

BMJ Open Characterising meaningful patient and public involvement in the pharmaceutical industry research setting: a retrospective quality assessment

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ABSTRACT

Objectives Patient and public involvement (PPI) in clinical research has a well-established infrastructure in the UK, and while there has been good progress within pharmaceutical-industry-sponsored research, further improvements are still needed. This review aims to share learnings from quality assessments of historical PPI projects within Pfizer UK to inform future projects and drive PPI progress in the pharmaceutical industry.

Design and setting Internal assessments of Pfizer UK PPI projects were conducted to identify all relevant projects across the medicines development continuum between 2017 and 2021. Five sample projects were developed into case studies.

Outcome measure Retrospective quality assessments were performed using the Patient Focused Medicines Development (PFMD) Patient Engagement Quality Guidance (PEQG) tool. Recommendations for improvement were developed.

Results Retrospective case study analysis and quality framework assessment revealed benefits of PPI to both Pfizer UK and to external partners, as well as challenges and learnings to improve future practice. Recommendations for improvement based on these findings focused on processes and procedures for PPI, group dynamics and diversity for PPI activities, sharing of expertise, the importance of bidirectional and timely feedback, and the use of understandable language in materials.

Conclusions PPI in medicines development is impactful and beneficial but is still being optimised in the pharmaceutical industry. Using the PFMD PEQG tool to define gaps, share learnings and devise recommendations for improvement helps to ensure that PPI is genuine and empowering, rather than tokenistic. Ultimately, these recommendations should be acted on to further embed PPI as an integral part of medicines development and health research within the pharmaceutical industry. This article includes a plain language summary in the supplement.

STRENGTHS AND LIMITATIONS OF THIS STUDY

- ⇒ This qualitative assessment was carried out against an established and validated framework, the Patient Focused Medicines Development Patient Engagement Quality Guidance tool.
- ⇒ The collaboration of pharmaceutical company employees and external partners with expertise in the field of patient and public involvement and lived experience who were brought together for the conduct of this review represented a heterogeneous and diverse range of perspectives.
- ⇒ The external partners may not be representative of the wider public, and their selection from a wider pool of potential partners may have resulted in the omission of some perspectives.
- ⇒ This retrospective qualitative assessment relied on the provision of historical evaluations and is subject to recall bias.
- ⇒ This work was conducted by the UK division of a single pharmaceutical company and their external partners; therefore the findings may not be fully representative of cross-industry or global perspectives.

INTRODUCTION

Patient and public involvement in health research

Patient and public involvement (PPI), also known as PPIE to incorporate engagement, is an initiative to include patients, family members, carers and members of the public in the research process of developing and improving health services and medicines.^{1 2} It may be both democratic, to enable the inclusion of voices on the basis of principle, and instrumental, to drive change and improvement according to the needs of users. There are many terms used to describe PPI interactions, which are often used interchangeably. These include involvement, engagement,

consultation and participation. The definitions used for the purposes of this research are as follows.

1. *Involvement*: when research is ‘carried out ‘with’ or ‘by’ members of the public rather than ‘to’, ‘about’ or ‘for’ them’. It is an active collaboration between patients, public and researchers.²
2. *Engagement*: when research information is shared with the public, for example, at research open days or on social media. Engagement is a two-way process, involving interaction and listening, with the goal of generating mutual benefits.²⁻⁴
3. *Consultation*: when feedback and input are solicited from people on how research or other PPI activities are carried out.⁵
4. *Participation*: when people take part in research, ranging from a one-off survey or questionnaire completion to recruitment into clinical trials or research studies for the active testing of experimental medicines and procedures.²

This paper focuses on PPI with patients and carers as external partners, also known as lay contributors or experts by experience, in the activities of a pharmaceutical company relating to medicines development, rather than as subjects of research.

The UK has a well-established infrastructure to enable meaningful PPI, backed by government policy, regulations and research funders.⁶⁻¹¹ The ‘gold standard’ model of PPI is based on both scope and depth of involvement; individuals should be contributing early on in research processes from strategy and governance with contributions to generating research ideas and priority setting, through to delivery and reporting of research, but also ensuring that PPI contributions are meaningful and of high quality regardless of the research stage. PPI in research has grown markedly in importance;¹²⁻¹⁴ it is integral to the conduct of academic research and service redesign in the UK and is often mandated by funders such as the National Institute for Health and Care Research (NIHR).¹⁵ However, the following questions remain pertaining to PPI in pharmaceutical industry-led research and projects.

- ▶ Is the development of a PPI infrastructure and meaningful involvement reflected within the pharmaceutical industry?
- ▶ What is meant by meaningful PPI?
- ▶ Why should PPI be important to the pharmaceutical industry?
- ▶ What is the importance of PPI opportunities to patients and carers?

PPI in medicines development: a Pfizer perspective

According to the Association of the British Pharmaceutical Industry, the goal of pharmaceutical companies is to bring the right treatment to the right patient at the right time.¹⁶ The pharmaceutical industry increasingly recognises that research strategies and products must be built based on what matters to patients, or companies will cease to have purpose. Pharmaceutical companies

are also recognising the value of PPI and are increasingly including and expanding PPI practices across the medicines development continuum, to ensure that research is truly focused on the needs of future users.¹⁷ Opportunities exist to implement end-to-end collaboration from study development and design—by capturing outcomes and values relevant to patients—through to dissemination of outputs and development of educational materials and policy. True partnership includes genuine dialogue and a feedback loop with all involved.

Leveraging their long-term partnership with the NIHR, Pfizer, like many innovative pharmaceutical companies, has involved patients and carers in clinical development programmes¹⁸ and continues to expand PPI practices across the medicines development continuum and earlier on in individual programme development. However, there are still barriers to becoming truly patient-centric, particularly for global companies in which organisational decision-making can sometimes be a challenge and/or feel very remote from patients, particularly for those operating in geographical regions where PPI is less well established. This is in part due to the differing compliance requirements across countries, a variable recognition of the benefits and impact of PPI and, sometimes, a lack of knowledge and operational expertise. There is also a need to manage shareholder expectations and the concrete manifestations these expectations create in terms of timelines and the ability to gain input from all external stakeholders; pharmaceutical companies are profit-making organisations, but they are increasingly defining themselves as purpose-driven. These industry-wide organisational challenges demonstrate the importance of undertaking and sharing PPI quality assessments and the relative novelty of doing so, both for democratic reasons to align with purpose-driven organisational goals and for instrumental reasons to ensure that medicines are fit for purpose.^{19 20} Ultimately, changing company culture to create an environment in which employees are compliantly and systematically involving patients and external partners is the first step, but maintaining meaningful, high-quality PPI is the goal.²¹

Guidelines and frameworks to enable meaningful PPI

There are several UK guidelines, frameworks and governance in place to facilitate and guide the integration and reporting of meaningful PPI in health research.²²⁻²⁶ Given the growing focus on meaningful PPI within pharmaceutical medicines development, there have been recent initiatives to develop frameworks specifically for the pharmaceutical industry.²⁷⁻²⁹

Within Pfizer UK, the Patient Focused Medicines Development (PFMD) Patient Engagement Quality Guidance (PEQG) tool³⁰ has emerged as a standard internal benchmark for PPI work for self-reflection and to recommend future ways of working. The preference for the PEQG tool is based on the robust process that went into designing it, specifically with medicines development in mind. It is a practical, easy-to-use guide that was co-created by the

multistakeholder PFMD collaboration, of which Pfizer is a partner, that involves industry, academia and patient organisations. The tool can be used prospectively or retrospectively to plan, develop and assess the quality of PPI activities and projects throughout medicines development.^{29 31} There are four key stages of the tool: (1) basic information, (2) quality assessment, (3) results and outcomes and (4) lessons learnt. The quality assessment stage considers seven key quality criteria: (1) shared purpose, (2) respect and accessibility, (3) representativeness of stakeholders, (4) roles and responsibilities, (5) capacity and capability for engagement, (6) transparency in communication and documentation and (7) continuity and sustainability.

PPI in pharmaceutical medicines development in the literature

Prior to undertaking the current retrospective quality assessment, an initial scoping review of the MEDLINE and Embase databases was performed via Ovid to contextualise the current landscape of reporting on PPI initiatives in pharmaceutical medicines development in the literature. Publications reporting on PPI in medicines development from database start dates to October 2021 were identified using the search terms, 'pharmaceutical industry', 'medicines development', 'drug development', 'patient engagement/involvement', 'carers', 'patient centricity' and relevant synonyms (online supplemental appendix 2). The initial search yielded 157 papers. There was an increase in publications over time, with 54 records (34.4%) having been published between 1970 and 2015, and 103 records (65.6%) published between 2015 and 2021, demonstrating the recent increase in the importance of, and emphasis on, PPI. Approximately half of the publications (n=76 [48.4%]) were specific to medicines development, but few (n=32 [20.4%]) were published by the pharmaceutical industry, confirming a gap in reporting on PPI in pharmaceutical medicines development.

Current aims and objectives

Pfizer UK is adopting a strategic and meaningful approach to PPI across medicines development by reflecting on practices, with the goal of sharing learnings with the wider community and presenting recommendations for future improvement, particularly in light of the limited literature on the topic. This assessment was a collaboration between Pfizer colleagues and non-pharmaceutical partners, including patients, carers, patient organisation representatives and NIHR and National Cancer Research Institute representatives, all of whom have previously been involved in Pfizer PPI projects. Here, we aim to consider the following.

- ▶ What PPI has Pfizer conducted across medicines development in the UK in the past 5 years?
- ▶ How has Pfizer partnered with patients and carers and what were the benefits to both parties?
- ▶ What does meaningful quality involvement look like, based on the PFMD PEQG tool?

- ▶ What were the challenges, lessons learnt and recommendations to drive future improvements?

METHODS

Statement of PPI and formation of the manuscript writing group

A multistakeholder group including Pfizer employees and external partners was established from the outset for project review, case study development, quality assessment and manuscript development. Pfizer employees (n=7) were from the following departments: Medical Affairs (n=4; SB, SH, BP and NB), Medical Patient Partnerships (n=2; S-AD and LC) and Global Product Development (n=1; SE). External partners (n=7) had previously worked with Pfizer on PPI in medicines development and have PPI expertise and/or lived experience. This included a young person (n=1; AB), a carer (n=1; RD), an adult patient (n=1; KS), an NIHR representative (n=1; GP) and patient organisation or advocacy group representatives (n=3; AD, JP and EK). All external partners were involved in the design and conduct of the review, selected outcomes measures and contributed to the development of this manuscript, in line with the International Committee of Journal Medical Editors authorship criteria.³²

Sample project identification, retrospective quality review and external validation

An internal assessment of Pfizer UK PPI projects was conducted to identify all projects undertaken across the medicines development lifecycle in the last 5 years before project commencement, from 2017 to 2021. Samples of these projects were chosen for assessment against the PFMD PEQG tool and written up into case studies according to a predefined proforma. Selection criteria for sample projects ensured that they represented different stages of medicines development across different years in the 5-year duration and that they had writing group individuals involved. The rationale for this was that writing group members would have a first-hand understanding of their projects and relevant connections for external feedback and were therefore best placed to evaluate the case studies. The PFMD PEQG tool was selected for assessment on the basis that it was designed specifically for medicines development, codeveloped with a multistakeholder group, has been used previously within Pfizer and is considered user-friendly and easy to apply.²⁹

Sample projects were retrospectively assessed using the PFMD PEQG tool to identify gaps and draw out benefits and challenges, as well as learnings and recommendations for future practice. For external validity and accuracy and to allow a wider perspective on specific areas of development, the case studies were reviewed by up to three individuals each who were external to the writing group but who had been involved in each of the respective projects, except for one case study for which finding such an individual was not possible. These individuals included 11 external partners (patients, carers, young people and patient organisation representatives) and three Pfizer employees who were all asked to evaluate the

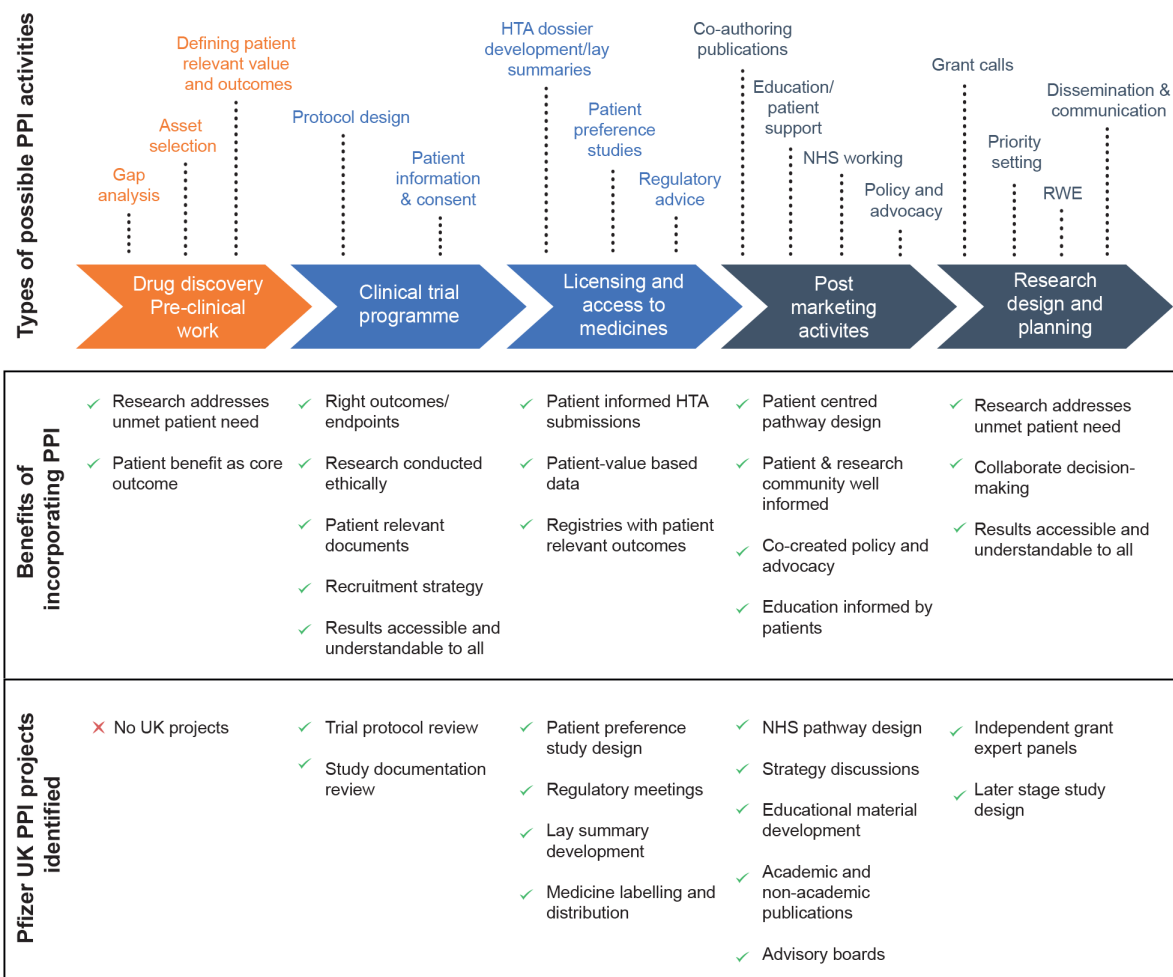


Figure 1 Types of possible PPI activities, the benefits of incorporating them and the Pfizer UK PPI projects identified across the medicines development continuum. HTA, health technology assessment; NHS, National Health Service; PPI, patient and public involvement; RWE, real-world evidence.

case studies against predefined questions (online supplemental appendix 3). Discussion and consensus of challenges and lessons learnt among the writing group led to establishing the final gaps and recommendations for future best practice.

RESULTS

Project identification

Overall, approximately 50 Pfizer projects were initially identified in which UK patients, carers or patient organisations were involved along the medicines development continuum over the past 5 years from 2017 to 2021. The level and type of involvement differed depending on the stage of medicines development (figure 1). Otherwise, there was a fairly even distribution of projects, with varying levels of involvement, across medicines development. Most involvement was identified at the clinical trial stage, within clinical development programmes (n=15). In contrast, few UK projects were identified at the drug discovery stage, with minimal involvement during the generation of real-world data and evidence and during post-authorisation activities. Five sample projects were

selected to be analysed in more detail and written into case studies (online supplemental appendices 4–8).

Retrospective assessment using the PFMD PEQG tool

Stage 1: basic information

Selected sample projects ranged from early phase clinical trials to post-authorisation independent competitive grant programmes. All sample projects were completed in a single year within the 5-year period, included a range of stakeholders and represented a combination of established groups and newly formed groups (table 1).

Stage 2: quality assessment

Sample projects were systematically and retrospectively compared against the seven PFMD quality criteria to assess quality of involvement and to identify gaps for future improvement. Table 2 provides an overview of each quality criteria assessment and the gaps identified.

Stage 3: results and outcomes

Collated feedback showed that benefits and challenges of PPI varied depending on the project (table 3), but bidirectional sharing of learnings was important across

Table 1 Basic information of the five sample projects

Project title	Type of engagement (number of meetings)	External collaborator	Individuals involved	New or established group	Stage of medicines development	Year of completion
Working with patients to review a rheumatology clinical trial protocol	Face-to-face (1)	Versus Arthritis	Nine adult patients	Established, but not all had worked together previously	Phase 4 clinical trial	2017
Working with young people to review a dermatology clinical trial	Face-to-face (1)	GenerationR Liverpool YPAG	Nine young people	Established	Phase 1–3 clinical trial	2018
Working with an NIHR patient focus group to review two gastroenterology clinical trials	Face-to-face (1)	NIHR Clinical Research Network	Five adult patients	New group	Phase 1–3 clinical trial	2019
Working with parents and carers to review dermatology study documentation	Virtual (2)	GenerationR Liverpool Parent/caregiver focus group	Seven parents and carers	Established	Phase 1–3 clinical trial	2020
Independent medical grant call: quality improvements in rheumatology practice: delivering change for patients	Virtual (5)	Versus Arthritis and NHS clinicians	Two clinicians, two adult patients, one carer and one patient organisation representative	New group	Awarding independent medical grant(s)	2021
NHS, National Health Service; NIHR, National Institute for Health and Care Research; YPAG, Young Persons' Advisory Group.						

all projects. For Pfizer, key learnings helped to change clinical trial protocols and associated documentation, to improve understanding of priorities and to ensure research is patient-focused. For patients and carers, there was benefit in understanding the workings of a large pharmaceutical company and how their feedback can lead to change, and in having the opportunity to have their opinions heard and feel listened to. Reported challenges included time taken to set up projects, timings of meetings and ensuring materials were truly lay-friendly.

Stage 4: lessons learnt and external validity check

Lessons learnt captured by the writing group and added to during the external validity check are included in table 4. The additional external validity assessments collected quality assessment feedback from individuals who were part of the individual project groups, but not included in the manuscript writing group; this was collected for four out of five projects and documented in the case study reports. This was not possible for the NIHR Gastroenterology project owing to a lack of availability of project group members. In general, the individuals that gave feedback felt that the case studies were an accurate representation of the projects they were involved with.

Development of recommendations

Based on the findings from the assessment using the PFMD PEQG tool, defined gaps and lessons learnt, a set of recommendations were established to improve PPI. These are grouped based on the seven quality criteria and will form the basis of an action improvement plan within Pfizer.

1. Shared purpose

- ▶ Time is required at the start of a project, to define a shared purpose that is agreed on by the group, written down and revisited at each meeting, and adjusted if the purpose changes.

2. Respect and accessibility

- ▶ Have a process in place to truly ensure that all materials used are accessible and in lay-friendly language. Consider the health literacy needs of group members and the format of pre-read materials, for example, written formats versus audio-visual formats.
- ▶ Ensure that contracting language is in plain English and that there is enough time during contracting to allow people time to understand, digest and ask questions.

Table 2 Quality assessments across all sample case studies summarised using the seven PFMD PEQG quality criteria

PFMD quality criteria	Summary of quality assessments across all sample case studies	Defined gaps
1. Shared purpose	<ul style="list-style-type: none"> ▶ This quality standard was considered and discussed for most projects in the context of why people were there, although it was not formalised. ▶ For some projects, this may not be suitable or required, such as a standalone 1-hour workshop. For others this would be beneficial to prevent confusion, to ensure everybody is clear on the project aim and why they are there, and to ensure everybody feels confident that their views are important and incorporated. 	<ul style="list-style-type: none"> ▶ There was not always a shared purpose written down, accessible for all and frequently revisited.
2. Respect and accessibility	<ul style="list-style-type: none"> ▶ For all projects, it was important that the timings and set-up of the meetings were planned around people's schedules and family lives. ▶ It was identified that considering the format of the meetings (face-to-face or virtual) offered wider opportunities, particularly during the COVID-19 pandemic, for improved participation and inclusion of people that may not have been able to get involved previously (ie, owing to reduced time requirements and absence of need for travel). This enables the voices of the less-often heard to be included. This was reflected in the reviewed projects, which were conducted in different ways, virtually and face-to-face, but always in collaboration with an external partner. ▶ How projects are undertaken, including what type of involvement is needed and whether the group is already formed, or whether individuals were brought together for the remit of the project, needs careful consideration. For example, for already-formed groups with established PPI expertise, codes of conduct may already be in place. However, this may not be the case if the project is undertaken without external partnership, or if it is the first time people have worked with a pharmaceutical company. Consideration of this is important. 	<ul style="list-style-type: none"> ▶ There were no written codes of conduct of what people could expect from Pfizer. ▶ Language used in contracting and other materials was not always accessible and written in a lay-friendly way. ▶ Logistics were a challenge for some projects.
3. Representativeness of stakeholders	<ul style="list-style-type: none"> ▶ Representation and diversity of project groups is a challenge for industry and patient organisations, evidenced in the case studies. ▶ Although much work was done throughout to ensure that the right people, skills and capabilities for the project were involved, for some of the sample case studies, especially those relating to clinical trials, this was a challenge because of feasibility in practice and the tight timelines leading to a lack of adequate time to do suitable outreach. 	<ul style="list-style-type: none"> ▶ More work is needed to ensure suitable outreach, and robust inclusion and diversity are needed to ensure representativeness.
4. Roles and responsibilities	<ul style="list-style-type: none"> ▶ In most of the sample case studies reviewed, this was done well, particularly when co-partnering with other PPI organisations. Co-partnering helped patients and carers involved in the projects to have an understanding and familiarity of who they should go to if they had questions. ▶ For some sample case studies, there was not clear documentation of roles and responsibilities outside of contractual arrangements, which external partners would not be familiar with reviewing. ▶ For the grant call project, it was deemed important that everyone (patients, clinicians and Pfizer employees) input into all of the stages of the grant call development (ie, research priority setting, request for proposal document, review of applications and decision to provide funding) to ensure alignment and to capture everyone's needs. ▶ It was considered that including patient co-chairs on research panels should be standard and good practice; this gives the other patients on the panel a feeling of parity of opinion and equity of voice. 	<ul style="list-style-type: none"> ▶ Roles and responsibilities were not always clearly defined and written down for people to refer to.

Continued

Table 2 Continued

PFMD quality criteria	Summary of quality assessments across all sample case studies	Defined gaps
5. Capacity and capabilities	<ul style="list-style-type: none"> ▶ We saw an improvement over time with this quality criterion. For earlier projects, pre-read materials were not always sent or were not sent in a timely manner. This is now done as standard and was deemed to be very important by the writing group. ▶ Existing PPI groups were more likely to have had some form of training, whereas newly formed groups will require more assistance in this area. The training could be project specific or general PPI training on what to expect and how to work together. 	<ul style="list-style-type: none"> ▶ Ensuring early on in the project that everybody has the correct skills to enable them to meaningfully contribute is essential, and training should be offered if needed.
6. Transparency in communication and documentation	<ul style="list-style-type: none"> ▶ This was done well in most projects, with communication plans and documentation storage and accessibility agreed early on at the start of the project so everybody was clear. ▶ Virtual platforms, when unanimously agreed upon, can allow equitable access to project-related documentation for all. ▶ Providing feedback in a timely manner is an important consideration and was not always achieved, particularly in projects conducted in earlier years. ▶ In some instances, more consideration should have been given to the style of communication. For example, the format of materials should be aligned with the group's needs; videos and infographics for the projects including young people were preferred. 	<ul style="list-style-type: none"> ▶ Pre-read materials were not always in an accessible format or sent with enough time to allow information to be digested and questions formulated.
7. Continuity and sustainability	<ul style="list-style-type: none"> ▶ The projects assessed for this work were mainly short-term projects that were not feasible to sustain and continue. Continuity was achieved by the understanding of how learnings would be implemented. For example, although a specific clinical trial protocol was being reviewed, general learnings were taken back to the development teams for consideration in future clinical research programmes. ▶ The group agreed that this aspect was of great importance and continued working should be acknowledged and discussed. 	<ul style="list-style-type: none"> ▶ Continuity of work was not always part of the agenda or discussion.

PEQG, Patient Engagement Quality Guidance; PFMD, Patient Focused Medicines Development; PPI, patient and public involvement.

- ▶ Develop a code of conduct document. This should be bidirectional and should define what can be expected from all parties. This is especially important for new groups being brought together for a specific project.
- ▶ Work as equal partners and be clear about boundaries and roles of stakeholders. Share expertise throughout; external organisations have significant experience that pharmaceutical companies can benefit from.
- ▶ Timings and locations of meetings need careful consideration. Think about who is attending, what times would be most suitable and which venues are accessible and comfortable. Online meetings have additional benefits and challenges to consider.

3. Representativeness of stakeholders

- ▶ Ensure true representativeness of stakeholders; consider using a sampling framework at the start of each project to determine suitable and appropriate outreach. Sufficient time should be given to do this well.

4. Roles and responsibilities

- ▶ At the outset, devise a roles and responsibilities document to use as part of project set-up and conduct. Provide adequate time for everybody involved in the project to share their input and ensure everybody is clear.
- ▶ When working with external partners, it is important that all involved provide input into all the stages of development to ensure alignment and agreement of needs. We saw this was particularly important with independent research grants.

5. Capacity and capability for engagement

- ▶ Consider who you will be working with and if the group is already formed or is being brought together for the purpose of the project; these will require different approaches.
- ▶ Be clear about the type of involvement needed and the skills and capabilities required; develop a template that can be populated for each project.

Table 3 Results and outcomes of the sample case studies in terms of benefits, impacts and challenges to stakeholders

Project title	Benefits and impact to Pfizer	Benefits and impact to patients, carers and other organisations involved	Challenges
Working with patients to review a rheumatology clinical trial protocol	<ul style="list-style-type: none"> ► Understanding patients' perspectives on the delivery of the trial itself; for example, adjusting patient-reported outcome measures, optimising patient symptom diaries or reducing the number of questionnaires to a more practical number for patients. ► Informing the focus of the clinical research plan and providing practical/logistic considerations around trial design for general inflammatory conditions. ► Clarifying the importance of patient insights when developing protocols. 	<ul style="list-style-type: none"> ► From the charity's perspective, supporting the voice of people with arthritis in influencing how the study was carried out and how the involvement of patients could be maximised in the study itself. ► For patients, having the opportunity to learn more about a large pharmaceutical company and how feedback can lead to change, ultimately breaking down barriers between pharmaceutical companies and patients. 	<ul style="list-style-type: none"> ► Timely project set up. ► Ensuring materials were in truly lay-friendly formats and language and timely communication for feedback. ► Patients felt that insights could have had more impact if involved from the outset, rather than being brought in once it was underway.
Working with young people to review a dermatology clinical trial	<ul style="list-style-type: none"> ► Having the opportunity to understand the perspectives of young people. ► Developing subject assent forms and understanding what young people would like to see included. For example, when shown a cartoon diagrammatic representation proposed for the assent forms, the young people made it clear that they would find it patronising and would prefer to see photographs instead. 	<ul style="list-style-type: none"> ► Being able to provide valuable insights that would shape a commercially sponsored protocol to benefit more patients. ► This was the group's first commercially sponsored protocol from a large pharmaceutical company, as opposed to academic protocols historically reviewed by the group. This allowed them to see how different areas of research operate. 	<ul style="list-style-type: none"> ► Creating materials and information to describe the study design in plain and clear language, suitable to be understood by a group of young people. ► Confidentiality and role of agreements. ► Time required for PPI managers to gain clarity on what Pfizer wanted and making sessions accessible for young people.
Working with NIHR patient focus group to review two gastroenterology clinical trials	<ul style="list-style-type: none"> ► Gaining a greater understanding of what it is like to live with the condition being studied. ► Receiving thoughts on the study design; feedback from participants was open and insightful and gave greater depth of consideration for obviously invasive and disruptive procedures. ► Clear information on where the patients would look if they were interested in taking part in a clinical trial. ► Insights into the role that treating clinicians play with trial decisions. 	<ul style="list-style-type: none"> ► Participating in direct conversation between Pfizer representatives and patient participants. ► Working with an established and trusted organisation (NIHR) gave patients greater peace of mind. ► Allowing patients the opportunity to explain what outcomes they value and would prioritise. 	<ul style="list-style-type: none"> ► Logistics, primarily due to weekend meeting requirements. ► Patient information should have been circulated earlier prior to the meeting.
Working with parents and caregivers to review dermatology study documentation	<ul style="list-style-type: none"> ► Understanding how to present the informed consent form and what information should be given, particularly with respect to procedures like blood sampling. 	<ul style="list-style-type: none"> ► Feeling their opinion was truly important. ► Understanding that pharmaceutical companies are willing to listen to patients and families. ► Accepting future invitations to support industry-led studies. 	<ul style="list-style-type: none"> ► Finding a convenient time for all. ► Ensuring Pfizer materials were in lay-friendly language.

Continued

Table 3 Continued

Project title	Benefits and impact to Pfizer	Benefits and impact to patients, carers and other organisations involved	Challenges
Independent medical grant call: quality improvements in rheumatology practice: delivering change for patients	<ul style="list-style-type: none"> ▶ Collaborating with patients ensured that there was a patient-prioritised research agenda. ▶ The research questions and outcomes were relevant to patients and translatable to the NHS. ▶ Including patient organisation perspectives allowed the research call to be generalisable to the wider community. ▶ The clinicians involved in the project reported that they found that the process was inclusive, and that the opportunity to listen and learn from the patients and carers would help inform future practice. ▶ Using learnings gained to optimise ways of working in future projects to enable more patients to be at the centre of decision-making. ▶ Patient insights were extremely valuable and helped to influence change; eg, the proposal draft received over 50 comments from patients that were incorporated into the final document. 	<ul style="list-style-type: none"> ▶ For the patient organisations, influencing the research call to ensure it aligned with their priorities, which were set in collaboration with external partners and healthcare professionals. ▶ For patients, feeling involved in the whole process, particularly co-chairing the external review panel, to ensure that the patient voice is heard and considered equally. 	<ul style="list-style-type: none"> ▶ Allowing adequate time for everybody to complete the reviews required. ▶ Comprehensively incorporating PPI processes within the grant call request for proposals.

NHS, National Health Service; NIHR, National Institute for Health and Care Research; PPI, patient and public involvement.

- ▶ Consider any additional training or support that people may require and offer this at the outset.
- ▶ Use an established PPI framework or quality guidance and/or a group of experienced PPI representatives to ensure meaningful, high-quality and impactful engagement.
- ▶ Do not underestimate the capabilities and value of young people; ensure young people are involved in a meaningful way and not excluded.

6. Transparency in communication and documentation

- ▶ Ensure enough information (eg, pre-read materials) is sent in advance of meetings and that this is done in a timely manner in appropriate formats and language.
- ▶ Thank people and provide feedback in a timely manner, ensuring that they understand what impact their contributions have had.

7. Continuity and sustainability

- ▶ Continually evaluate the meaningfulness, benefits, challenges and impact of PPI in medicines development to enable improvement in practice.

Other general considerations

- ▶ There are valuable learnings within pharmaceutical companies, which should be shared openly and in a practical way to improve practice throughout the industry.

- ▶ Involve people as early as possible in the process; understand within the organisation where this needs to happen and implement change.
- ▶ Patient organisations have significant experience and expertise that pharmaceutical companies can benefit from. They can also gain a better understanding about pharmaceutical companies' approaches to implementing PPI learnings and vice versa. Sharing expertise and evaluating along the way is important.

DISCUSSION

This work was carried out from a multistakeholder perspective, with direct learnings from a pharmaceutical company. Most PPI assessment carried out internally by companies and institutions is rarely shared with the wider community. The initial scoping literature review prior to this work discovered limited published pharmaceutical company experience, with 20.4% of the relevant literature originating from the pharmaceutical industry, demonstrating the need for sharing and reporting of industry findings in the PPI space.

This work therefore recognised from the outset the importance of communicating internal findings and aimed to share learnings from the selected projects, which will provide additional material to the existing literature and improve the PPI knowledge base in a practical way. This review identified some expected challenges,

Table 4 Lessons learnt from the five sample case studies

Corresponding PFMD quality criteria	Lessons learnt
1. Shared purpose	<ul style="list-style-type: none"> ▶ Time is required at the start of meetings to ensure that a shared purpose is created and consistently reviewed. This is essential for optimal outcomes.
2. Respect and accessibility	<ul style="list-style-type: none"> ▶ For future projects, a code of conduct—also known as a list of expectations or ways of working documents—should be codeveloped by all involved and implemented. This could be a document or slide outlining what is expected of people and what people can expect from Pfizer, as well as how their feedback will be used. This should be sent to everybody prior to the first meeting or presented at the start of a meeting. This would help the group to understand what mutual respect should look like and to be able to form and respect diverse views. This is especially important for a new group, as existing groups may already have their own codes of conduct in place. ▶ Language and health literacy should be given thought, time and consideration, and involving patients and carers in drafting of materials is key. Technical research language, when read by a patient, may not only be unclear but actively dispiriting. There is a need to agree on consistent language at the outset. Glossaries of terms can also be very helpful. ▶ Contracting language should be accessible and explained to patients and other organisations; there should be enough time given for contracting to allow people to understand, digest and ask questions. ▶ Timings of meetings, the types of meetings and where meetings are held should be given careful consideration, with particular regard to accessibility of amenities.
3. Representativeness of stakeholders	<ul style="list-style-type: none"> ▶ More planning, time and resources to ensure suitable outreach and representativeness is required. ▶ The capabilities and value of young people should not be underestimated and should be included in relevant projects. ▶ This is a call to action for industry, academia and patient organisations to work together.
4. Roles and responsibilities	<ul style="list-style-type: none"> ▶ At the outset of a project, it is valuable for the project team to review roles and responsibilities and co-create these with patients and carers. ▶ Having this written down and discussed at project meetings would help to make sure people are clear on what is expected of them.
5. Capacity and capabilities	<ul style="list-style-type: none"> ▶ Project leads should consider who they will be working with. For example, an already-formed group who are used to working with each other may feel more confident to speak up, compared with a group that has been newly brought together specifically for a project. This will require consideration by the project lead to ensure everybody can contribute equally. ▶ Using an established PPI framework and/or a group of experienced, trained representatives to ensure meaningful, high-quality and impactful engagement is key.
6. Transparency in communication and documentation	<ul style="list-style-type: none"> ▶ Ensuring that pre-read materials are sent with plenty of time in advance of meetings. Consideration should also be given to the format of these materials (eg, written vs audio-visual or multimedia materials). ▶ Consideration should be given to the health literacy needs of the group and the relevance of material types based on the format of the project. ▶ Feedback should be timely and in a format that is acceptable. It should include enough detail so that stakeholders can understand how their involvement has made a difference.
7. Continuity and sustainability	<ul style="list-style-type: none"> ▶ Continuity of work should be discussed towards the end of the project and involve listening to ideas, suggestions and views on continued partnership.
PFMD, Patient Focused Medicines Development; PPI, patient and public involvement.	

such as the difficulty of addressing and achieving true inclusion and diversity. It also highlighted organisational constraints often associated with the complicated procedures that global pharmaceutical companies have owing to the nature of their activities,³³ noting that not all research timelines are conducive to undertaking suitable outreach activities. This work demonstrated that Pfizer is currently involving patients and carers in many different areas across the medicines development continuum, but there is room for improvement. In the assessed projects, external partners were not involved early on enough in the process to have maximal impact on medicines development, limiting the value they could provide. In the authors' view, these organisational barriers may, in part, be due to delays to starting research when pharmaceutical companies collaborate with external organisations,

detracting from the time available.³³ It could also be due to a lack of understanding within pharmaceutical companies of the value of PPI during these early stages and the need to manage shareholder expectations,³⁴ or it could be due to the global nature of many pharmaceutical companies, meaning that much of the early preclinical and clinical research is conducted outside of the UK.^{35 36} For example, regulatory requirements may mean that not all proposed changes are feasible. However, regardless of the stage of PPI, it is the depth and scope of the involvement, as well as honesty and transparency in managing expectations, that will ensure quality and meaningfulness, with meaningful PPI being characterised by providing relevant opportunities to contribute to and have a tangible impact on research.

There are differences in motivations, practices and outcomes of PPI when comparing non-profit and for-profit organisations; these differences can affect how PPI is embedded and developed, and how it is perceived as meaningful. When asked to reflect on and compare experiences of involvement in non-profit versus for-profit environments, our external partners felt that in PPI activities organised by non-profit institutions, people may contribute time and effort for altruistic reasons, and may not always be driven by an offer of financial compensation. These organisations are typically valued and trusted more by the public, whereas for-profit organisations may not be perceived in the same way. PPI activities driven by for-profit organisations may, in some cases, be less impactful, for example, when PPI is not sufficiently considered at all stages of research. Non-profit and for-profit organisations can learn from each other's PPI practices and collaborate on activities, such as through cross-funding on a grassroots level to establish 'profit for purpose' approaches. Cross-industry collaborations can also help to bust myths or assumptions about each other. In terms of defining meaningfulness, our external partners also felt that when PPI is done in a limited or prescribed way, it risks becoming tokenistic, for example, when used solely as a requirement to secure funding or approval. The answer is to strike a balance—involve external partners in all stages of research and in a variety of activities, including contributing to performing research and interpreting results, when possible. PPI is an essential ingredient for furthering science and clinical research and improving health outcomes for communities, and rationale and accountability matter in ensuring quality and meaningfulness. For example, rationales for embedding PPI may come from different places, from wanting to understand impactful best practices through to simply meeting requirements. However, as long as the result is that PPI is fully embedded at an involvement level and not just at an engagement level, its inherent value should become clearly demonstrated over time in terms of outcomes, although the measure of the benefit will vary across organisations, countries and disease areas. Pharmaceutical research has historically been an inaccessible field for external partners, so PPI is an important tool for improving collaboration and understanding between parties and making people aware of how they can positively impact research.

From the perspective of our external partners, there are also considerations to be mindful of in terms of how external partners are given a seat at the PPI table, whether they are explicitly invited or whether they have requested to have their voice heard. Our external partners felt that those who are invited to the table may be afraid of voicing criticism and unpopular opinions for fear of offending the people they have been invited by, and thus not being invited to future consultations. On the other hand, those who apply for a place at the table are likely to be more critical and honest, and thus make more insightful contributions. In addition to how external partners reach the

table in general, it also matters which table they are at and under what terms they are at it. If the table is just an echo chamber with no impact on strategy or output, it does not matter how the external partners got there if there is no meaningful effect. This needs to be given thought and consideration and, ultimately, the goal is to inspire and influence a world in which voices are being heard, and collaboration between for-profit and non-profit organisations is a key way that this can be achieved.

This work highlighted that future projects should clarify the shared purpose between stakeholders and have clear roles and responsibilities designated to each from the outset. It is helpful to explain why PPI is being sought and to convey this in an enthusiastic and collaborative way to inspire maximum engagement. Ensuring PPI work is carried out in friendly, approachable and familiar environments allows people to feel empowered and engaged.³⁷ Familiar environments could be supported by virtual engagements³⁸ and by working collaboratively with external organisations, such as patient organisations in which people are likely to already feel comfortable.^{39 40} The need for trust and rapport is vital, especially when public trust in the pharmaceutical industry may be low.⁴¹ Trustworthiness should be established early on in the PPI process, through collaborative ways of working and mutual respect; contributors need to have the value of their input recognised by being thanked and given the opportunity to provide feedback in a timely manner. This was evidenced in this work, in which external partners commented on the positive benefits of working with a pharmaceutical company and felt their opinion was truly important and that pharmaceutical companies were listening to patients and families. Through PPI activities and continuous sharing of learnings, patients, carers and patient organisations are able to gain a general understanding into pharmaceutical companies' approaches and vice versa. Such dialogues and interactions may even help to dispel preconceived notions each party may have about the other.⁴² Accessible language and formats of materials are vital when establishing rapport and engagement. It is important to be mindful of being accessible without being patronising. Language is an important enabler of meaningful involvement, but can also create barriers.⁴³ In recognition of this, a glossary of terms was developed to supplement this paper (online supplemental appendix 9). Health literacy principles should be embedded into materials development; Pfizer is implementing robust staff training for health literacy and expects its principles to be applied throughout the conception, development and approval of health information materials. Furthermore, relevant UK contracts are being rewritten to be more easily understood and aligned with the health literacy and readability needs of the public; other documentation is also being reviewed to improve the understandability and accessibility of information. Accordingly, informative pre-read materials that are provided to people must be carefully considered in terms of format and content to ensure information is conveyed in a way that is accessible to the

target audience. Useful formats can include PowerPoint slides and PDFs, but there is scope to use more imaginative and novel formats when suitable, particularly when engaging with young people.^{44–46}

Based, in part, on the authors' interpretations of these learnings, Pfizer has recently undertaken several actions to overcome some organisational challenges that may limit the capacity for and effectiveness of PPI identified in this quality assessment. These include incorporating new roles within the UK medical and policy teams specific to partnering with patient organisations, patients and carers. Pfizer has also produced internal company training on meaningful PPI as part of an initiative to raise awareness of the importance of patient involvement and to align with Pfizer's overall aim of 'being known as the most patient-centric organisation'. Further to this, Pfizer is devising templates for systematic, efficient and meaningful engagement with people to gain maximum feedback, while being respectful of the value of each person's time. Ultimately, PPI is not a quick fix but an initiative that needs long-term investment of time and resources.^{47 48} Considering all of the above will allow inclusion of a more diverse and enduring patient voice across medicines development.

Limitations of this study

Limitations of this study included the selection process of a relatively small manuscript writing group. Because Pfizer has conducted many PPI projects across medicines development and across therapy areas, not all voices could be included in this assessment, potentially leading to a lack of representation. Opinions of the group may also not be representative of the wider public and particularly of demographics that are typically under-represented in research, such as healthy young people or migrant populations. However, it should be noted that, although PPI and representation cannot provide the definitive voice, for example, of the public or of a patient group, it still provides an important voice. The external validation step of the case studies was employed to help counter this limitation. This work was also UK-specific and the findings need to be viewed in this light. As many pharmaceutical companies have global headquarters and operations, the distribution of projects across the medicines development continuum may differ, particularly in the earlier phases.^{35 36} Further research is required to draw conclusions about PPI in medicines development in a global context. In the time since starting this work, PPI across medicines development in Pfizer has expanded and additional case studies have been assessed; these may add value as part of a subsequent review to look at a greater breadth of projects for improved representation. Furthermore, this manuscript represents the work and views from a single pharmaceutical company. Future work should expand what has been learnt here as a cross-industry collaboration. Lastly, retrospective analysis is challenging and subject to recall bias.⁴⁹ This was evidenced in the external validation feedback, in which one patient reviewer noted the age of the projects and the difficulty in remembering details. Involving

several individuals in writing and reviewing the case studies may have helped close any recall gaps.

Strengths of this study

The manuscript writing group was heterogeneous and represented different stakeholder groups including industry representatives, patient organisations, NIHR representatives, carers, young people and adult patients. The diverse range of voices and perspectives enabled a greater ability to draw meaningful conclusions from this research; future groups may consider learning from these experiences. This work was systematically carried out against an established framework, which is considered a valid and robust tool.²⁹ External validity assessments were also employed to incorporate as many voices and views as possible. The results of this work will be used by Pfizer to refine a PPI improvement and implementation action plan that is currently in development. This work provides value by contributing to the limited literature and raising awareness of the importance of meaningful PPI in the pharmaceutical industry and may be particularly informative for organisations and individuals wishing to partner with pharmaceutical companies. It also has translational benefits for PPI in future medicines development activities across the Pfizer global organisation and for other industry professionals.

CONCLUSIONS

There is wide acceptance that PPI in clinical research and medicines development is impactful and meaningful for all parties involved, but it is still developing and, while there has been good progress within pharmaceutical industry research, further improvement is needed.^{12–14} This includes involvement early on in medicines development so that maximum input can be achieved at all stages. There is already established PPI guidance available,^{6–11} but, to overcome organisational challenges and to embed PPI consistently and comprehensively throughout all stages of medicines development, it is the authors' views that pharmaceutical companies should have a dedicated team or role whose focus is PPI, with resources and capacity appropriately allocated. PPI must be genuine and empowering for people and not tokenistic or a 'tick-box' exercise; trust and rapport from the outset is vital. External partners need to feel that their contributions are being genuinely valued and used. It is also important to respectfully give thanks for their time and to ask for feedback of their experience of involvement so that lessons can be learnt. Here, the PFMD PEQG tool enabled useful reflection to define gaps, establish learnings and devise recommendations for future improvement. The recommendations presented in this work represent Pfizer's efforts to share learnings with the wider PPI community and industry, and are already a starting point for continuous improvement of future practice within Pfizer. Ultimately, these recommendations should be acted upon to further embed PPI as an integral part of

medicines development and health research within the pharmaceutical industry.

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Dews et al_supplementary materials

Appendix 1: Plain language summary

See next page

Do pharmaceutical companies involve patients and carers as partners in their research in a way that is helpful and meaningful?

This is a plain language summary based on the article *“Characterising meaningful patient and public involvement in the pharmaceutical industry research setting: a retrospective quality assessment”*. It was published in a medical journal called *BMJ Open* in August 2023.

You can read the article for free here: <http://dx.doi.org/10.1136/bmjopen-2022-071339>

- This research aimed to understand how the pharmaceutical company Pfizer has partnered with patients and carers, and whether this partnership was meaningful.





Who is this research of interest to?

- Anybody who would like to know more about how the pharmaceutical industry involves patients and carers in their work.

What was this research about?

- Patient and public involvement is also known as **PPI**. It happens when patients, carers and the public are included as partners in the design of health research.

There are 4 types of PPI:

Involvement	Engagement	Consultation	Participation
When people are actively involved in helping design or carry out research. The people involved in these projects are known as PPI partners .	When researchers share information with people, such as on social media or at open days.	When people are asked to give feedback on how research or other PPI projects are done.	When people take part in research, such as completing a survey or being an active participant in a clinical trial.
			

- Pharmaceutical companies and other research organisations are regularly including patients, carers and the public in PPI projects and activities. Looking back to review completed PPI projects is important to make sure they were done in a way that is helpful, meaningful and respectful to everyone involved, and to make sure that progress is being made. This is known as **retrospective quality assessment**.
- This research focuses on PPI projects that were carried out by the pharmaceutical company Pfizer across different areas of pharmaceutical research.

Why was this research done?

- It is important to share the results of PPI project retrospective quality assessments to help to improve PPI projects across the research industry. So far, there are not many pharmaceutical companies doing this. In this research, Pfizer and a group of their PPI project partners performed retrospective quality assessments of some of the company's PPI projects since 2017. They wanted to share their results with the public to help other pharmaceutical companies to learn and improve their PPI projects, and also to encourage others to share their results.

How was this research done?

- In this research, the researchers first looked in medical journal databases to find out what has already been published on this topic. Next, they looked at some of the previous PPI projects carried out by Pfizer to do retrospective quality assessments.
- There are different tools to help do retrospective quality assessments. One of these tools is called the Patient Focused Medicines Development Patient Engagement Quality Guidance tool, also known as the **PFMD PEQG tool**. The PFMD PEQG tool was designed by PPI partners. [It is available online for anyone to use here](#). It helps researchers decide if a PPI project was helpful and meaningful by looking at 7 aspects:

1. Shared purpose
2. Respect and accessibility
3. Representativeness of stakeholders
4. Roles and responsibilities
5. Capacity and capability for engagement
6. Transparency in communication and documentation
7. Continuity and sustainability

- The flowchart below shows how the researchers carried out the research and how they used the PFMD PEQG tool.



What were the recommendations the researchers developed?

- The researchers created and agreed upon 20 recommendations to help improve PPI projects in the future. They categorised these recommendations by the 7 aspects of the PFMD PEQG tool. The recommendations are:

1. Shared purpose	<ul style="list-style-type: none"> Time is required at the start of a project, to define a shared purpose which is agreed upon by the group, written down and re-visited at each meeting, and adjusted if the purpose changes.
2. Respect and accessibility	<ul style="list-style-type: none"> Have a process in place to truly ensure that all materials used are accessible and in lay-friendly language. Consider the health literacy needs of group members and the format of pre-read materials, for example, written formats versus audio-visual formats. Ensure that contracting language is in plain English and that there is enough time during contracting to allow people time to understand, digest and ask questions. Develop a code of conduct document. This should be bi-directional and should define what can be expected from all parties. This is especially important for new groups being brought together for a specific project. Work as equal partners and be clear about boundaries and roles of stakeholders. Share expertise throughout; external organisations have significant experience that pharmaceutical companies can benefit from. Timings and locations of meetings need careful consideration. Think about who is attending, what times would be most suitable and which venues are accessible and comfortable. Online meetings have additional benefits and challenges to consider.
3. Representativeness of stakeholders	<ul style="list-style-type: none"> Ensure true representativeness of stakeholders; consider using a sampling framework at the start of each project to determine suitable and appropriate outreach. Sufficient time should be given to do this well.
4. Roles and responsibilities	<ul style="list-style-type: none"> At the outset, devise a roles and responsibility document to use as part of project set-up and conduct. Provide adequate time for everybody involved in the project to share their input and ensure everybody is clear. When working with external partners, it is important that all involved provide input into all the stages of development, to ensure alignment and agreement of needs. We saw this was particularly important with independent research grants.
5. Capacity and capability for engagement	<ul style="list-style-type: none"> Consider who you will be working with and if the group is already formed or is being brought together for the purpose of the project; these will require different approaches. Be clear about the type of involvement needed and the skills and capabilities required; develop a template that can be populated for each project. Consider any additional training or support that people may require and offer this at the outset. Use an established PPI framework or quality guidance and/or a group of experienced PPI representatives to ensure meaningful, high-quality and impactful engagement. Do not underestimate the capabilities and value of young people; ensure young people are involved in a meaningful way and not excluded.

6. Transparency in communication and documentation	<ul style="list-style-type: none"> • Ensure enough information (eg, pre-read materials) is sent in advance of meetings and that this is done in a timely manner in appropriate formats and language. • Thank people and provide feedback in a timely manner, ensuring that they understand what impact their contributions have had.
7. Continuity and sustainability	<ul style="list-style-type: none"> • Continually evaluate the meaningfulness, benefits, challenges and impact of PPI in medicines development, to enable improvement in practice.
Other general considerations	<ul style="list-style-type: none"> • There are valuable learnings within pharmaceutical companies, which should be shared openly and in a practical way to improve practice throughout the industry. • Involve people as early as possible in the process; understand within the organisation where this needs to happen and implement change. • Patient organisations have significant experience and expertise that pharmaceutical companies can benefit from. They can also gain a better understanding about pharmaceutical companies' approaches to implementing PPI learnings and vice versa. Sharing expertise and evaluating along the way is important.

What did the researchers learn from this research?

- Overall, the researchers learned that:
 - There is little information published about PPI projects in pharmaceutical research. The researchers hope that publishing these results will help encourage other pharmaceutical companies to do the same.
 - The PFMD PEQG tool is useful for assessing previous PPI projects, to help make sure new PPI projects are even better in the future. This includes ensuring that PPI projects are helpful, meaningful and respectful for everyone involved. It also helps to make sure that researchers are not just doing PPI projects because they have to or because they want to look good.
- Pfizer is already using the recommendations that the researchers developed from this research to help create action plans for improving future PPI projects. The researchers hope other pharmaceutical companies and research organisations can also learn from and use these recommendations too.

More information

You can read the full article that this plain language summary is based on for free here: <http://dx.doi.org/10.1136/bmjopen-2022-071339>.

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Glossary

External validity – this is when researchers use a second method, unrelated to the first method, to make sure that the results are fair and accurate.

PFMD PEQG tool – the Patient Focused Medicines Development Patient Engagement Quality Guidance tool is a tool for measuring if a PPI project was done in a way that is helpful and meaningful.

PPI – Patient and public involvement is when patients, carers and the public are included in research activities.

PPI partner – Patient and public involvement partners are the patients, carers or members of the public who are involved in a particular research project.

Retrospective quality assessment – this is when researchers look at a previous project or activity to find out how well it was done.

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Appendix 2: Search strategy

Below is literature review search terms etc for supplementary;

OVID MEDLINE(R) 1946-present, OVID MEDLINE(R) In-Process & Epub Ahead of Print
Embase <1974 to 2021 October 20>

Terms used and lines related to in the table:

Pharmaceutical (company/industry ?) **line 31 & 32**
medicines development, drug development,
patient engagement **line 28**,
patient involvement **line 25**,
carers **line 36 & 37, 46-49**,
patient centricity

#	Query	Results from 9 Nov 2021
1	exp *biomedical research/	304,627
2	exp *medical research/	304,627
3	exp *health service research/	10,754
4	exp *health services research/	70,614
5	exp *research/	567,353
6	exp *research priority/	334,279
7	exp *clinical protocols/	114,557
8	exp *clinical protocol/	114,557
9	exp *clinical trials as topic/	77,005
10	exp *publications/	128,035
11	exp *publication/	128,035
12	1 or 2	304,627
13	3 or 4	70,614
14	5 or 6	567,353
15	7 or 8	114,557
16	10 or 11	128,035
17	exp *patient participation/	26,449
18	9 and 17	1,020
19	12 and 17	1,317
20	13 and 17	562
21	14 and 17	1,853
22	15 and 17	43
23	16 and 17	93

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24	18 or 19 or 20 or 21 or 22 or 23	3,124
25	(patient\$ adj2 involve\$5).ti,kw.	4,860
26	(patient\$ adj2 partner\$).ti,kw.	2,098
27	(patient\$ adj2 participat\$).ti,kw.	3,433
28	(patient\$ adj2 engage\$).ti,kw.	2,695
29	participatory research\$.ti,kw.	3,079
30	(patient adj centric\$).ti,kw.	714
31	25 or 26 or 27 or 28 or 29 or 30	16,614
32	exp drug industry/	132,384
33	(pharma\$ adj (industr\$3 or compan\$3)).ti,kw.	8,419
34	exp *drug development/	81,885
35	((drug or medic\$) adj develop\$).ti,kw.	16,977
36	32 or 33 or 34 or 35	220,365
37	24 and 36	38
38	31 and 36	147
39	exp *caregiver/	54,235
40	exp *caregivers/	54,235
41	39 or 40	54,235
42	9 and 41	91
43	12 and 41	368
44	13 and 41	1,043
45	14 and 41	1,042
46	15 and 41	36
47	16 and 41	64
48	42 or 43 or 44 or 45 or 46 or 47	2,170
49	(care\$ adj2 involve\$5).ti,kw.	839
50	(care\$ adj2 partner\$).ti,kw.	1,703
51	(care\$ adj2 participat\$).ti,kw.	836
52	(care\$ adj2 engage\$).ti,kw.	877
53	49 or 50 or 51 or 52	4,221
54	48 and 36	1
55	53 and 36	13
56	37 or 38 or 54 or 55	192
57	remove duplicates from 56	157

Explanation of search terms used:

* <term>

An asterisk is used to “focus” subject headings. This means that results will only be returned if the subject heading relates to the *main topic* of the paper.

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	If no asterisk is present then the subject heading may relate to something <i>mentioned</i> in the paper, but the focus of the actual paper itself may not be on that topic.
exp <term>	Exp before a term is used to “explode” a subject heading. As well as searching for the subject heading itself, the database will also retrieve results for narrower terms that sit beneath the subject heading in the hierarchy.
<rootword> \$<n>	\$ is used to truncate search terms. Where a \$ is used at the end of a root word, the database will search for all words that begin with the root. A number is sometimes used (e.g. \$2) to limit the number of letters after the root word. For example, participat\$2 would find participate, participates but not participatory or participation.
<term> adj<n> <term>	ADJn retrieves records that contain your terms (in any order) within a specified number (n) of words of each other.

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Appendix 3: Predefined questions for external validity

Questions asked to external individuals reviewing the case studies

1. Does this information accurately represent the project you were involved in and your specific role? Could you please comment on the accuracy of this case study?
2. In the project, what did you feel was done well, could be improved and what were the benefits to you?
3. What were your key learnings from the project?

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Appendix 4: Case study 1 – Working with patients to review a rheumatology clinical trial protocol

<p>Project title and date of completion</p> <p>A face-face meeting: Working with patients to review a Phase 4 Clinical trial protocol</p> <p>Completion: November 2017</p>
<p>What was the project aim? Brief description</p> <p>Pfizer partnered with the charity Versus Arthritis (VA), formerly Arthritis Research UK, and 9 of their patient insights partners (PIPs) to review a Pfizer-sponsored Phase 4 clinical trial protocol. The aim of the project was to understand the PIPs' experiences of living with rheumatoid arthritis (RA) and how these lived experiences can begin to inform the efforts to develop medicines. Insights were gathered around the 'patient journey' in the clinical trial and several aspects of the proposed study design, assessments, and outcome measures. Pfizer also wanted to understand what the future role of patients within research should look like.</p>
<p>Why was it important to partner with patients/carers on this project?</p> <ul style="list-style-type: none"> • It was important to partner with VA and their PIPs on all aspects of the project. • This included all the operational aspects of the project. This ensured that the meeting setup, any of the materials that were developed for the meeting (letters, agendas, pre-read materials and presentation slides) and the running of the meeting was accessible and aligned with the needs of the PIPs. • Co-designing the clinical trial protocol would ensure that the study fully considered and accommodated participants' needs. • VA perspective - the project also allowed VA to work with another organisation to share best practice and ensure the voice of people affected by RA was included from the start. As with every Patient Public Involvement (PPI) experience, we hoped to learn from the partner's approach to PPI and improve our own activities.
<p>How was the project done - what was involved and what were the processes and timelines?</p> <ul style="list-style-type: none"> • VA's Research Involvement Manager and Pfizer's UK Scientific Lead in the Medical team worked closely together to develop, plan and facilitate a workshop in which people with RA were invited. • VA assisted with inviting PIPs with suitable skills and capabilities to be part of the project. Following this, Pfizer contracts were executed with VA and PIPs. • Attendees were provided with pre-read materials explaining the purpose of the workshop, the expectations of what we were wanting to gather and achieve from the day, and what they could expect in terms of support and feedback. Attendees were also provided with key pieces of information and sections from the trial protocol that would enable more focused and informed discussions on the day. • On the day, crucial areas of review and associated questions to be addressed formed the structure of discussion. Short 5-10 minute presentations were followed by group facilitated discussion, with notes taken by facilitators. The workshop was three hours long with a tea/coffee break. • Notes were then written up and were signed off by facilitators and attendees. This information was then provided to the study's principal investigators to use to amend and enhance the study protocol. Written feedback was provided to the group.

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<ul style="list-style-type: none"> This was the first time that Pfizer UK had worked with a charity to involve patients on a clinical protocol and, as such, the setup took much longer than anticipated.
<p>What was the benefit/impact to Pfizer and to patients/carers?</p> <ul style="list-style-type: none"> For Pfizer, there were clear areas of positive impact from gaining understanding from PIPs on the delivery of the trial itself. These included adjusting patient reported outcome measures, optimising patient symptom diaries, and reducing the number of questionnaires to a practical number for patients. More broadly, the insights helped inform the focus of the clinical research plan and provided practical/logistical considerations around trial design for general inflammatory conditions. For VA, the partnership enabled the charity to support the voice of people with RA in influencing how the trial was to be carried out and how the involvement of patients could be maximised in the trial itself. It was a great opportunity to support meaningful patient involvement.
<p>What were the gaps, as identified by comparison with the PFMD PEQG tool?</p> <p><u>PFMD criteria 1: Shared purpose:</u></p> <p>A clear brief was sent to the patients (via the Research Involvement Manager at VA) explaining what the project was about and what skills, capabilities and time commitments were required to take part. Based on this, patients, via VA, replied expressing their interest and were selected. The shared purpose wasn't specifically devised for the group.</p> <ul style="list-style-type: none"> Making sure there is a clear shared purpose that is defined by the group and clearly written down for everybody to access is important and helps understanding and prevents mission creep. This could be done more effectively in future meetings. <p><u>PFMD criteria 2: Respect and accessibility</u></p> <p>The meeting was held at a familiar and convenient meeting venue (VA offices), rather than a virtual or corporate setting. The meeting start time was aligned to ensure people were not travelling during rush hour or on busy trains. Overnight accommodation was provided for those who had a long distance to travel. The meeting lasted three hours with regular breaks and was jointly run by Pfizer and VA and people were actively encouraged to participate throughout the meeting.</p> <ul style="list-style-type: none"> Writing down a code of conduct and what mutual respect should look like for all stakeholders wasn't specifically done for this project and is something to consider in the future. It would have been useful to provide this ahead of the meeting. <p><u>PFMD criteria 3: Representativeness of stakeholders</u></p> <p>VA reached out to their group of PIPs with a clear brief on what skills and capabilities would be relevant for the project. One of the attributes had to be lived experience of the condition. No further outreach was done. Patients that volunteer to work with patient organisations may not be truly representative of the general population.</p> <ul style="list-style-type: none"> For future projects, there could have been further outreach to improve diversity of the group, including ethnicity and age. Also, as this was relating to a clinical trial, it may have been useful to bring together a cross-European group of people. <p><u>PFMD criteria 4: Roles and responsibilities</u></p> <p>Roles and responsibilities were outlined in the contracts signed by PIPs. Prior to the meeting, PIPs were given an option to ask questions if anything in the information sent to them was unclear. At the start of the meeting there was also a chance to ask any further questions if any areas required clarity to ensure everybody was comfortable and happy.</p>

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- For this one-off meeting, additional follow-up to discuss roles and responsibilities wasn't required. Although, for some people, this was the first time working with a pharmaceutical company and providing clear information about the Pfizer team and their roles would have been useful and should be considered for the future.
- Prior to the meeting, being clear on the roles and boundaries between Pfizer, VA and the PIPs would have been beneficial to the group. For the future, this could be written down and sent along with any pre-read materials.

PFMD criteria 5: Capacity and capability for engagement

PIPs were provided with pre-read materials and given two weeks to read the information prior to attending the meeting. All PIPs were selected by VA and consideration was given to their experience in reviewing research proposals for VA and being comfortable and confident in this area. All materials were co-created between Pfizer and VA and accessibility and language was assessed along the way.

- There should have been more time given, as there was a lot of information to read and digest. This was dictated by the time taken to execute contracts, which took longer than anticipated due to the novelty of the activity.
- Some of this information was not lay friendly, e.g. aspects of the nature of scientific/technical language and study flow charts. Health literacy should be considered with any pre-read materials.

PFMD criteria 6: Transparency in communication

Pre-read materials (as outlined above) and the agenda were provided prior to the meeting. The team at VA coordinated the arrangements with the PIPs and ensured that they had everything they needed. As this was a short, one-off meeting, longer term communication channels were not required.

- No gaps identified.

PFMD criteria 7: Continuity and sustainability

VA and Pfizer have continued their relationship and are working collaboratively on other projects. Learnings from this project have been shared with other unrelated organisations to help further developments in patient involvement within research. Feedback was provided to the group following the workshop to inform them how their insights had been implemented.

- The feedback took longer than expected to provide, partly due to the complicated procedural requirements within pharmaceutical companies. For the future, a structured form that can easily be filled out and returned quickly would be optimal.

Learnings and improvements that could be made for future projects

- Be clear about the type of patient involvement needed. Provide clear meeting objectives in order to focus discussions.
- Be legitimate and manage expectations at the outset.
- Patients offered very valuable insights that enhanced the clinical trial protocol; partnering earlier on in the process would be more beneficial in the future.
- Language should be given thought, time and consideration and involving patients is key. Common research language, when read by a patient, can be not only unclear, but actively dispiriting.
- It is important to consider contracting language and execution of contracts in a timely manner.
- Working as equal partners (Pfizer & VA) was essential.

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Case study 1 feedback

Feel free to make comment on the above case study, particularly the identified gaps and then specifically answer the 3 questions shown below:

<p>1. Does this accurately represent the project you were involved in and your specific role? Could you please comment on the accuracy of this case study?</p> <ul style="list-style-type: none"> <i>Difficult to recall exactly as the product was conducted 3 years ago.</i> <i>Yes, generally the project is accurately described in this document. However, it wasn't clear beforehand what was exactly wanted from the PIPs, other than to input and feedback about the study design. It wasn't clear of the roles and boundaries between those attending the workshop; the agenda being an example – i.e. who had responsibility for its content, putting it together and producing it? Some of the information received was not lay friendly (aspects of the nature of scientific/technical language with text used, and study flow charts being examples) and details of the proposed study could have been more detailed.</i>
<p>2. In the project, what did you feel was done well, could be improved and what were the benefits to you?</p> <ul style="list-style-type: none"> <i>It is always good to have the opportunity to be involved and to be asked our opinion as people with arthritis. That being said, the number of points we raised and insights we had made me think how much more impact we could have had if people with arthritis had been part of the conversations at the outset. Then we'd have been shaping the project as partners rather than being asked once it was underway.</i> <i>I enjoyed the opportunity to learn more about how a major pharmaceutical company undertakes research and I commend Pfizer for actively partnering with a charity to hear our thoughts and where they could be making changes based on that feedback.</i> <i>The arrangements leading to the day, and how the day ran, in terms of agenda and subjects covered with opportunity for asking questions was good.</i> <i>The background information supplied before the day could have been better, more explanation of the project holistically (e.g. demographics of proposed participants, so we could think about diversity and representation). This all becomes important because Patient and Public Involvement (PPI) can exist at all stages of the research cycle and it should. And all this information should be laid out and made available pre-meeting using good lay language or 'plain English', with science/technical aspects and acronyms and abbreviations well described and with ease of 'lay' reading and understandable charts.</i> <i>It's also an opportunity to have breakout round-table sessions, even with a small group which could have been beneficial as this allows for separate discussions, greater engagement with others who might struggle in a larger group, and it allows conversations to develop at different tables and at different levels, which don't always happen in a single group format due usually to time constraints and agenda timetabling.</i> <i>Research dissemination to the public often gets forgotten and research can end up behind a paywall. Getting research with findings into the public domain is an area patients can help with to ensure understanding, marketing and take-up of a product in terms of risk and benefits.</i>
<p>3. What were your key learnings from the project?</p>

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- *How much people with arthritis have to contribute if asked! And that our views were valued and led to improvements.*
- *Great collaboration creates a number of aims, goals and benefits, such as mutual and shared values achieved through various parties working together. It breaks down barriers between 'pharma' and patients as well as in a wider setting, with healthcare professionals.*
- *It's about engagement, collaboration and opening up with transparency about its work and to engage and utilise the skills, the experience and knowledge, plus with the stories to tell, after all what are its end users – 'patients'. They should have a central, pivotal role. They have a lot to give, share and contribute. In many cases 'they have been around a bit'. And it's about collaboration and co-production. It's about working with the patients being the key stakeholder through receiving treatment, and not for them.*

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Appendix 5: Case study 2 – Working with young people to review a dermatology clinical trial

<p>Project title and date of completion</p> <p>A face-to-face engagement between GenerationR Liverpool Young Person's Advisory Group (YPAG) and Pfizer to review a clinical trial testing a new, as well as existing, medicine for the treatment of eczema in children and adults.</p> <p>Completion: February 2018</p>
<p>What was the project aim? Brief description</p> <p>The aim of the project was to obtain the thoughts, comments and concerns of the YPAG with regards to the trial, including the amount of visits scheduled and burden of subject assessments, as well as thoughts around the informed consent documentation (ICD) content.</p>
<p>Why was it important to partner with patients/carers on this project?</p> <p>It was extremely important to understand, from a young person's point of view, their thoughts around the study design and timings of assessments, as well as the proposed subject consent/assent forms, and in particular the language and proposed pictures to be used in the study documents.</p>
<p>How was the project done - what was involved and what were the process and timelines?</p> <ul style="list-style-type: none"> • The project involved working with a Senior Patient and Public Involvement (PPI) Manager/Coordinator of GenerationR YPAG and her team to organise and facilitate the engagement and support the creation of a set of slides in layman's terms. • From Pfizer, the engagement was attended by the clinician who was writing the clinical trial protocol and the head of patient recruitment. • The project took approximately 8 weeks from initially contacting the YPAG to having the patient engagement.
<p>What was the benefit/challenges for Pfizer and to patients/carers?</p> <p>Benefits:</p> <ul style="list-style-type: none"> • For Pfizer, the benefits included understanding what to include in the patient assent forms, as the young people had a lot of valuable feedback as to what they would like to see included. For example, when shown the cartoon for the assent forms, the young people made it clear that they would find that patronising and would prefer to see photographs instead. • The young people benefitted from being able to provide valuable insights that would shape a commercially-sponsored protocol, which was the first for them from a large pharmaceutical company, as historically the group had reviewed only academic protocols. <p>Challenges:</p> <ul style="list-style-type: none"> • Challenges for Pfizer included being able to create a slide deck to describe the study design in plain and clear language suitable to be understood by a group of young people. • Challenges for the PPI Manager was that it took longer than expected to develop the session as it involved gaining clarity on what the team wanted from the young people, reviewing various versions of the slide deck – which entailed numerous conversations – in advance of the meeting, and finding a suitable date for the session to take place that was convenient for all. • The PPI Manager also needed to make sure the payment and reimbursement processes were correctly in place.
<p>What were the gaps, as identified by comparison with the PFMD PEQG tool?</p> <p>PFMD criteria 1: Shared purpose</p>

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The YPAG team ensured the young people were prepared ahead of the engagement and were aware of what was expected to occur at the meeting. The Pfizer attendees had prepared a slide deck and questions for the young people.

- There wasn't a specific shared purpose agreed by the group. It may have been useful to share this prior to the meeting, so it was accessible. This is something that should be incorporated for future meetings.

PFMD criteria 2: Respect and accessibility

The meeting was facilitated by the experienced PPI team, who tried to ensure that every young person was able to voice their feedback. An agenda explained the format of the engagement and this already formed group had its own codes of conducts in place.

- A formal written code of conduct of what the group could expect from Pfizer was not relayed prior to the meeting and this could be considered for future meetings.

PFMD criteria 3: Representativeness of stakeholders

The study being discussed was an eczema study in children and adults and therefore it was important to get the young person's perspective on the study and informed consent design. Nine members of the group attended the meeting, with a good mix of girls and boys between the ages of 11-19, and the group was racially diverse. One young person had experience of eczema as a young child. Some group members had either a family member or a close friend (currently on trials) with eczema.

- Careful thought was given to the representativeness of the group. However, all were recruited through the YPAG which could limit this. For the future, wider outreach should be considered, alongside having sufficient time to do this.

PFMD criteria 4: Roles and responsibilities

The preparation and facilitation of the meeting by the GenerationR team meant that the young people and the Pfizer attendees knew exactly what was expected of them at the engagement.

- All requirements were met for this criterion.

PFMD criteria 5: Capacity and capability for engagement

A lot of effort and energy went into creating a set of slides that was in clear and plain language. This was the first patient engagement experience that the Pfizer individuals had participated in and therefore they learned a lot from a skilled PPI team on what to focus on when creating a set of slides, to give a high level overview of the study design and assent forms in language that could be understood by a group of young people. Throughout the engagement, the GenerationR team verified that the young people understood what was being explained to them.

- The Pfizer team's initial materials were not written in a lay friendly manner and the learnings from the PPI team at GenerationR have been translated into future projects. In addition, for future engagements, consideration needs to be made for young people/patients with audio-visual problems that may require additional support on top of making the slides in clear and plain language.

PFMD criteria 6: Transparency in communication

The slide deck was shared ahead of the meeting with the young people. The GenerationR team took and provided notes during the meeting, and shared with the team, which were very helpful in ensuring that all the fabulous points made by the young people were captured.

- All requirements were met for this criterion.

PFMD criteria 7: Continuity and sustainability

Following the meeting, a feedback form was completed on how the engagement was received by the Pfizer colleagues and a thank you letter outlining the considerations the Pfizer colleagues were able to make was written and sent to the YPAG co-ordinator to pass onto the young people.

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<ul style="list-style-type: none"> Changes made to the design of the assent form were made according to the feedback provided by the young people. However, mainly due to time there wasn't a validation step performed to confirm that this implementation was consistent with the feedback received. Future engagements will benefit from this validation step being implemented.
<p>Learnings and improvements that could be made for future projects</p> <ol style="list-style-type: none"> An appreciation of never underestimating what a group of young people can contribute to supporting the design of a clinical trial protocol and assent forms was a key takeaway from this early project. The key role of the PPI team in encouraging all researchers to feedback any impact that the young people's views had on the trial design and ultimately how this impacts on recruitment and retention, and the trial participants experiences of participating in the trial.

Case study 2 feedback

Feel free to make comment on the above case study, particularly the identified gaps and then specifically answer the 3 questions shown below:

<p>1. Does this accurately represent the project you were involved in and your specific role? Could you please comment on the accuracy of this case study?</p> <ul style="list-style-type: none"> Yes
<p>2. In the project, what did you feel was done well, could be improved and what were the benefits to you?</p> <ul style="list-style-type: none"> <i>This was the first time someone from industry came to our group with a study plan (protocol) asking for our opinions. This was presented really well so we could understand the study design and we felt able to contribute to a discussion and ask questions of the scientific team.</i> <i>It was great that the scientific members of the team visited the group personally so we could ask questions directly to them as they knew the study inside-out.</i> <i>We felt listened to and valued.</i> <i>We were thanked for taking part in the session.</i>
<p>3. What were your key learnings from the project?</p> <ul style="list-style-type: none"> <i>We didn't hear straight away about whether the study went ahead and whether our feedback helped with study recruitment, so we were not sure whether our opinions mattered. Early feedback would be helpful.</i>

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Appendix 6: Case study 3 – Working with an NIHR patient focus group to review two gastroenterology clinical trials

<p>Project title and date of completion</p> <p>A face-to-face engagement between a National Institute Health Research (NIHR) Patient Focus Group and Pfizer to review two ulcerative colitis (UC) studies.</p> <p>Completion: August 2019.</p>
<p>What was the project aim? Brief description</p> <p>The aim of the project was to obtain the insights, comments and concerns of the patient focus group with regards to the two proposed studies.</p>
<p>Why was it important to partner with patients/carers on this project?</p> <ul style="list-style-type: none"> • It was extremely important to understand, from a patient's point of view, their thoughts around the design of the two studies and the proposed medication. • All patients had lived experience of UC and were on different points of the disease spectrum: some with quiescent, well-controlled disease and some with flare-ups/a relapse. For some patients, UC is an invisible disability. Patients used different words/phrases to describe the burden of living with UC and this was important to understand. • Patients also had varied experience of participating in clinical trials which ranged from extremely positive to negative. • UC patients are usually self-disciplined and seek opportunities to further medical research into the understanding of UC and future treatment, even if participation in a clinical trial comes with some inconvenience or they do not necessarily help themselves but rather future generations.
<p>How was the project done - what was involved and what were the processes and timelines?</p> <ul style="list-style-type: none"> • The project involved working with a Business Development Officer at the NIHR to schedule the focus group engagement and recruit the relevant patients. Recruitment for the event started over a month in advance. Outreach was conducted via the NIHR Local Clinical Research Networks, a UK patient organisation, and an ad was placed on peopleinresearch.org, which is a go-to website for public contributors. • The focus group was facilitated and attended in person by the NIHR representatives and the Pfizer Study Optimisation Lead, who supported the set up and facilitation of the meetings. The study clinicians and operational colleagues from both the UC studies attended virtually. • Five patients living with UC attended the session. • The time from initially contacting the NIHR to having the patient engagement was around eight weeks.
<p>What was the benefit/impact to Pfizer and to patients/carers?</p> <p>Benefits:</p> <ul style="list-style-type: none"> • In general, the benefits to Pfizer were largely around getting a greater understanding of what it is like to live with UC, thoughts on the study design (e.g. acceptability of a repeat colonoscopy within the course of the study), as well as where the patients would look for information if they were interested in taking part in a clinical trial. The Pfizer attendees were all very grateful for the feedback received and all felt they learned a lot from the engagement. • Having a diverse mix of participants (even geographically) with different experiences and levels of knowledge and understanding regarding clinical trials was very beneficial. This was demonstrated in the feedback provided and needed consideration for how someone who is completely naïve to research may consider this and how best to share key information to support access and retention during the trial.

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- Pfizer and patients valued the direct conversation between the Pfizer team and patient participants. The latter are very motivated to help and seek opportunities for engagement/involvement with pharma companies.
- Other benefits for the patients included being listened to and being able to provide open and honest feedback about the study. The feedback received gave greater depth of consideration for obviously invasive and disruptive procedures, such as the proposed colonoscopy and the use of the laxative prior to the procedure, especially when considering this in 'public' environments such as an office space. It transpired that, to patients, the relationship with a treating consultant is absolutely crucial as patients pay heed to their advice with regard to which clinical trials to participate in. Consultants are also the primary contact to enquire about ongoing and future trials.
- Different patients value and prioritise different health outcomes, which may not necessarily align with the ones researchers have in mind e.g. resolving incontinence vs. fatigue/pain.
- Working with the NIHR ensured all formal requirements (e.g. travel and accommodation) were met to support participants. Knowing that the NIHR was involved as an intermediary gave patients greater peace of mind.

Challenges:

- These were mainly logistical; the NIHR were able to open their offices over the weekend to support this activity, as a neutral and geographically convenient location. However this did bring some unforeseen challenges including:
 - Not having adequate business support for IT systems, possibly compounded by using NIHR & Pfizer technology together and firewall issues.
 - Due to limited NIHR staff on site, there was limited support to manage the logistics and hospitality during the meeting to ensure participants were comfortable throughout. For example, access to toilets was also more challenging than it should have been.
- Whilst it was seen to be a benefit to share the patient notification sheets with participants on the day to discuss this and ensure their understanding and agreement, in hindsight, we should have circulated these either electronically or physically to participants to give them earlier visibility of this.

What were the gaps as identified by comparison with the PFMD PEQG tool?**PFMD criteria 1: Shared purpose**

Patients signed a patient notification form which explained what was expected of them with regards to time commitment and the reimbursement for that engagement. As the meeting was only 1.5 hours long, and the agenda of the meeting was laid out at the beginning of the engagement, there was not a need to have a checkpoint in the engagement.

- No gaps identified for this criterion.

PFMD criteria 2: Respect and accessibility

The timing of the engagement, being a Saturday afternoon, was scheduled to ensure it was at a suitable time for the patients, taking into consideration their commitments to their work/family life. Travel to the NIHR offices was arranged in advance, where possible, by the NIHR. Facilitation of the engagement was done to ensure that every patient was able to voice their feedback, this was enabled by having a small group. One of the advantages of the virtual insight sessions is that participants have access to additional functionality to share their thoughts in a way which suits them and makes them more comfortable, such as the use of comments rather than speaking directly.

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- The agenda explained the format of the engagement, a formal written code of conduct and what to expect from Pfizer and the meeting was not relayed prior to the meeting. This may help with some challenges faced for future meetings.

PFMD Criteria 3: Representativeness of stakeholders

The five patients were of different genders, ethnicity, and age, all living with UC with a variety of experience of clinical trials. It needs to be noted, however, that the NIHR are at times limited by uptake for patient engagement activities which makes ensuring a diverse mix of participants a challenge. There is a national (NIHR) piece of work to tackle this and this is also being seen across the life sciences sector with other equality, diversity and inclusion (EDI) activities. This is a challenge in all patient and public involvement (PPI) activities to involve underrepresented groups.

- The information was not collected from the patients themselves, which in future may be something that should be considered to ensure that the group of patients are as diverse as possible. More time to do further outreach and incorporating learnings from the EDI activities underway will help further fill this gap.

PFMD criteria 4: Roles and responsibilities:

All patients signed a patient notification form which explained what was expected of the panel when they arrived at the engagement meeting. Patients were also informed of the nature and purpose of the discussion as part of the outreach and engagement activities.

- This information was not however relayed verbally to the patient group prior to the start of the meeting to ensure that nobody had any concerns; this could have been useful to ensure everyone was clear on what they had signed.

PFMD criteria 5: Capacity and capability

Participants were recruited to take part in the activity, with varying experiences, but all living with UC. A set of slides that were used during the meeting were created and written in layman's terms. The slides were not sent to the patients ahead of the meeting, however, and this lack of pre-meeting reading materials may have contributed to making the discussion more spontaneous as participants had not had a chance to prepare their answers in advance or overthink things.

- It would have been beneficial for the patients to have reviewed the slides before the meeting. Pre-read slides are now sent ahead of all patient engagement sessions.

PFMD Criteria 6: Transparency in communication and documentation

Feedback was provided to the group. A thank you letter was sent to the patients following the engagement informing them of the changes that were being considered to the protocols and associated learnings.

- Feedback could be enhanced in the future. There is a consensus from those writing this case-study that feedback shared regarding what has and has not changed is incredibly valuable, demonstrating the impact from the discussion to the participants and that they have been listened to. Regarding addressing comments where changes to clinical trial documents have not been implemented, this should also be addressed so it does not feel ignored and can be justified if these have been disregarded as not feasible, ethical, or scientifically important, etc. It is important to share more information with participants rather than less, otherwise you risk disengaging participants from future activities and not treating them as partners.

PFMD criteria 7: Continuity and sustainability

The minutes from the meeting were shared with the Pfizer study team to benefit a future inflammatory bowel disorder study in this patient population. In fact, Pfizer is currently planning for a large UC study in which this feedback is being reviewed to improve the design of that study and associated documentation.

- No gaps identified for this criterion.

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Learnings and improvements that could be made for future projects

- The Pfizer team learned a lot from this engagement, which helped shape the design of the study, the provision of recruitment support material, and allowed them to gain a greater understanding of where patients may look to join a clinical trial.
- Future engagements could benefit from allowing the patients to review materials ahead of the engagement session.
- Since the pandemic, patient engagements have moved to a virtual platform such as Webex, meaning engagements have worked better than having some attendees being face-to-face and some virtual.
- Patients have fed back that they enjoy meeting other patients living with the same condition and getting other people's perspective on the indication.
- The experience for the participants could be different between an organised group that know each other and have a rapport versus a disease specific group that have not met. This rapport and familiarity may or may not increase confidence to speak up and needs to be considered.

Case study feedback was not possible for this project due to a lack of availability of project group members.

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Appendix 7: Case study 4 – Working with parents and carers to review dermatology study documentation

<p>Project title and date of completion</p> <p>A virtual engagement between the National Institute for Health and Care Research (NIHR) Alder Hey Clinical Research Facility Parent and Carer Research Forum and Pfizer to review an eczema study informed consent document for infants aged 3-24 months.</p> <p>Completion: August 2020.</p>
<p>What was the project aim? Brief description</p> <p>The aim of the project was to obtain the thoughts, comments and concerns of the parent/carers group with regards to the proposed study and informed consent document.</p>
<p>Why was it important to partner with patients/carers on this project?</p> <p>In this example, it was extremely important to understand, from a parent/carers point of view, their thoughts around the design of the study informed consent documentation, as this study was planning to enroll very young patients between 3-24 months of age.</p>
<p>How was the project done - what was involved and what were the processes and timelines?</p> <ul style="list-style-type: none"> • The project involved working with a Senior Patient and Public Involvement (PPI) Manager/Coordinator of the Parent and Carer Research Forum and her team to schedule the PPI activity and how it would run. • The PPI Manager worked with Pfizer to advise on the layout of the slide deck covering the study design, to ensure it was written in layman's terms. • The meeting was attended by the Pfizer study team, including clinicians who were writing the informed consent document, the study manager, and study optimisation team, who supported the setup and facilitation of the meeting. • The PPI Manager recruited seven carers who attended the two sessions, six of whom had direct experience looking after a child with eczema and one who had a nephew with the condition. • From initially contacting the PPI Manager, it took eight weeks to complete the project.
<p>What was the benefit/impact to Pfizer and to patients/carers?</p> <p>Benefits:</p> <ul style="list-style-type: none"> • The benefits were largely around the development of the study and informed consent documentation. The carers had a lot of valuable feedback around various aspects of the study design and what they would wish to see incorporated in the informed consent form and how it should best be presented. For example, a detailed discussion focused on the blood draws and how the blood would be taken, i.e. venously or from a heel prick. The carers advised Pfizer to make it very clear in the informed consent document what the parent/carers is to expect as to how the blood is to be drawn and whether a line could be left in the arm of the infant in between the blood draws. The carers were also very receptive to the idea of having home health support, especially in the current pandemic. <p>Challenges:</p> <ul style="list-style-type: none"> • Challenges on Pfizer's side included being able to create a slide deck to describe the study design from the protocol documentation in plain language, suitable to be understood by a group of carers. A challenge for the PPI team was making sure a convenient time was organised to hold the meeting around the carers' needs.

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What were the gaps as identified by comparison with the PFMD PEQG tool?**PFMD criteria 1: Shared purpose**

Carers signed a patient notification form which explained what was expected of them with regards to time commitment and the reimbursement for that engagement. At the beginning of the second engagement, Pfizer gave an overview of the key take-away messages from the first session, to ensure that the carers had the opportunity to clarify or add to the understanding.

- There wasn't a validation step after the second engagement, which future engagements would have benefited, even if it had been written in an email and sent to the carers.

PFMD criteria 2: Respect and accessibility

The timings of the two virtual Webex sessions (early evening) were made to ensure they were at a suitable time for the carers, taking into consideration their commitments to their work/family life. It was made clear to the carers that they were able to leave and attend to their family at any time during the engagements. Facilitation of the engagement was done to ensure that every carer was able to voice their feedback.

- Although an agenda explained the format of the engagement, a formal written code of conduct around what could be expected from Pfizer was not relayed prior to the meeting.

PFMD criteria 3: Representativeness of stakeholders

The carers were part of the NIHR Alder Hey CRF Parent and Carers Research Forum and included seven female carers who had experience of caring for a child with eczema. Five of the carers considered themselves White British and two Black British (AfroCaribbean).

- No male representatives were part of the panel, which could have proved beneficial from a diversity standpoint.
- Carers that volunteer to join a carer focus group may have a certain demographic and thus not be truly representative of the general population. This could be further considered for future projects.

PFMD criteria 4: Roles and responsibilities

The carers had signed the contract, however there wasn't any subsequent confirmation that the panel remained clear on their role and responsibilities.

- For future meetings a simple question to confirm this at the beginning of each engagement would have supported this criterion.

PFMD criteria 6: Transparency in communication

A set of slides written in layman's language and the proposed study informed consent document was provided to the carers ahead of the engagements.

- For the future, following feedback, video/animations or a recording of the presentation sent across with a voiceover may have worked better.
- Feedback could have included the impact of discussions on the trial protocol.

PFMD criteria 7: Continuity and sustainability

- Having a sustainable process to feedback specific study details – that are aligned with Pfizer processes – to those involved in the project, is something that needs to be worked on for future projects.

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Learnings and improvements that could be made for future projects

1. The Pfizer team learned a lot from this engagement which helped shape the design of the study and informed consent form.
2. Having the two engagements split to cover the study design and the informed consent form meant the team could focus in on one topic at a time at the engagement sessions and have a meaningful discussion.
3. It would have been preferable to give the carers/parents slightly longer to review the slide deck and the proposed informed consent documentation and in the future, this is something that Pfizer can ensure on.

Case study 4 feedback

Feel free to make comment on the above case study, particularly the identified gaps and then specifically answer the 3 questions shown below:

1. **Does this accurately represent the project you were involved in and your specific role? Could you please comment on the accuracy of this case study?**

- *Yes very accurately described.*

2. **In the project, what did you feel was done well, could be improved and what were the benefits to you?**

- *There was a diverse mix of parents from across the UK with different experiences of having a child with eczema which was good to hear, and as a parent we truly felt our opinions were important.*
- *The team explained the study really well and we felt we could ask questions if we didn't quite understand certain aspects of study design.*

3. **What were your key learnings from the project?**

- *That pharma companies are willing to involve and listen to patients and families.*
- *That we would accept future invitations to support industry-led studies.*
- *Being informed of progress with study recruitment and if possible future findings would be really interesting.*

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Appendix 8: Case study 5 – Independent medical grant call: *Quality Improvements in Rheumatology Practice: Delivering change for patients*

<p>Project title and date of completion</p> <p>Independent Medical grant call: <i>Quality Improvements in Rheumatology Practice: Delivering change for patients</i>. https://www.cybergrants.com/pfizer/UKLocalRFPs/QI_in_Rheumatology_Practice-Delivering_Change_for_Patients.pdf</p> <p>Completion: November 2021.</p>
<p>What was the project aim? Brief description</p> <p>Pfizer partnered with Versus Arthritis (VA), and three of their patient insight partners (PIPs) alongside two clinicians to develop a Competitive Quality Improvement grant programme, focusing on improving patient care and outcomes in various musculoskeletal (MSK) conditions. The aim of the programme was to fund projects that lead to measurable improvement in healthcare services and the health status of individuals. The benefit to patients and implementation into the National Health Service (NHS) had to be very clear in all submissions.</p>
<p>Why was it important to partner with patients/carers on this project?</p> <p>It was vitally important for the benefit of the end-user to co-create the grant scheme from start to finish. This was to ensure that the research priorities, request for proposals (RFP), and projects funded would be aligned with the needs of the NHS and those living with MSK conditions.</p>
<p>How was the project done - what was involved and what were the processes and timelines?</p> <ul style="list-style-type: none"> • Pfizer organised an initial meeting with VA to determine if there was a shared interest in this area. • Following successful discussions, Pfizer and VA set up a project plan, which included working with the VA Research Programme Manager and their Research Involvement Officer to conduct the project. VA were involved with identifying panel members (patients, carers and clinicians) and Pfizer executed contracts with VA and recruited individuals. • All stakeholders were involved with all aspects of the project, including designing the RFP, chairing and participating in the external grant review panel (ERP) which reviewed the submitted proposals, and being part of the grant decision-making process. The funding awarded was a grant, independent of both Pfizer and VA. • The project started in March 2020 but was paused due to the COVID-19 pandemic until late 2020 and was completed in November 2021.
<p>What was the benefit/impact to Pfizer and to patients/carers?</p> <p>Pfizer collaborated with patients and carers, with aligned skills and expertise and lived experience of the conditions the grant call focused on. This ensured that there was a patient-prioritised research agenda and the research questions and outcomes were relevant to patients and translatable into the NHS.</p> <ul style="list-style-type: none"> • Inclusion of VA perspectives allowed the call to be generalisable to the wider community. • The clinicians involved in the project reported that they found the process to be inclusive and had the opportunity to listen and learn from the PIPs, which will help inform future practice. • The learning gained will be utilised to optimise ways of working in future projects to enable more patients to be at the centre of decision-making. <p>Versus Arthritis was able to influence the research call to ensure it aligned with their priorities, which were set in collaboration with patient partners and healthcare professionals.</p>

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<ul style="list-style-type: none"> VA PIPs were involved from the start of the process in shaping the grant call, and having a PIP co-chair on the review panel ensured that the end-user voice was heard and taken in to account equally.
<p>What were the gaps as identified by comparison with the PFMD PEQG tool?</p> <p><u>PFMD criteria 1: Shared purpose</u></p> <p>A clear brief was sent to the PIPs (via the Research Involvement Officer at VA) explaining what the project was about and what skills, capabilities and time commitments were required to take part. Based on this, patients replied via VA expressing their interest. A patient panel meeting was held specifically with the PIPs to explain the project detail, estimated timelines, and answer any concerns or questions they had on the RFP drafts and processes.</p> <ul style="list-style-type: none"> There was not a clear shared purpose defined by the group and clearly written down for everybody to access and re-visit regularly for the project. This would have been particularly important for this project, which was conducted during the pandemic, as things took longer than usual to complete, and focus may have been lost. <p><u>PFMD criteria 2: Respect and accessibility</u></p> <p>An inclusive approach was adopted and this was discussed with the group, including accessibility to information, convenience of meetings (times and platforms), equal share of voice and clarification of rules relating to the panel member voting system.</p> <ul style="list-style-type: none"> There was not a specific code of conduct for this project written down, including what mutual respect was and what expectations from stakeholders were. Having this accessible on the digital platform would have been useful. <p><u>PFMD criteria 3: Representativeness of stakeholders</u></p> <p>The group chosen to be part of the project was diverse, including clinicians with a specialist interest, a VA representative and PIPs with defined skills and capabilities, representing the different MSK disease areas.</p> <ul style="list-style-type: none"> There were limitations on the size of the group and numbers of individuals on the expert review panel. For future projects there should be further outreach to improve diversity of the group, including ethnicity and age, to achieve inclusivity for all groups. <p><u>PFMD criteria 4: Roles and responsibilities</u></p> <p>Roles and responsibilities were outlined in the contracts. There was also a follow up meeting at the outset with the different stakeholders, VA, patient representatives and clinicians to ensure that everybody understood what was expected of them. Following this, there was a wider stakeholder meeting to address this as a whole group. All meetings had agendas and follow up notes/comments.</p> <ul style="list-style-type: none"> No identified gaps for this quality criterion. <p><u>PFMD criteria 5: Capacity and capability for engagement</u></p> <p>It was clearly outlined and agreed at the outset how communication would happen amongst the group. An online group was set up to share documents, comment and chat with each other. This worked really well for the group. The people chosen to work on the project had previous experience of chairing meetings and they could provide their insights and expertise to the group in terms of running panel meetings. The shift to virtual engagement was greatly accelerated by the pandemic and thus the sharing of project details, documents and timelines was essential.</p> <ul style="list-style-type: none"> No identified gaps for this quality criterion. <p><u>PFMD criteria 6: Transparency in communication and documentation</u></p> <p>There was an agreed way of communication amongst the group via the online platform. Regular communication between meetings and sharing of documentation in a timely manner were really important to ensure everyone had time to review.</p> <ul style="list-style-type: none"> Methods and times of communication could have been agreed earlier on in the project, in order to assist with timelines. Timeline documents were later shared with the ERP and this assisted with

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<p>progressing the workflow. This was challenging at the time due to the pandemic and something that should be implemented for future projects.</p> <p>PFMD criteria 7: Continuity and sustainability</p> <p>The learnings from this project have been shared widely within Pfizer for others to learn from.</p> <ul style="list-style-type: none"> • No identified gaps.
<p>Learnings and improvements that could be made for future projects</p> <ol style="list-style-type: none"> 1. The pandemic posed several challenges to keep people motivated and involved when dealing with other demands on their time. It was important to be realistic and flexible with timings and make sure that everyone was kept up to date to avoid delays. 2. Cross-stakeholder inclusive working was paramount to the success of the project and should be thought about at the planning stage early on in a project lifecycle. 3. The project group considered that including sections on patient involvement in the RFP, should be a mandatory for all relevant grant calls. 4. It was essential that everyone within the ERP input into the RFP to make sure we are capturing the alignment to patients needs and the NHS and were working in collaboration. It was imperative that everyone's voice and feedback was heard in aligning the RFP. 5. A virtual platform was agreed to be used amongst the group very early on in the project and this allowed equitable access for all to all project-related documentation. IT issues need to be resolved, or alternatives options put in place too. 6. There was more time involved for the project team than had been initially considered, so thinking carefully and scoping out realistic and achievable time requirements is important. 7. There were separate meetings for PIPs without the rest of the panel so they could ask specific questions about the process and their place within it. This enabled people to feel more confident in their roles. 8. Patient co-chairs should be the norm and gives the other patients on the panel a feeling of parity of opinion.

Case study 5 feedback

Feel free to make comment on the above case study, particularly the identified gaps and then specifically answer the 3 questions shown below:

<p>1. Does this accurately represent the project you were involved in and your specific role? Could you please comment on the accuracy of this case study?</p> <ul style="list-style-type: none"> • <i>The case study reflects in general terms the PIP engagement within the project. Clear definition is required as to many individuals a patient/carer is identical to a PIP which is not always the case.</i>
<p>2. In the project, what did you feel was done well, could be improved and what were the benefits to you?</p> <ul style="list-style-type: none"> • <i>The project was very positive and a fast-moving learning and developmental process. It allowed me to critically reflect and analyse in a beneficial manner my current practice relating to projects in general and PPI in specific terms.</i> • <i>The overall success of the project was reliant on 3 quite defined factors: the selection of the panel with the knowledge and expertise to positively impact meaningfully on the task in hand, the Pfizer staff whose generosity of spirit, patience, guidance and courtesy was a delightful, shared experience, those individuals should be commended for their professional and personal</i>

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approach to the task in hand. Significantly the approach of the professional medical panel members in levelling out and welcoming the lay members as not only equals, but also respecting their background and experience as being on occasions greater than their own.

- *Having a patient co-chair was a really important aspect of the project.*
- *The panel as a whole was a fantastic group, everyone brought something unique to the table and everyone's voice was heard and respected. Having the PIPs well represented proportionally in the group added weight to our communal voice.*

3. What were your key learnings from the project?

- *It was important to have that central point person to coordinate and field all of our queries.*
- *Levels of PPI involvement in the applications was very variable or sometimes non-existent. It may be beneficial for the inclusion of a PPI checklist, to determine appropriate engagement levels as against funding criteria.*
- *The PPI requirement must be a pre-requisite in future projects (not just a consideration as detailed on their case study!). This also should be included in the scoring criteria.*
- *Time should be considered carefully at the start of the project. With a project of this kind, depending on submissions, it's difficult to calculate from the outset the amount of time patients and carers will need to commit.*
- *Highly effective personal time management skills are essential in this time critical project model.*
- *Requirement for a real-life working definition of public involvement. Is it NHIR definition, parent/carer, PPI and is this separate to charity engagement?*
- *Requirement for lay reviewers to have access to medical staff/researchers to explain as necessary the current medical practice and terminology in use for clarification purposes.*
- *There was a need to have adequate time to review proposals. This will allow meetings to be more productive and provide people space who like to reflect on information.*
- *More IT support/earlier intervention would be good for future projects.*
- *Overall, it was a fantastic opportunity that I feel privileged to be a part of– the whole panel worked extremely well as a unit and I feel that we have approved some very worthwhile projects. I would welcome another opportunity to participate in a Pfizer project again and hope that they continue to improve and refine the process on the next project and beyond.*

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Appendix 9: Glossary of terms

Carers – Anyone, including children and adults who looks after a family member, partner or friend who needs help because of their illness, frailty, disability, a mental health problem or an addiction and cannot cope without their support. The care they give is unpaid. When we refer to carers in this document, this is inclusive of both adult and young carers (from [NHS commissioning » Who is considered a carer?](#)).

Clinical trial – A method of comparing the effects of one type of treatment to another in order to assess how well a drug works. They may involve a mixture of patients or healthy volunteers.

Engagement – When researchers share information with people, such as on social media or at open days.

External validity – When researchers use a second method, unrelated to the first method, to make sure that the results are fair and accurate.

Involvement – When people are actively involved in helping design or carry out research.

Medicines development – The process of bringing new drugs or medicines to the market, so patients can eventually have access to them.

Participation – When people take part in research, such as completing a survey or being an active participant in a clinical trial.

Patient and Public Involvement (PPI) – When patients, carers and the public are included in research activities.

Patients – A person receiving healthcare services from healthcare professionals.

PFMD PEQG tool – the Patient Focused Medicines Development Patient Engagement Quality Guidance tool is a tool for measuring if a PPI project was done in a way that is helpful and meaningful.

PPI partners – The patients, carer or members of the public who are involved in a particular research project.

Pharmaceutical company – Can also be called a drug company and is a profit-making business that researches, develops, and sells drugs, most commonly in the context of healthcare. They can deal in generic and/or branded medications.

Pharmaceutical industry – the collection of all pharmaceutical companies that discover, develop, produce, and sell drugs or pharmaceutical products for use as medicines for patients, with the aim to cure them, vaccinate them, or alleviate the symptoms of diseases and medical conditions.

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Phase 1 clinical trial – Helps researchers understand the safety of a study medicine. Usually involves around 20-100 participants and lasts around 1 week to several months (from [Pfizer » How clinical trials work](#)).

Phase 2 clinical trial – Helps researchers better understand how well the study medicine may work for the condition being studied, and the side effects that may occur. Usually involves several hundred participants and lasts around 1-2 years on average (from [Pfizer » How clinical trials work](#)).

Phase 3 clinical trial – Helps researchers determine whether the study medicine is safe and effective for people with that condition. Usually involves several hundred to several thousand participants and takes around 1-4 years on average (from [Pfizer » How clinical trials work](#)).

Phase 4 clinical trial – Long term clinical studies designed to better understand the effects of an approved medicine over time. Usually involves several thousand participants and takes more than a year (from [Pfizer » How clinical trials work](#)).

Protocol – a statement of rules, guidelines or instructions explaining the correct procedure or process to follow.

Real world data (RWD) – Any data that is generated outside of a clinical trial, relating to a patient's health status and/or the delivery of healthcare. This is routinely collected from a variety of sources, such as health records, product and disease registries, and patient-generated data.

Real world evidence (RWE) – the clinical evidence regarding the usage and potential benefits or risks of a medicine derived from analysis of RWD. RWE can be generated by different study designs or analyses, such as randomised trials, including large simple trials, pragmatic trials, and observational studies (prospective and/or retrospective).

Research study – A scientific study that sometimes includes processes involved in health and disease. For example, clinical trials are research studies that involve people.

Retrospective quality assessment – When researchers look at a previous project or activity to find out how well it was done.

Shared purpose – A clear definition understood by all participants of a project, which accurately describes why everybody is involved in the project and what they want to achieve by being involved.