PATIENT FOCUSED
MEDICINES DEVELOPMENT

rareLife solutions
collaboration + science + technology



A sickle cell community built by the community for the community

Organisation: Rare Life Solutions

The PFMD Book of Good Practices

2nd edition | 2019





A sickle cell community built by the community for the community (www.oneSCDvoice.com)

Organisation:
Rare Life Solutions



Basic Information

PE project description

JoneSCDvoice.com is an online platform specifically designed to empower its members in the sickle cell community to "self-advocate" through learning by example and by having a support group and resources at their fingertips guided by expert advocate mentors. oneSCDvoice.com is created by rareLife solutions on the backbone of its "onevoice community building software" designed to be customized from the start with the input and guidance of individuals on our "Working Groups" who represent perspectives of the multi-stakeholder community. rareLife solutions is powered by knowledgeable and experienced technologists, medical information researchers, and community engagement specialists.

BACKGROUND: Initial investigatory research and interviews with leading healthcare professionals, advocates, caregivers, patients and researchers in the area of sickle cell revealed (1) an advocacy landscape that was fractured, (2) individuals in need of trustworthy disease and lifestyle information and (3) objective resources about participating in clinical trials.

While the sickle cell community has long standing advocacy representation provided by the Sickle Cell Disease Association of American (SCDAA), founded in 1971, the landscape of organizations in the United States has proliferated yielding anywhere from 100-150 different groups, ranging in sophistication, longevity, scope and purpose. Consequently, there is a disparity within the advocacy arena making it difficult for individuals seeking guidance.

Likewise, the scientific literature in the National Library of Medicine demonstrates the large volume of papers on sickle cell with almost 28,000 papers, of which approximately 3,800 note “sickle cell” as its MeSH Major Topic. The numerous papers when combined with the explosion of information aggregated by search engines, only serves to compound the frustration and difficulty for individuals attempting to learn more about sickle cell disease.

In the United States, approximately 97% of those diagnosed with sickle cell disease are African American. As studied and reported in the scientific literature, African Americans have historically had a reluctance to participate in clinical trials. Such trepidation is well-known and attributed in some part to programs such as the “Tuskegee Syphilis Experiment” and the experience of Henrietta Lacks at Johns Hopkins University; each have been memorialized in books and movies popularizing a disregard for humanity in the clinical trial process that occurred before improvements and safety measures created to protect patients.

THE PROJECT: The fractured advocacy landscape, proliferation of information, and the historical trepidation of African American participation in clinical trials, all conspired for the oneSCDvoice.com team to investigate initiatives to provide solutions to these issues to the sickle cell community. Seeking to “build the advocacy power of the individual” to self-advocate, we embarked on a fact-finding and perspective gathering mission to understand multi-stakeholder views of the patients, advocates, caregivers, healthcare professionals, researchers and pharmaceutical manufacturers.

The results of the discovery process, which involved numerous individual and group virtual and in-person meetings, surveys, demonstrations, conversations, and reports, yielded the following solutions:

1. an online platform, free to users was the most efficient form of media to provide the desired solution packages;
2. the platform should offer:
 - a private, registration only community social wall for people to connect and share;
 - a multi-disciplinary library of resources addressing topics related to not only sickle cell disease but also the lifestyle considerations of having a lifelong rare condition;
 - the vetted library should contain resources from various formats and sources to provide accessibility to varying degrees of educational ability and preferred learning channels (video, reading, audio);
 - the vetted library should be a “curated” experience of trustworthy resources already produced and that are available on the internet, to avoid duplication, dilution of resources, and competition with the efforts of advocacy organizations and other educators; and
 - a section dedicated to debunking the historical myths surrounding African-American participation in clinical trials and seek to create diversity in participant populations.
3. The platform should be co-created by a coalition of patients, advocates, caregivers, healthcare professionals, researchers and pharmaceutical manufacturers to provide initial and ongoing guidance to the development and research teams, which in turn, provide regular feedback to the coalition for consideration of implementation of new features or resources.

THE PLAN: The oneSCDvoice.com team set out to build a coalition of “working groups” by assessing the landscape of individuals involved in sickle cell disease from the vantage point of patients, advocates, caregivers, healthcare professionals, researchers and pharmaceutical manufacturers. Once the Working Groups had been built, they were regularly convened by the oneSCDvoice.com team to investigate

such topics as content resources, look and feel of the platform, features and functions, nomenclature, taxonomy structures, search effectiveness and engagement materials including social media digital assets, conference giveaways, logos, and socialization slide decks. The Working Groups participate in the ongoing management of oneSCDvoice.com with ad hoc and regular quarterly meetings where use metrics, new resources and other issues are discussed and action plans decided to keep the platform up-to-date and address community feedback.

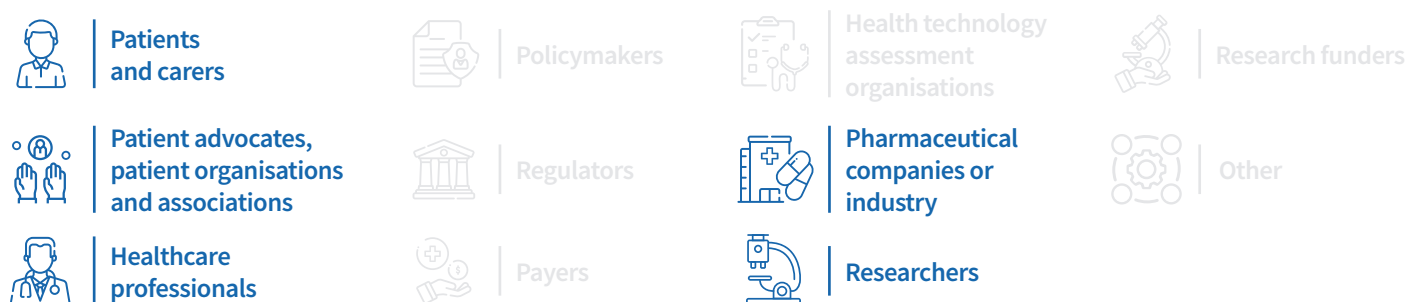
Which phases of research, medicines development, lifecycle or disease management does your PE project cover?



Post-registration/ -launch activities: real-world evidence generation, patient and carer support programmes

Other: oneSCDvoice.com is not tied directly to a lifecycle phase; in fact, its launch and longevity are development phase agnostic; we have only chosen those phases for which this particular community has been created in a point in time.

Which stakeholders does this PE project involve?





The quality of patient engagement

1. Shared purpose



What did you do to achieve this criterion?

The oneSCDvoice.com team consisting of professional developers, patients, advocates, community builders, researchers and engagement professionals, built a complex “Working Group Framework” that provided structure internally for the team to organize, solicit and track Working Group feedback and exposure to the progress of the development of all of the platform components – from user interfaces, the vetted resource library, and social wall management. This framework allowed the oneSCDvoice.com team to produce a simple yet effective communication network to obtain input and feedback on slide decks, surveys, research guidances and engagement plans from the Working Group.

Each member had their own individual assignment based on their experience, talent and interest, in addition to assignments given to all members (for example, features, functions and “look and feel” of the platform). Advocates were focused on engagement tactics while healthcare professionals and researchers were focused on guiding the oneSCDvoice.com resource research team).

Feedback was garnered through multiple avenues – surveys, individual & group meetings. During these meetings the oneSCDvoice.com team would address questions and request guidance from the Working Group member(s). Members of the Working Groups received copies of the analyzed and raw data for their own review, consumption and comment.

Having a pre-built framework for the Working Groups and submitting elements of it to the Working Group for review and comment, proved a powerful step forward in efficiency to move from planning to iteration and then actual deployment.

What is your stated “shared purpose”?

oneSCDvoice.com is specifically designed to empower its members to “self-advocate” through learning by example and by having a peer support group and trustworthy resources at their fingertips guided by expert advocate mentors from the Working Groups.

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?

Confirmation of the value and influence of the Working Groups, both individually and as a whole, is sought and procured through individual conversations, group meetings, survey results and iterative acknowledgement on the development of the platform which is shown to and accessible by the Working Groups during development and after deployment. Every quarter, the oneSCDvoice.com team meets with the Working Groups and prepares reports demonstrating how the feedback provided at earlier meetings has or has not been addressed in the platform.

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

Yes. The oneSCDvoice.com model is built on the foundational dependency of integrating multi-disciplinary leaders (patients, advocates, caregivers, healthcare professionals and researchers) on to the Working Groups to ensure balance and coverage of the topics and expertise of topics relevant to the actual community

members who are learning to self-advocate. The purpose of developing “self-advocacy” capabilities for the community members is paramount, shared and acknowledged by the Working Group members. Such agreement is parsed out during an interview process and memorialized in a written contract with each individual member.

At what time points?

Potential candidates are introduced to the concept platform and invited to apply for the Working Groups. We meet regularly with the Working Groups. At each meeting, the Working Groups are given updates followed by discussions or demonstrations to advance to the next milestone. In addition, at each meeting, the focus for each discussion is: “Is this what the community needs and/or wants?”

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

The oneSCDvoice team readily acknowledges its position and role as a steward of the sickle cell community information and a facilitator of building solutions that reflect the stated needs and wants of the community.

There are two aspects to consider for the oneSCDvoice.com initiative to address respect and accessibility. The first is the Working Group membership. As part of the outreach process, candidates with expertise in a particular aspect of SCD and / or extensive advocacy work, are extended an invitation to apply to the Working Groups. Such individuals are also interviewed for their capacity and desire to be involved in a collaborative coalition.

The other aspect is the community membership who are using oneSCDvoice.com social wall and the trusted resources library. A measure of success for oneSCDvoice.com is to build a “safe and positively-charged” social wall experience where members can participate to seek answers, knowing their most private information is being shared.

The oneSCDvoice.com team, in conjunction with the Working Groups, built a multi-tiered process to protect our members and provide them with a positive experience:

1. appropriate disclosures and participation guidelines:
 - the Code of Conduct provides a simple, easy to read understanding for the membership regarding the guardrails around communicating on the social wall and with other members;
 - the Privacy Policy provides a straightforward explanation of how data collected on the social wall is used and reminds members to guard their privacy to the extent they wish;
 - the “First-Post PopUp” reminds the community member about salient points of the Code of Conduct before they post their first message;
 - FAQs are available for additional easy-to-read guidance surrounding conduct using the platform; and
 - the Terms of Service provides guidance to the membership regarding the use of oneSCDvoice.com;
2. the oneSCDvoice.com team, selects, interviews and hires individuals from the community (typically a patient, caregiver or advocate) and after completing training by the oneSCDvoice team on diplomacy and dispute-resolution methods, who moderates and responds to all posts using text and links from the knowledge library to create a positively-charged and educational environment; and

3. a software system that scans all posts for various “trigger words” (for example, foul or inappropriate language) which violate the Code of Conduct and may require some additional action that ranges from deletion of the post, or revision. In that case the Community Manager contacts the community member directly and explains why the post is not adherent to the Code of Conduct and suggests how it could be modified to be compliant.

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

With respect to our Working Groups, there is a high level of professional decorum and respect. The oneSCDvoice.com team provides access to engagement by tailoring communication channels and media review to the individual’s particular needs and preferences. For example, to ensure the 25 Working Group members could review and be heard on a particular aspect of development, the oneSCDvoice.com team held 6 “webex” virtual meetings over 2 days to provide scheduling convenience to each member (considering existing responsibilities, geographic time zones, and other commitments).

With respect to our community members, the oneSCDvoice.com platform has proven to provide civil online conversation due to (1) the particular disposition of individuals attracted to a “serious” educational platform and (2) the multi-tiered safety program (code of conduct, software monitoring, and human moderation with dispute resolution capabilities). The oneSCDvoice.com platform provides accessibility to anyone with an internet connected device anywhere in the world taking into account: (a) responsive design for a user-friendly mobile experience across desktops, smartphones and tablets, and (b) the trusted resources library is open to anyone without registration to oneSCDvoice.com (only the social wall and some additional features require minimal email registration).

3. Representativeness of stakeholders



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

The Working Groups have experiences and perspectives of varied roles valuable to the sickle cell ecosystem including: patients, advocates, caregivers, healthcare professionals, researchers and pharmaceutical manufacturers.

The oneSCDvoice.com team has a process for assessing the literature and information needs of the community to gain a basic knowledge regarding the issues surrounding the disease and lifestyle. Consequently, a matrix of specific expertise categories is created to ensure that the various aspects of the disease and lifestyle will be addressed by inviting leaders who have expertise in that particular category. Within each role are members with expertise to address particular topics (for example, the Working Group would not have multiple hematologists per se due to their “key opinion leader” reputation, but rather a hematologist for adults, and a pediatric hematologist to help parents understand their children’s needs would both be invited). Additional expertise may include: psychosocial issues, community healthcare, medical specialties and subspecialties, genetic counseling, advocacy, caregiving, social networking, etc.

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

The Working Groups and community members are queried through regular contact, polls or formal surveys to determine their level of satisfaction with the outcomes of their guidance and feedback in the co-creation,

development, deployment and ongoing iteration of oneSCDvoice.com. The results of those polls and surveys are digested and analyzed by the oneSCDvoice.com team to share back with the Working Groups for additional feedback.

The community members are also surveyed using “Customer Satisfaction” surveys to understand various specific issues (including content topics and formats). The oneSCDvoice.com also surveys the community membership to calculate the Net Promoter Score (NPS) of oneSCDvoice.com. NPS is a management tool that can be used to gauge the loyalty of a brand’s customer relationships. Given the NPS range of -100 to +100, a “positive” score or NPS above 0 is considered “good”, +50 is “Excellent,” and above 70 is considered “world class”. Based on global NPS standards, any score above 0 would be considered “good.”

oneSCDvoice.com achieved NPS of +50 (excellent). By comparison, Amazon has a +61 NPS and facebook has a NPS -21.

4. Roles and responsibilities



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

Written contracts provide the formal relationship memorializing the roles and responsibilities of each Working Group member, including a schedule of meetings, time commitments, and a description of the work to be performed.

Each member of the Working Group is given specific written assignments to provide feedback and review materials including:

1. surveys (both taking individually and reviewing the collective Working Group results);
2. links to content for potential inclusion in the resource library to match with pre-established quality criteria;
3. guidance strategies and tactics for research and engagement of the community-at-large; and
4. static mockups and staging versions of oneSCDvoice.com

Virtual ad hoc or structured meetings are held weekly and monthly, respectively, before the launch of the platform and post-launch virtual meetings are held quarterly.

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

As part of the contractual arrangement, in the event a Working Group member is unable to complete an assignment, a oneSCDvoice.com team member is assigned to connect with that Working Group member to provide any assistance or clarity that might enable completion of the assignment.

At what frequency have you checked this in?

The oneSCDvoice pre-launch process requires frequent contact, including daily, weekly or monthly depending upon the situations being presented.

5. Capacity and capability for engagement



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

Members of the Working Groups were objectively evaluated using criteria that allowed the oneSCDvoice.com team to select individual candidates that were “fit for purpose” based on the needs and wants expressed by the community. Additional evaluation was performed regarding the candidate’s capabilities and interests, and the candidate’s feedback of the oneSCDvoice.com team’s expectations.

The role of community management is an emerging profession. Upon completion of the training provided by the oneSCDvoice team, the Working Group members understand how to manage a social wall to resolve disputes, enforce adherence to the community Code of Conduct, and create a safe, positive experience which engenders membership satisfaction and trust.

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

During our ad hoc conversations and as part of our regular meetings, we provide the opportunity for each Working Group member to comment on the delivery of their assignments and engage in live discourse with other Working Group members.

6. Transparency in communication and documentation



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

The oneSCDvoice.com team used its Working Group Framework to provide a schedule of milestones to the Working Group members. The schedule provided details of the communications the Working Groups would be receiving as part of any pre-meeting preparation and any post-meeting follow up.

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

The Working Groups are contracted using simple and easy to understand language setting out their roles, responsibilities, deliverables and a schedule for completion. The oneSCDvoice.com team tracked each Working Group member against the contract obligations to understand compliance and implementation.

7. Continuity and sustainability



What did you do to achieve this criterion?

At the outset, the oneSCDvoice.com team provided long term contracts to each Working Group member using simple language ensuring that each individual understood the commitment was not just to build an online community and launch it, but to nourish and grow it over time. The commitments were clearly delineated during both the pre-launch phase and the post launch maintenance phase.



While the Working Group Framework selected virtual “webex” meetings and teleconferences over face to face meetings (mostly to respect time commitments of Working Group members), the oneSCDvoice.com team traveled to numerous patient conferences to meet face to face with Working Group members also in attendance. These face to face meetings provided positive relationship building opportunities that are reflected in expanded activities.

As relationships were building between the oneSCDvoice.com team and the Working Group Members, additional projects with members outside the scope of the original oneSCDvoice project began to blossom. Understanding more about the missions of the individual patients and advocates deepened the learning for the oneSCDvoice.com team to become aware of many new ways to engage with the community (for instance, awareness tours, educational programming, conferences, social media campaigns).

How did you gather feedback on what you have done?

The Working Group-oneSCDvoice.com-Community Member feedback loops consist of formal surveys, polls, group teleconferences / webex meetings and informal ad hoc discussions.

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

The initial discussions between the oneSCDvoice.com team and the potential candidate for a position on the Working Groups were very clear about the roles and responsibilities of becoming a member. The details were set forth in writing for consideration by each invitee prior to such individual applying for membership on the Working Group.

Results and outcomes

The oneSCDvoice.com team set numerous key performance indicators (KPIs) to measure the success of the oneSCDvoice.com platform. The development of the KPIs took into consideration the innovative nature of the project and the lack of appropriate surrogate benchmarks which may normally provide relative assessment measures.

Objective KPIs evaluated that were collected by the interaction of people with the oneSCDvoice.com platform:

1. registered members – number of members, frequency of over specific periods
2. engagement with social wall – number of posts, frequency of posting, length of posts
3. polls & surveys – number of polls completed
4. engagement with trusted resources – click data regarding links, including time on page
5. visitor traffic for unregistered members – engagement with trusted resources
6. SEO page ranking changes over time
7. Net Promoter Score – currently +50 (excellent)

Additional feedback was collected using “Membership Surveys” and polls administered to registered members.

Positive impact for specific medicines development phases

The use metrics of the knowledge library has identified unmet educational needs of the community including deeper education about clinical trials and overcoming the barriers to diversity in clinical trial participation. The polls and survey results demonstrate numerous insights about patient preferences and perspectives, however, a valued insight is the high percentage of registered members answering multiple polls. Attraction opportunities using social media has identified topics of interest to the community that are compelling, and conversely, topics that are of little interest.

Direct or indirect positive impact for patients

Patients have overwhelmingly communicated that oneSCDvoice.com provides a sickle cell “home on the internet” for them which they will recommend to others (as evidenced by an excellent Net Promoter Score of +50). The patient segment of the membership scores the quality of and access to vetted resources as 9.3 (out of 10) and that having such a library has improved their knowledge of sickle cell issues “significantly” (76%).

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

The non-patient members of the Working Groups and the registered community members have expressed a “significant” (53%) or “slight” (19%) increase in their understanding of the patient experience, preferences and needs from prior to their interaction with the oneSCDvoice.com platform. Only 5% responded the platform had a “negative impact” on their knowledge of sickle cell disease and its associated lifestyle issues, while the remaining 23% responded “no change” with respect to the same.



Lessons learned

The oneSCDvoice.com team has culled the following lessons:

1. active listening to all stakeholders provides a platform for open sharing;
2. creating a coalition of multi-disciplinary stakeholders on par with each other exponentially increases ideation and creativity;
3. feedback loops to the at-large community provides not only insights but invaluable loyalty by the community members;
4. general email solicitations and awareness campaigns generally do not resonate with the sickle cell community;
5. consultation with community leadership from the outset in creating oneSCDvoice.com allowed avoidance of costly missteps and provided a patient-focused service that truly resonates with the end user as an authentic home on the internet.



made
with
patients

HeadUp Collar: Co-creation of a new cervical orthosis for patients with neck weakness

Organisation: Sheffield Biomedical
Research Centre

The PFMD
Book of Good Practices

2nd edition | 2019





HeadUp Collar: Co-creation of a new cervical orthosis for patients with neck weakness

Organisation: Sheffield
Biomedical Research Centre



Basic Information

PE project description

Many people with Motor Neurone Disease (MND) develop weak neck muscles, leading to pain, restricted movement, and problems with swallowing, breathing and communication. Available neck collars were reported to be of limited use for people with MND and frequently rejected by patients. This issue was identified directly by patient representatives in the NIHR DeNDRoN clinical research network and confirmed by clinicians in the same network. The project leader, as a member of the DeNDRoN network, consultant neurologist and academic clinical researcher began to look more closely into the lack of suitable neck support for MND patients. Initial work centered on finding out exactly what the patient identified problems with existing neck supports were and their requirements for an ideal neck support through patient engagement.

A cross-organisation collaboration of NIHR Devices for Dignity Med Tech Co-operative (D4D), Sheffield Hallam University (SHU) and The University of Sheffield (incorporating the Sheffield Institute for Translational Neuroscience) secured funding from NIHR Invention for Innovation (i4i) to enable focus groups with patients, their families and with a multidisciplinary group of health professionals and design engineers in order to develop and explore new prototypes.

Patients helped to input into multidisciplinary design workshops to reach new prototype design concepts. A local research advisory group, the Sheffield Motor Neurone Disorders Research Advisory Group (SMNDRAG) remained engaged with the project over a long-term period from 2010 -2015 and a member of SMNDRAG was a co-applicant on the NIHR i4i grant to develop and test prototype design.

Patient views from expert user group workshops fed in to an iterative co-design process with technical experts to arrive at a final collar design that was patented. An extension of NIHR i4i grant funding in conjunction with the Motor Neurone Disease Association charity was secured and used to manufacture 100 collars of the new design (the 100 collars project) to test for support, durability, freedom of movement and wearability with patients with neck weakness from MND and other neurological causes at 10 sites across the UK. This HeadUp study received Trial and Project Management from D4D, who also established an Expert Patient Group in order to monitor trial participation experience and also to test out iterative collar design aspects from data obtained during the trial.

The panel of patients in the SMNDRAG helped to review, evaluate and improve a data collection tool to evaluate existing cervical orthoses for comfort and aesthetics during the development of the HeadUp collar. The tool specified the location and perceived scale of discomfort as well as overall perception of wearing cervical orthoses. For the latter assessment 10 statements were used based on the experiences of people living with MND when wearing orthoses. All statements were positively phrased e.g. 'this device caused no restriction to my breathing'. SMNDRAG provided feedback on consent forms and patient information sheets to help run the study.

This multidisciplinary collaborative project involved patients, researchers, clinicians, academics and designers across the different organizations. Patients were involved at all stages of the research cycle – from identification of the need for a new fit for purpose orthotic device, involvement as co-applicants for grant funding requests, providing input into the design process and developing protocols for user testing of prototype designs, through to disseminating the research results by featuring in press coverage upon market release of the product. Patients and carers living with MND who are members of the SMNDRAG at the core of involvement activities have been able to benefit directly from the development of the HeadUp neck collar (<http://www.youtube.com/watch?v=Db7yrIDNszs>).

The quarterly meeting group continues to support a broad range of MND research through regular interaction with researchers. This and the HeadUp Study Expert Patient Group are, providing an exemplar model for patient engagement in different areas of neurology across NIHR Sheffield Biomedical Research Centre (BRC) and beyond. The SMNDRAG established documentation provides templates for new research advisory groups that have been set up within the NIHR Sheffield BRC. The HeadUp collar (previously known as the Sheffield Support Snood) is now available for patients with neck weakness through 25 NHS Trusts across the UK as of May 2018 and to purchase commercially through TalarMade worldwide.



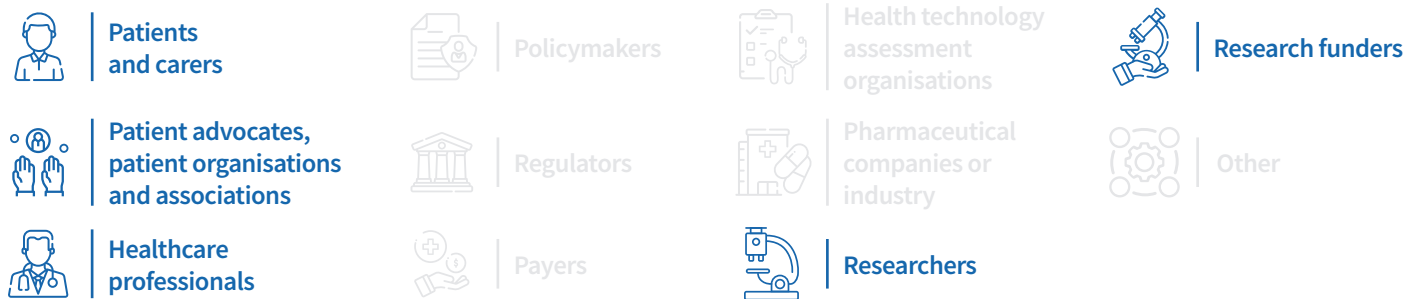
Which phases of research, medicines development, lifecycle or disease area does your PE project cover?



Post-registration/ -launch activities: marketing insights

Other: Research advisory input into observational clinical studies

Which stakeholders does this initiative involve?





The quality of patient engagement

1. Shared purpose



What did you do to achieve this criterion?

- Held focus groups to define patient needs for a neck collar
- Developing project discussed at 18 meetings over 5 years with the Sheffield MND Research Advisory Group (SMNDRAG) panel and the HeadUp Expert Patient Study Group of patients
- Co-developed questionnaire to systematically record limitations of existing cervical orthoses
- Patient expert attendance at further design workshops and ongoing SMNDRAG panel involvement in iterative design process and protocol development for user testing of prototype designs

What is your stated “shared purpose”?

The key shared objective was to develop a cervical orthosis to meet previously defined unmet needs of patients with neck weakness due to neurological disease.

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?

Patients defined the limitations of existing neck collars and their requirements for an ideal collar. This included aspects of aesthetic appearance for a discrete orthosis for daily use not previously considered by the clinical and engineering collaborating researchers.

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

The patient and carer members of SMNDRAG were tenacious to see the project through and expressed that it should not be given up on even when it had been rejected for funding twice and volunteered to attend workshops to establish prototype designs anyway.

As the project progressed the group requested that the prototype be brought in to the next meeting to facilitate their continued involvement.

At what time points?

The HeadUp project was reviewed by SMNDRAG research advisory panel as a standing agenda item at every meeting, 4 times a year between 2010 and 2015.

Dissemination of the project outcomes has been shared by and with members of the all patient groups.

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

- The meeting time and place, frequency of meetings and defined dates for email contact between



meetings are all agreed by the SMNDRAG panel and the HeadUp Study Expert Patient Group.

- SMNDRAG provided patient consultation on developing wording for a tool to user test neck collars and study information for patient audiences.
- This formed part of SMNDRAG's input into recruitment strategy for the 100 collars project
- The project adhered to INVOLVE guidance on payment for travel expenses to design workshops and co-applicant involvement on project
- Respect for participants during the collar design and trial phases is evidenced by the strength of impact that patient feedback had on specifying the design requirements for the collar, and the selection by the PPIE groups of a preferred design for the clinical trial.
- Careful discussions were held with patients taking part in the dissemination and publicity campaigns in order to respect what information was made public and which aspects of dissemination they wished to participate in – and to what degree.

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

The SMNDRAG panel of patients and carers are invited to report annually via a questionnaire on their experience of involvement (engagement) in research. Regarding the specific HeadUp project, a member of this group recorded a testimony about her experience of the workshops during the project and noted <https://youtu.be/ZrtM2quaelA>

D4D received very complimentary feedback from panel members (in the form of social media acknowledgement and personal written correspondence) regarding the element of mutual respect and participation in the project.

3. Representativeness of stakeholders



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

The SMNDRAG group set out agreed terms of reference, person specifications and application for membership includes checking for time commitment, email and computer skills, the ability to read and comment on potentially complex documents. This group was at the core of patient engagement for the HeadUp project.

To widen the representation of stakeholders, the MNDA charity helped to advertise the HeadUp project through their channels and featured a focus group meeting at their AGM in 2011 to giving people outside the limited membership of SMNDRAG a voice in the development of the project. The opportunity to share their thoughts on the current neck collars available and whether another design was needed was given at this meeting including the chance to make suggestions on how to make a new collar more suitable.

In 2012 the MNDA helped again to identify people to take part in a working group to be involved in the design process. Subsequently patient attendance at design workshops was kept accessible through providing travel costs, offering reimbursements and expenses.

During the clinical evaluation stage of the project, the project team engaged with clinicians and patient groups across 10 sites in the UK and Ireland in order to reach a diverse population of stakeholders. D4D also linked in with Sheffield Teaching Hospitals NHS FT (STH) Consultant Nursing Staff in order to offer trial

participation to patients with neck weakness resulting from Late Stage Effects on cancer treatment. This was welcomed by these patients as they have had difficulty accessing ongoing multidisciplinary support in the community for head drop problems.

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

A strong case was put together for funding after the MNDA AGM.

The HeadUp Study Expert Patient Group provided ideas for further iterative neck collar improvements. These were shared with the whole project team and novel prototypes (for example incorporating flesh coloured collars and supports and a trial of a side fastening collar) were developed and evaluated by the HeadUp Expert Patient Group members.

Their feedback helped ensure the project not only delivered on the shared outcomes identified at project outset, but also provided very valuable ongoing information for further developments for the collar and offers from the group of help with dissemination and publicity.

4. Roles and responsibilities



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

SMNDRAG meetings are open discussion forums with a friendly mix of at least 50% patient and carer or past carer, members together with clinical and scientific staff members. The group has been chaired well by past carers on voluntary basis who ensure that all voices are heard at meetings. The meetings are minuted by an administrative member of staff and all members have the chance to modify or approve the minutes at the next meeting.

The HeadUp study expert patient group was held in a slightly more informal capacity, without the need for a designated chair. Communications and practical arrangements for meetings (via a range of contact methods, as convenient to individual group members (e.g. telephone, email, or text messages). Discussions were regularly held regarding different roles and/or responsibilities so that individuals could select these at all stages according to their health, other time commitments and individual preferences.

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

SMNDRAG agreed terms of reference sets out the role and expectations of the group. A membership application form was co-developed to check new members would be capable of fulfilling the role.

At what frequency have you checked this in?

Issues can be raised freely on a quarterly basis at the face to face meeting or intervening dates for email contact.



5. Capacity and capability for engagement



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

In conjunction with the Clinical Research and Innovation Office at Sheffield Teaching Hospitals NHS Foundation Trust, members of SMNDRAG are invited to training days and educational activities such as International Clinical Trials Day and a wide range of public outreach event around Sheffield Neurosciences. A free online training course (European Patient Ambassador Programme) was also advertised through the SMNDRAG group.

SMNDRAG members can access some University of Sheffield library services with day passes organised through the group.

Reimbursement for travel expenses was offered to SMNDRAG and HeadUp Study Expert Patient Group members. Members were aware that they could opt out of the project at any stage if they wished to do so.

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

Through open discussion at SMNDRAG meetings and feedback forms of training and public outreach events offered. A member of SMNDRAG completed the EPAP course in 2013 and recommended it to other members of the group. A further member subsequently applied for a place on the training course.

The PPIE focus groups were given information about the project and care was taken to ensure that they felt that they could speak freely on both positive and negative aspects of different neck collar prototype designs. They fed back that they felt their input had a very meaningful and powerful impact since they could see how their feedback had been incorporated into iterative prototype models, and they had selected their overall preferred design to be used in the clinical evaluation.

6. Transparency in communication and documentation



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

The Sheffield MND Research Advisory Group decided democratically the pattern for email communication between face to face quarterly spaced meetings. The meetings are minuted and feedback and updates are recorded, circulated and approved by the membership at the next group meeting. HeadUp remained a standing item on the meeting agenda over the course of a 5-year period in which the HeadUp project was discussed at 18 out of 20 meetings. The group were kept updated on the study they helped to develop and secure funding for once it was underway with recruitment and other information. Study information was kept updated online and the link was circulated to the group.

Ongoing communication with the HeadUp Study Expert Patient Group was maintained using whichever medium preferred by each participant (telephone, email, text and/or social media). This communication was two-way (i.e. not always initiated by the project team) and is ongoing after completion of the project, at the request of the group members.

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

At the face to face meetings the minutes of the last meeting were checked for approval as a standing agenda



item. The group Chair checked that all members had received the link for and were able to access online information. The minutes of the meetings are stored on a secured drive for the Faculty of Medicine, Dentistry and Health at the University of Sheffield.

It is a testament to good documentation of the meetings that they were sufficient that an appropriate third party (the Patient and Public Involvement and Engagement Lead for Sheffield BRC) was able to recount the long-term involvement in the project retrospectively for assessment against PFMD quality guidance criteria.

In preparation for the HeadUp Study, the Protocol and Patient Information documents were shared for feedback. During the clinical trial communication with the Expert Patient Group was maintained via newsletters, telephone, social media and email.

7. Continuity and sustainability



What did you do to achieve this criterion?

Continued PE was enabled by the collectively funded SMNDRAG of patient and carer members. A stable membership (that is open to new members) meet regularly every quarter to discuss a variety of projects with researchers, clinicians and academics. The HeadUp project remained a standing item on the agenda of these meetings for 5 years from June 2010 until December 2015 and the group was kept updated with all aspects of the developing design process and the deliverables of clinical assessment of the collar, including recruitment rates for participation in user testing. Members of the group were involved again in disseminating the research and featured in a short film (available on youtube) and press releases.

- <https://youtu.be/ZrtM2quaelA>
- <https://vimeo.com/272414469>
- <https://www.youtube.com/watch?v=Z6tmADMDSgM>

Forward looking budgeting for the group to be sustained beyond the duration of the HeadUp project was applied for and this is providing a model for funding newly set up groups in other neurological disease areas.

All organisations collaborating in this project feel strongly that the collaboration between academics, clinicians and patients has been the key factor contributing to the success of the project and that it has been a powerful experience for all involved to experience the power of Patient and Public Involvement and Engagement when it is fully optimised. Learning from this experience has been an additional impact of the project and is being shared via conference presentations, publications and social media in order to share this learning beyond this particular project.

How did you gather feedback on what you have done?

The SMNDRAG group continues to minute their meetings and operate as normally.

The HeadUp Study Expert Patient Group have volunteered their feedback (via personal written communication, and via social media). One group member also made his own YouTube video to document his experience: <https://www.youtube.com/watch?v=66Lgyv1r6E0>

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 direct consultation with the members of SMNDRAG and HeadUp Study Expert Patient Groups.

Results and outcomes

Expert user panels fed into the development of a new design for a neck collar and reviewed funding applications and protocols for user testing to the eventual success of the objective to create a new product. The panel of patients in the Sheffield MND Research Advisory Group helped to review, evaluate and improve a data collection tool to evaluate existing cervical orthoses for comfort and aesthetics during the development of the HeadUp collar. The tool specified the location and perceived scale of discomfort as well as overall perception of wearing cervical orthoses. For the latter assessment, 10 statements were used based on the experiences of people living with MND when wearing orthoses. All statements were positively phrased e.g. 'this device caused no restriction to my breathing'. Details published in 'A comfort assessment of existing cervical orthoses Ergonomics 61(2):329-338 01 Feb 2018.

Positive impact for specific medicines development phases

For example,

- identifying unmet medical needs,
- accurately prioritised research agenda,
- improved study design (for example, fewer protocol amendments to procedure),
- financial impact due to faster set-up and fewer amendments,
- possible decreased timing to registration,
- patient-driven solutions,
- increased patient adherence to medication and treatment,
- extension of a medicine or treatment to new patient groups or new country/ regions.

All of the above! The results of the HeadUp Study 100 collars project showed that 80% of patients preferred the HeadUp collar - a patient prioritized and co-designed solution to patient identified unmet need – over existing designs.

Continued patient involvement in press-related activities following the launch of the product has helped to raise awareness, drive and drive demand for the collar with a spike in healthcare providers and private customers making enquiries for the product after these activities, extending the reach of the new device.

Direct or indirect positive impact for patients

The impact is the international availability of a novel cervical orthosis that is adaptable to a patient's needs using adjustable removable supports that can be changed according to requirement during different daily activities and that is comfortable and wearable over time in the case of chronic and progressive diseases. This has led to the direct impact of improved quality of life for patients using the HeadUp collar referred to by a patient and carer couple featured in communications about the new collar (<https://www.youtube.com/watch?v=Db7yrIDNszs>).

Indirectly, the promotion and widespread recognition of the impact true partnership with patients has had through the HeadUp project is helping to endorse and inform further patient engagement activities around



the NIHR Sheffield BRC and of the other project partners, thus maximizing the chances for positive patient influence on many areas of research.

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

- New IP generation through patient driven research prioritisation and better investments in research and development.
- A better understanding of patients' requirements for cervical orthoses made for compelling cases to fund development of a new product that could be competitive on the over-the-counter market.



Lessons learned

The format of the regularly meeting Research Advisory Group of patients and carers facilitated long-term co-development of research and succeeded in taking a patient priority from an idea to a clinically adopted and commercially available product. **The value of patient engagement from early stages of research can sometimes be overlooked when overstretched investigators need to arrange activities ad hoc or find budget for any service charges incurred. A relatively small amount of public or collective funding to run such a group that a variety of researchers can access with ease can have a large impact. In this exemplar case the total cost to run such a group was no more than £1500 per year including the salary costs for administrative time. The total global market potential for cervical orthoses is \$27M.** Applied over many different research areas the value of patient engagement as an investment should not be underestimated.

This project illustrated how PPIE can and should be a central theme throughout each stage of health research and innovation. This helps protect against risks of tokenistic collaboration, instead achieving partnership working at a genuinely impactful level – thus optimising opportunities for the final output to be successful and fit for purpose.



PATIENT FOCUSED
MEDICINES DEVELOPMENT

CAPA

Canadian Arthritis
Patient Alliance

made
with
patients

Pregnancy and Parenting with Arthritis

Organisation: Canadian Arthritis
Patients Alliance

The PFMD Book of Good Practices

2nd edition | 2019





Pregnancy and Parenting with Arthritis

Organisation: Canadian
Arthritis Patients Alliance



Basic Information

PE project description

Background

Arthritis affects individuals in many aspects of their life including decisions regarding pregnancy and in carrying out their role as a parent. Many decisions need to be taken, including the risks of taking or not taking medication while trying to conceive and during pregnancy, and the ability to carry out their role as a parent.

The Canadian Arthritis Patient Alliance (CAPA) is a grass-roots, patient driven organization managed by people living with various forms of the disease. CAPA has no full time employees, is run solely by volunteers who live with arthritis and supported by consultants providing administrative and accounting support, and is a completely virtual organization. Further, its funding is very limited and most of its communications vehicles are based online.

Based on the gaps identified through our lived experience, CAPA launched a project to identify patient information needs as it relates to pregnancy and parenting and develop educational resources to guide patients. The first phase of the project was the creation and dissemination of a survey (launched in September 2015) and the second phase focused on the creation of resources informed through the results of the survey.

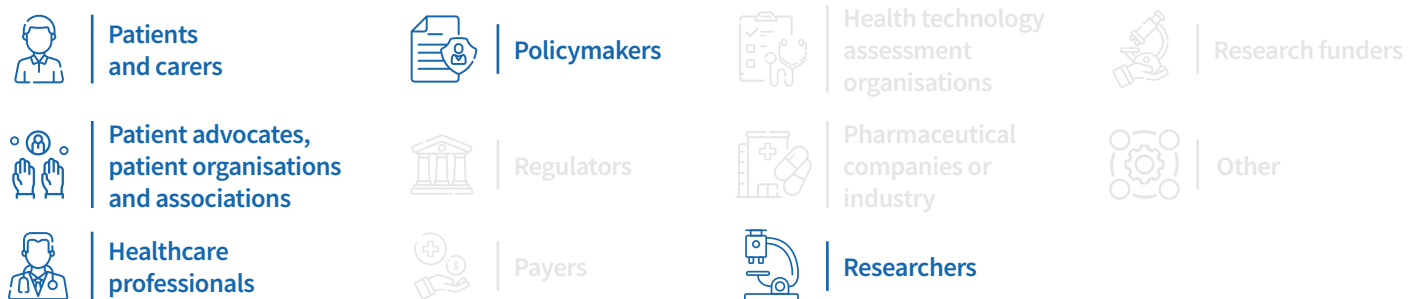


Which phases of research, medicines development, lifecycle or disease management does your PE project cover?



Post-registration/ -launch activities: patient education, patient and carer support programmes, disease management.

Which stakeholders does this PE project involve?



The quality of patient engagement

1. Shared purpose



What did you do to achieve this criterion?

A project proposal was drafted and shared with the CAPA Steering Committee and the researchers / health care professionals engaged in the pregnancy and parenting project. Support was secured early in the project and check-ins with stakeholders occurred throughout the project, such as communicating the survey results and preparing the outline for the educational resources. In addition, the project was regularly communicated to the CAPA network, patient advocates, other patient groups and interested researchers and health care professionals who expressed interest in the project. Interest in the project has grown to include European and U.S. based patient groups, allied health care professionals in Canada and maternal health researchers across Canada.

What is your stated “shared purpose”?

The shared purpose broadly speaking is to improve care and meet the needs of women considering pregnancy and parenting and who live with arthritis, by providing information from individuals who have been through these experiences themselves.

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?

Regular communication with stakeholders occurred throughout the project including responding to comments on the survey and resource design to clarify intent and confirm which suggestions were incorporated. Updates were also provided to stakeholders on the overall status of the project and outreach activities such as poster and oral presentations.

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

The shared purpose was the subject of regular updates at monthly CAPA conference calls, and through e-mail interactions with stakeholders including health care professionals and researchers. CAPA is a virtual organization so many interactions were undertaken by email or phone. Where possible, in-person communications were undertaken (for example, if CAPA members were at conferences where researchers and health care professionals also were).

At what time points?

Communications happened at all points throughout the project where required. This included major milestones, such as survey development, communications approach, scoping the initial content of the resource, as well as resource development.



2. Respect and accessibility

How have you addressed respect and accessibility in this project?

The project proposal was developed in consultation with the CAPA Steering Committee members and shared with the health care professionals / researchers closely involved in the project. Stakeholder engagement was respectful and inclusive and regular updates were provided throughout the project.

As a patient-driven project, many of these considerations did not apply however we do take great care in ensuring the survey and communications relating to the project were written in plain language. The internal review function, a key part of the day to day operations of CAPA, was employed throughout the project to ensure the survey and educational resources were written in lay language. Communication approaches used throughout the project also leveraged our existing networks and partnerships within the arthritis community. For example, guest blog posts were written with well-known bloggers such as The Seated View and Mamas Facing Forward, an on-line support community for parents living with arthritis and other chronic diseases.

The health care professionals that conducted a medical review of the resources were offered an honorarium with respect to their effort and expertise however payment was declined.

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

Given the limited resources within CAPA, a formal evaluation has not taken place. However, we continue to be approached for new partnerships and collaborations from stakeholders and view this as an indicator of success.

3. Representativeness of stakeholders



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

To ensure representativeness from a broad range of people with arthritis, a survey was developed to identify the information needs of the broader community. There were 150 survey respondents and they were predominantly female and represented different subtypes of the disease, i.e. 51% live with Rheumatoid Arthritis followed by 20% with Ankylosing Spondylitis. Respondents represented the geographic distribution of the Canadian population and represented both rural (24%) and urban (72%) residents. Survey respondents have lived with the disease for a variety of time but more than half have lived with arthritis from 0-14 years.

Survey design incorporated the viewpoints of the CAPA Steering Committee who live with various forms of the disease (e.g. Ankylosing Spondylitis, Juvenile Arthritis, Rheumatoid Arthritis, Vasculitis). Input was also sought from other people with arthritis who are not involved in patient advocacy to ensure representation was sought from people with less knowledge of health care systems.

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

We ensured that representation supported project outcomes by seeking feedback on survey design, communication approaches and in the development of the educational resources.

4. Roles and responsibilities



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

As previously noted, a project proposal was developed by one member of the CAPA Steering Committee (the project lead) and shared with the other CAPA Steering Committee members, health care professionals and patient advocates. The project lead had some in-person meetings and/or phone calls with the researchers / health care professionals involved in the project to ensure understanding of roles and responsibilities, timelines, as well as the possibility of honorarium / compensation for their contributions.

Regular check-ins were held with the CAPA Steering Committee members, health care professionals / researchers and patient advocates interested in the project. It was made clear by the project lead that feedback, and questions were welcome at any time during the project. Although expectations were not set out in writing, an open, collaborative, and respectful environment was developed, and continues with many of these stakeholders today.

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

Through ongoing discussions by phone, email, or in-person, the project lead established a collaborative environment with stakeholders and indicated that feedback and questions were welcome on an ongoing basis.

At what frequency have you checked this in?

The project lead checked in at least once every 3 months, depending on the project's activities. It is important to note that CAPA is a volunteer-driven organization and projects are implemented in line with the Steering Committee member's time and other life responsibilities (e.g. paid employment, family commitments, health status).

5. Capacity and capability for engagement



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

Since it is a patient-driven project, we are flexible in our approaches to engaging patients. We live with health conditions and are very adaptable to the episodic nature of the disease. Timelines and calls are typically scheduled at times when most would be able to participate and are always negotiable. Strict timelines, which can discourage patient participation, are not adhered to and is possible given the longer-term view of the project (2015 to present).

Since the initial launch of this project, there has been a considerable increase in the amount of patient capacity and capability relating to pregnancy and parenting with arthritis. It created a network of like-minded patients, researchers, health care professionals, patient groups, and rheumatologists with a keen interest in maternal health for people living with rheumatic diseases.

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

Ongoing communication with stakeholders was central to the implementation of this project. Because of regular check-ins with stakeholders, there were no issues or concerns with the ability for all participants to contribute effectively and meaningfully. No concerns were expressed by stakeholders at any time during the project.

6. Transparency in communication and documentation



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

CAPA is an organization that operates in the open by default. We established a [project page](#) on our website and post all relevant project materials for members and other stakeholders. The project page contains information on poster presentations, the report summarizing the survey results, oral presentations, as well as advocacy activities. In addition, a [resource page](#) was created along with important information highlighting collaborations which can increase patient knowledge relating to pregnancy and parenting with arthritis. Project updates were communicated to the CAPA memberships via the [organization's newsletter](#) and thorough collaboration with arthritis bloggers and other patient groups.

In terms of communications to stakeholders, regular updates were provided to CAPA Steering Committee members as well as other stakeholders throughout the project. A number of posters and oral presentations were made at scientific meetings and conferences, such as the Canadian Rheumatology Association annual scientific meetings (2015 and 2016), European League Against Rheumatism (2017), International Conference on Reproduction, Fertility and Rheumatic Disease meeting (2016), meeting with Health Canada (2017) and a presentation is planned for the Arthritis Health Professionals Association 2018 annual meeting. The project was also accepted for an oral presentation at the 2018 International Conference on Reproduction, Fertility and Rheumatic Disease meeting however a CAPA representative was unable to attend.

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

N/A

7. Continuity and sustainability



What did you do to achieve this criterion?

This project has been undertaken over many years. Project stakeholders have come in and out of the project, while it's been driven by one project lead who is a volunteer. Given that the project is lead by a volunteer-based organization that is very open to feedback, input, and collaboration with others, the project has evolved over time as well.

How did you gather feedback on what you have done?

N/A

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

Support for the project was initially secured to allow its execution over time. Stakeholders have been brought in as necessary (e.g. to respond to or share the survey, to review materials for scientific and medical accuracy, etc.). These engagements and their terms are made obvious to the stakeholders as they engage.

Results and outcomes

Based on the lived experience of CAPA Steering Committee members, CAPA developed a resource for people living with arthritis regarding pregnancy and parenting. CAPA aims to raise the profile of this issue and help people living with arthritis engage in dialogue with their healthcare providers during these critical life events. It is expected that the use of the resource will enable shared decision-making, improve communication with healthcare professionals, and reduce stress for people living with arthritis and their families.

Various methods were used to solicit patient input throughout the project including survey design, survey analysis, and in the development of educational resources. Wide-ranging feedback was solicited through a survey completed by 150 respondents, the majority of whom live with rheumatic disease.

Positive impact for specific medicines development phases

n/a

Direct or indirect positive impact for patients

It is expected that the use of the resource will enable shared decision-making, improve communication with healthcare professionals, and reduce stress for people living with arthritis and their families.

The survey identified a number of unmet medical needs, such as gaps in counselling provided to people with arthritis and regulatory gaps in terms of medications used during pregnancy and breastfeeding. In addition, the project has supported new collaborations and advocacy activities:

- Over 500 website visits have been recorded for the pregnancy and parenting with arthritis resource found on the CAPA website;
- Development of new research projects focused on [counselling needs of women with Systemic Lupus Erythematosus](#);
- Knowledge translation activities focused on family quality of life for mothers living with inflammatory arthritis;
- Working collaboratively with [Mother to Baby](#) – an organization supporting research into medication use during pregnancy and breastfeeding;
- Working with Creaky Joints to review a [resource on family planning](#);
- Advocating to Health Canada to express unmet patient needs relating to medication use during pregnancy and breastfeeding;
- a Tweet Chat on pregnancy / parenting which was well-attended by researchers, people with arthritis and patient groups. There were 28 participants in the Tweet Chat and almost 1 million impressions on Twitter.

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

As a result of the project, new relationships have been developed between CAPA and others in the arthritis community such as The Seated View, Mariah Leach Zebrowski (founder of Mamas Facing Forward), Creaky Joints, Mother to Baby, researchers in maternal health, and others.



Lessons learned

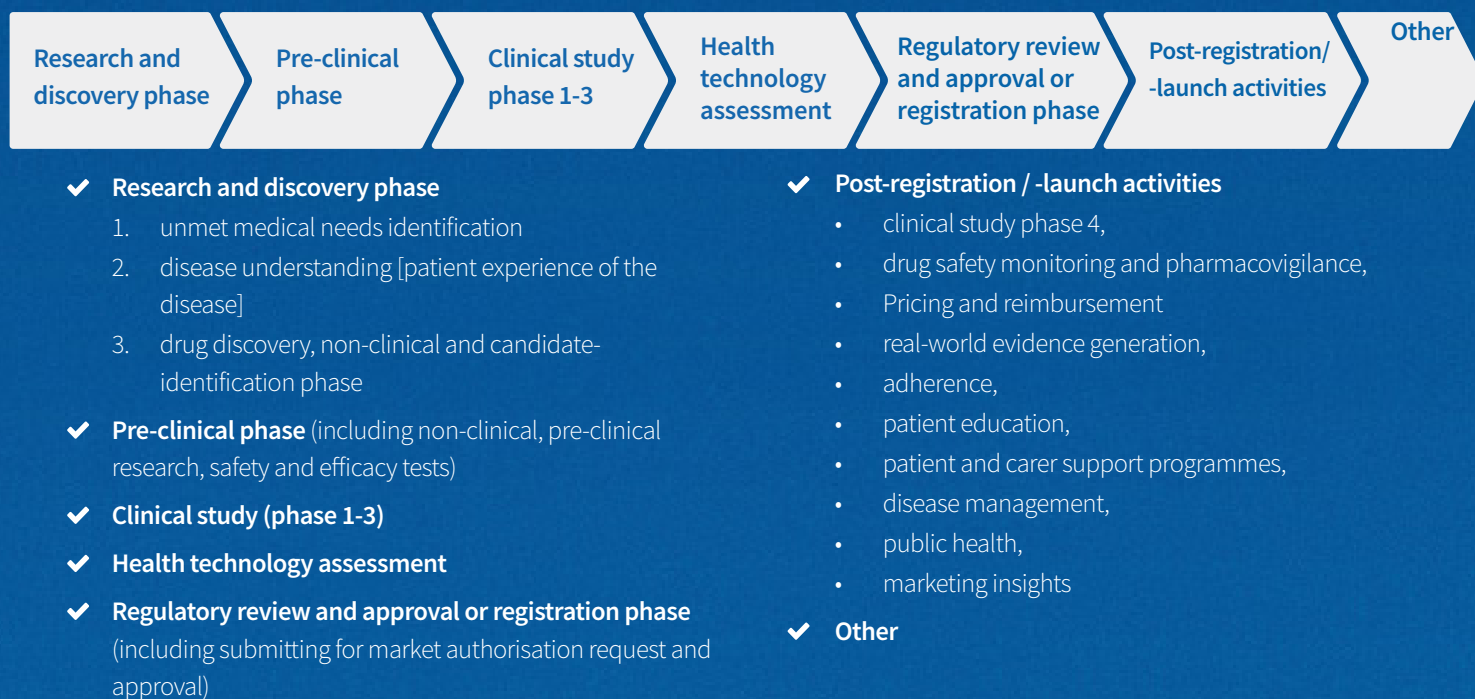
The project was identified as an important project to undertake given the first-hand experiences of the Steering Committee in managing pregnancy and parenting, and knowing there was a lack of credible, evidence-based information to support patients. We continue to be impressed by the response from other patients, health care professionals in rheumatology and other specialties and we continue to be approached for presentations and collaborations. More work is needed to sustain the momentum on this important project and we continue to remain open to future collaborations and partnerships to improve the patient experience.

The project was undertaken over a long period of time given that it was led by a volunteer patient organization. There are difficulties in managing resources and time commitments with the volunteer nature of our work and other life commitments including managing a serious, complex disease like inflammatory arthritis.

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.