The PFMD
Book of Good Practices

1st edition I 2018

@The_Synergist
@PFMDwithPatient

#PEQualityGuidance
#PEQualityCriteria

www.PatientFocusedMedicine.org

May 2018

Message from PFMD

Dear reader,

Patient Focused Medicines Development (PFMD) was established in 2015 out of a need that was expressed by many stakeholders in various roundtable discussions. This need was eventually translated into a dedicated organisation that aims to drive systematic patient engagement and involvement forward in the research, development and delivery of medicines.

Today we are very proud that you are reading the Book of Good Practices, as this too was born from a need expressed by many stakeholders. As patient engagement is becoming a norm instead of an exception or a one-off practice, there was a need to share knowledge about

- how other organisations have involved patients in their activities,
- what can be considered as high quality patient engagement, and
- how can different organisations reach the level of patient engagement that is both meaningful to patients but also to the research and development processes so that the output or outcomes will serve the end users better.

The PFMD Patient Engagement Quality Guidance, that was launched in 2018, introduces 7 Quality Criteria for good patient engagement that can be used to plan patient engagement activities, or to assess the level of patient engagement in ongoing or completed activities. Where the Patient Engagement Quality Guidance serves as a tool to help you to do patient engagement, the Book of Good Practices serves as a set of real life cases from a variety of organisations, that illustrate in detail how they have done it. These cases have been chosen from a big pool, assessed by an external group of reviewers and chosen to be included because they exemplify exceptionally well the 7 Quality Criteria. For detailed descriptions of the criteria and explanations for icons used, check the annexes at the end of the book.

The Book of Good Practices will be growing year by year with new cases. To contribute to this work, you can also submit your patient engagement experiences to the PFMD team.

We hope this book will inspire and help you in your patient engagement journey. We encourage you to explore all the tools at your disposal within PFMD and Synapse - the mapping and networking tool, and connect with us for more guidance if needed.

We'd like to extend our thanks to all the reviewers, all case owners and all readers for making the Book of Good Practices possible.

PFMD Team

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https://synapse.pfmd.org/book-of-good-practices/

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HEALTH COLLABORATORY

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PATIENT FOCUSED MEDICINES DEVELOPMENT

HEALTH COLLABORATORY

Patient/Caregiver Pathway Mapping & Patient Shark Tank®

Organisation: Health Collaboratory

Basic Information

The objective of these interventions is to understand the path that patients and care/support partners have travelled, with a focus on their challenges, emotional state, and support mechanisms. Proprietary tools were used to translate the patient and care/support stories into quantitative data. The stages across their journey were discussed beginning with the experience of symptoms through long term management. Qualitative semi-structured interviews were conducted utilizing a conversational, narrative, ethnographic approach to allow patients and care/support partners to piece together and share their story in a meaningful way. An interactional exchange of dialogue took place using a biographical, fluid and flexible structure, which provided the opportunity for a co-production of the patient and care/support partner journey map. A participatory storytelling technique was used to provide a safe zone for information exchange with a deeper dive into the following domains:

- Emotions experienced across phases of the care journey
- Challenges and barriers faced
- Main questions across the care journey
- Main channels/sources used for information and support
- Relationship with doctors and care team
- Impact of condition on main aspects of life

Objectives:

- To provide quantitative and qualitative data on unmet needs and therapeutic burden of patients and care/support partners through pathway mapping, life impact, and emotional journey across disease states
- To empower patients and their care/support partners to provide perspective on healthcare innovations

The methodology of the patient engagement initiative:

1. Qualitative semi-structured interviews utilizing a conversational, narrative, ethnographic approach were conducted to allow patients and care/support partners to piece together and share their stories and unmet needs in a meaningful way
2. Participatory techniques were deployed providing an opportunity for co-production of patient and care/support partner pathway mapping. Proprietary tools were created to assess some of the following domains and translate unstructured data into quantitative data points. Example domains include:
   - Impact of Condition on Life
   - Challenges and Barriers Faced
   - Triggers and Behaviours
   - Therapeutic Burden
   - Decision Points & Healthcare/ Clinical Trial Experience
3. The Patient Shark Tank® was created to amplify the voice of the patient and care/support partner in the design, development and/or continuous improvement of healthcare innovation, whether it's technology, research design, education, policy, strategy etc. Although there are expert patients and care/support partners who are part of the tribe, the majority of patients and their care/support partners have not intersected with players in the healthcare ecosystem other than their healthcare team. Patients and care/support partners are coached on how to reflect on their personal stories and experiences as they provide perspectives and/or partner on the development of innovations. To ensure diversity in patient and care/support partner populations, emphasis is put on recruiting a balance of those who represent various ethnicities, races, religions, age groups, socioeconomic status, geographies and sexual orientation. To certify innovations a co-designed scorecard is utilized to allow for standard criteria and assessments to be used in the evaluation of innovations resulting in a "Patient Shark Tank Tested" seal. Today, over 18,000 patients and care/support partners across disease states have evaluated innovations globally.

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

Research and discovery phase: including drug discovery, non-clinical and candidate-identification phases

Post-marketing or clinical study phase 4: including post-regulatory approval phase

Which stakeholders does this PE project involve?

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

Other: Investors

The quality of patient engagement

1. Shared purpose

The initiative was created to ensure that patients and care/support partners have an opportunity to voice unmet needs and therapeutic burden outside of a typical interview session. Pathway mapping was conducted utilizing 1:1 (patient), 1:2 (patient: care/support partner), and 1:many (group of patients) approaches.

Group and 1:1 feedback was obtained from patients and care/support partners to ensure they felt their specific needs were being addressed. In addition, scorecards that were used as part of the Patient Shark Tank® were co-designed with patients and their care/support partners.

2. Respect and accessibility

All patients and care/support partners received coaching during the Patient Shark Tank® to ensure they were comfortable interacting with various stakeholders. In addition, we created a process to provide patients who were not comfortable participating in a group setting, and those with limited mobility to also participate. Patients were compensated for their time.

Benchmarking of fair market reimbursement vs. expectations was also conducted. There were also frequent 1:1 touchpoints.

3. Representativeness of stakeholders

Different ethnicities, races, religions, age groups, socioeconomic status, geographies, sexual orientation, and stages of disease were incorporated into the selection of our patient/caregiver partners using our database, which consists of ~18,000 patients and care/support partners representing various patient and care/support partner segments across disease states.

To ensure the activity represented varied demographics and was fair balanced, we set criteria with specific goals that were documented to also ensure the right patient and care/support partner was aligned with the right project.

4. Roles and responsibilities

Patients and care/support partners were clear on their role in the pathway mapping and how the data would be used to determine trends in unmet need. Patients and care/support partners were also provided direction on how their Patient Shark Tank® participation would be used with feedback loops built in throughout.

Commitment documents were developed with patients and care/support partners. A scorecard co-designed with patients and care/support partners was used during the assessment.

5. Capacity and capability for engagement

All patients and care/support partners received training and coaching during the Patient Shark Tank ®to ensure they were comfortable interacting with various stakeholders. Not all patients and care/support partners that were interviewed were used in the Patient Shark Tank®, thus comfort level and boundaries were determined in advance.

Each patient and care/support partner completed an assessment on their engagement preferences and overall experience in the activities.

6. Transparency in communication and documentation

Some interviews were recorded and notes taken throughout. Feedback was captured in Patient Shark Tank® scorecards that were co-designed with patients and care/support partners.

7. Continuity and sustainability

All patients and care/support partners that participated were kept abreast of developments and continuous improvement of the initiative through frequent updates, even as other patients and care/support partners rotated through different parts of the project.

We used various communication channels to ensure continuity throughout the project.

Results and outcomes

Patient Shark Tank®:

- **>18,000 patients and care/support partners across the globe**
- **~900 innovations evaluated**
- **Patient Shark Tank® compensation assessment conducted in alignment with expectations**
- **12 Different Assessment Models Created**
- **Virtual Build Underway**

Pathway Mapping Initiative led to the following (measured) outcomes:

- **1,800 patients/1,200 caregivers**
- **Short term and long term unmet needs of patients and care/support partners were identified (traditionally from KOL perspective)**
- **Trends in burden of therapy were identified**
- **Co-design of potential research questions**
- **5 innovations were evaluated using Patient Shark Tank® during [this project] utilizing 300 patients and caregivers**

Improvement in understanding unmet needs and therapeutic burden.

Evolution from KOL perspective of patient/caregiver burden.

Positive impact for specific medicines development phases

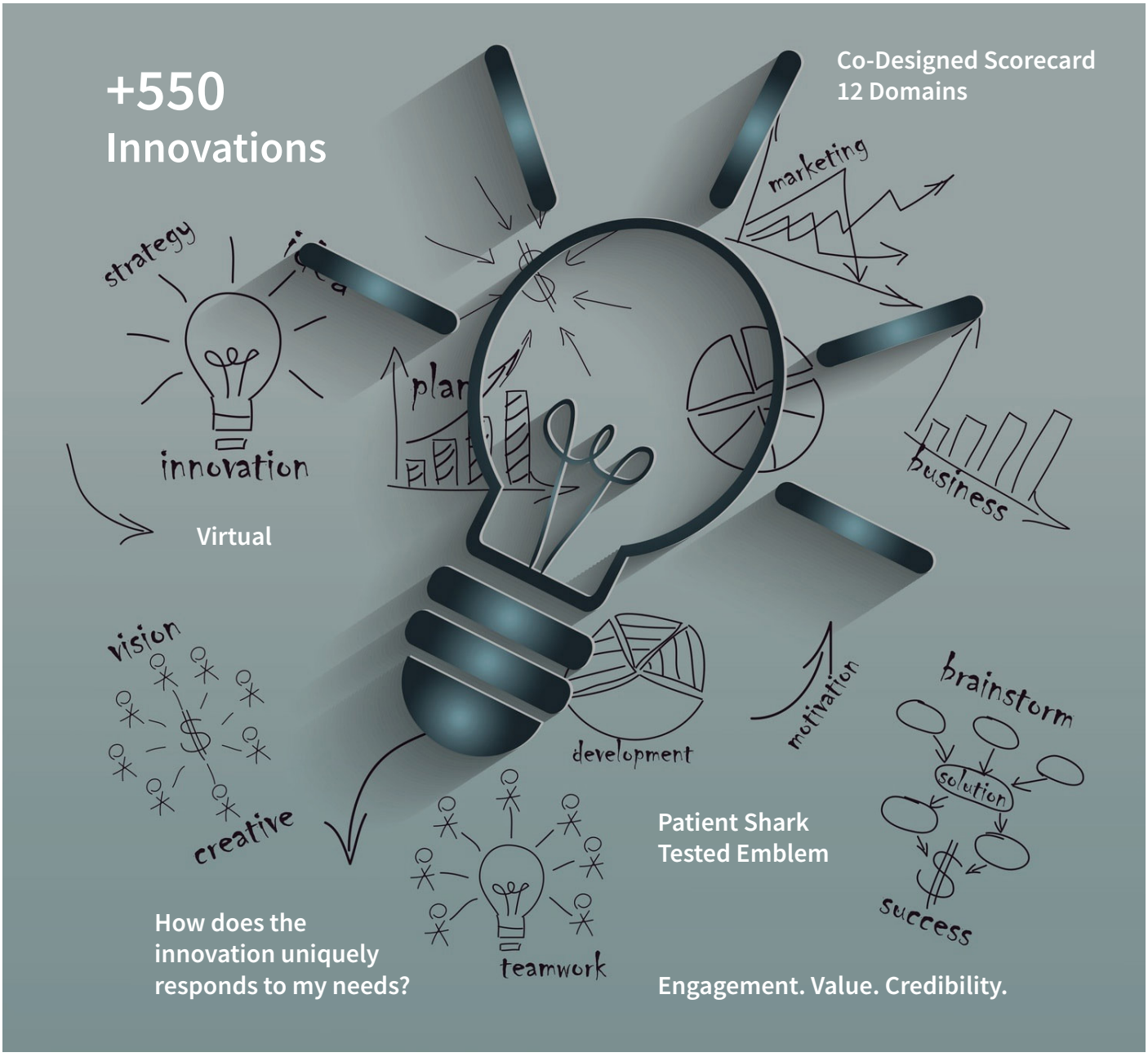
- Co-design of research question
- Opportunity for non-traditional voices to be heard
- Diverse representation of patient and care/support partner populations
- Quantified patient stories and unstructured data through various proprietary tools to translate qualitative data into quantitative data points that could be used in submissions

Direct or indirect positive impact for patients

- Opportunity for patients and care/support partner perspectives to be heard and dimensions of life affected to be measured
- Opportunity to understand how others are also affected through simplified comparative data reports
- Allowed patients and care/support partners to understand how perspectives were utilized through feedback loops

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

- Informed understanding from patient and care/support partner perspective
- Validation against EBM



Lessons learned

@sarahkrug1

About the Health Collaboratory

The Health Collaboratory™ is an innovation hub, founded in 2011, that amplifies the voice of patients and their families in healthcare innovation, creating impact through co-designed solutions that advance collaboration across the healthcare ecosystem. As a change agent in advancing participatory medicine, the Health Collaboratory™ is at the intersection of innovation, research, education and advocacy, revolutionizing the way in which we impact and improve health. The Health Collaboratory™ has empowered over 18,000 patients and their care/support partners globally to co-design and provide perspective on the design, development and continuous improvement of healthcare innovations.

PATIENT FOCUSED
MEDICINES DEVELOPMENT

Janssen
PHARMACEUTICAL COMPANIES
OF Johnson&Johnson

Driving Innovation and change via PE Dashboard

Organisation: Janssen

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PATIENT FOCUSED MEDICINES DEVELOPMENT

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Driving Innovation and change via PE Dashboard

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Basic Information

Janssen modified and developed new tools to include direct patient perspectives and drive patient centered decisions:

1. **Patient Insights Playbook and Integrated Patient Journey Template**
   Patients from US, UK, Australia, Taiwan and Korea provided feedback on the PE Playbook. This occurred via in depth interviews. Some modifications have been made and further modifications will be made, especially to terminology, and also to ensure that interactions are mutually positive.
2. **Updated Target Product Profile (TPP): includes direct patient insights**
   To validate insights from patients, various qualitative and quantitative approaches have been utilized, depending on the project. For example, during a patient advisory board meeting, patients consistently expressed and described the impact of fatigue on their daily lives. Fatigue is well documented in the literature, however hearing directly from patients influenced the R&D team to take action and study fatigue. Although the Target Product Profile (TPP) is proprietary, it includes primary and secondary endpoints, patient reported outcomes, patient benefits, safety, tolerability, and dosing/presentation. Insights should be provided directly by patients based on descriptions of disease and treatment burden and unmet needs. **Please see Table of Contents of the PE Playbook and Dashboard examples.**
3. **Customer Value: patient insight into formulation/supply chain**
4. **Patient Voice Plans in R&D: patient insight into compound and clinical strategies and protocol design**
   Elements of study designs modified include reordering tasks/tests and reducing the length of visits. A new Director, Patient Insights has joined the team and is currently conducting studies in over 13 countries with more than 1,300 patients across all 11 disease areas (update from 09/2019: 1900 patients from 26 countries). The purpose is to gather patient feedback on protocols in order to make modifications before protocols are finalized.

A recent example of modifying study design was reordering tasks based on patient feedback. The team originally planned to start with more difficult tasks and end with simpler tasks as a "reward". However, based on direct patient insights, a decision was made to start with simpler tasks and build up to more difficult, so that patients could build confidence. This change will apply to multiple studies for the same disease area.

5. **Internal Compliance Guidebook: best practices on how to implement patient engagement**
   Practical examples: modifying a device design based on patient insights, resulting in more correct dosing of medication, re-ordering tests in a clinical trial, reducing the length of visits in a clinical trial, decision to measure fatigue and sleep patterns based on patient insights, working with patients to develop a new symptom diary, and involving patient groups in development of web site content. These examples are from multiple disease areas within the company.

Driving change via Patient Engagement Dashboard

- Objective: To drive behaviour to incorporate direct patient voice early and understanding barriers.

Activation: They are measuring the amount of organizational change.

Enablers: They are seeking to understand roadblocks to organizational change.

Sustainability: They are measuring the impact of these Patient Engagement activities.

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

"These tools and processes span the drug development life cycle"

- Research and discovery phase
- Pre-clinical phase
- Clinical study phase 1-3
- Health technology assessment
- Regulatory review and approval or registration phase
- Post-registration/ -launch activities
- Other

Which stakeholders does this PE project involve?

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

The quality of patient engagement

1. Shared purpose

Janssen's Patient Engagement Mission Statement: We will systematically interact with patients, obtaining direct and inferred insights, so we can effectively act on their perspectives early.

In one example, the Patient Reported Outcomes team worked with patients to understand their perspectives, ultimately resulting in the development of a new tool to measure what matters to patients, as reported directly by patients. Direct patient interviews were used to develop the questionnaire, and patients participated in cognitive interviews to ensure that the questionnaire's instructions, questions, recall periods and response criteria were clear and easily understood. Validity and reliability were assessed in an observational study.

How did you check that what you did was appropriate to achieve this criterion? (If possible, please provide examples.)

We worked with patient groups to create and validate our mission statement.

2. Respect and accessibility

Our existing tools and processes have been updated to include Patients as an additional stakeholder with equal respect in our decision making (Target Product Profile, Customer Value/Formulation Decisions and Integrated Patient Journey). Patient-Reported Outcomes team also created a Patient Voice Plans for R&D at a compound level, to strategically assess and determine what were the best ways to engage with patients in our clinical plans. Patient-Reported Outcomes team knows that there is not a one size fits all methodology for Patient Engagement so we recognize that the unique needs of all stakeholders should be taken into account upfront.

How did you check that what you did was appropriate to achieve this criterion? (If possible, please provide examples.)

In working with patients on the PE Playbook, Patient-Reported Outcomes team worked with facilitators who are based in the local countries and understand the local cultures, and a discussion guide was used.

3. Representativeness of stakeholders

For representativeness, it is important to specify at the beginning what is the target population.

The Patient Voice Plans, at a compound level, will strategically assess and determine which sample groups should be involved in the project. The Internal Compliance Guidebook and the Patient Insights Playbook

also provide guidance and recommendations on selecting representative groups.

How did you check that what you did was appropriate to achieve this criterion? (If possible, please provide examples.)

One example in France, a patient group provided input into a trial protocol, changes were made, and the group was notified about which changes were implemented and some which were not. Later we decided not to further develop the medicine and the same patient group was notified.

4. Roles and responsibilities

We have Best Practices for certain types of Patient Engagement activities which detail accountability and commitment expectations. We are planning to expand these Best Practices to other Patient Engagement activities.

In collaboration with Patient Groups, a video was produced to help patients manage a new diagnosis with IBD. This was a truly collaborative effort, with roles and responsibilities outlined at the beginning, which were modified as the project was underway based on real time learnings. In the UK, the operating company has partnered with a patient organization for a three year period to be measured by the patient group against a set of criteria evaluating the level of patient centricity. A baseline has been identified, and plans have been agreed upon.

Patients also helped develop the Patient Engagement Mission, Patient Engagement Playbook, and have provided insights into Target Product Profiles as well as other tools. Patients provided feedback and insights through various interactions including advisory boards, interviews with patients and patient groups, focus groups, surveys, market research and other means. Patients also helped one R&D team develop a new survey to measure symptoms for a disease. The process began with members of an online patient community providing feedback on a draft which was then further modified, and further tested.

How did you check that what you did was appropriate to achieve this criterion? (If possible, please provide examples.)

In both cases (IBD project and patient centric culture partnership), contracts were mutually agreed upon and entered into up front – before the start of the projects - and modified as appropriate along the way. One lesson learned is collaborating on a project slowed down timelines, but it was the right thing to do.

5. Capacity and capability for engagement

The Patient Voice Plans, at a compound level, will strategically assess and determine what support is needed for meaningful engagement in the project(s). The Patient Insights Playbook also provides guidance and recommendations for meaningful collaborations.

How did you check that what you did was appropriate to achieve this criterion? (If possible, please provide examples.)

Training and toolkits have been developed for internal personnel, and moderators from outside the

company are selected based on experience working with patients. For a patient advisory board recently put in place for a 1.5 – 2 year period, patients went through an application process. The application was written in a manner appropriate for lay persons, and mutual expectations are being set for engagement between throughout the project.

6. Transparency in communication and documentation

What did you do to achieve this criterion? (If possible, please provide examples.)

Within Janssen, we have standardized tools and processes to ensure a harmonized approach to patient engagement. We also store and share anonymized project specific insights so that they can be leveraged in the future. The dashboard is attached here.

How did you check that what you did was appropriate to achieve this criterion? (If possible, please provide examples.)

A new internal system was developed and put in place to store market research and other appropriate documents so that patient insights can be referred to and acted on regularly. Patients and patient groups have provided consent and privacy rules and agreements have been followed. Before a project begins, materials are produced which are clear and written in lay language.

7. Continuity and sustainability

Janssen has policies on Patient Engagement, inclusive of transparency (Global, Regional and National regulations).

How did you check that what you did was appropriate to achieve this criterion? (If possible, please provide examples.)

Janssen's policies are set according to global/local laws and regulations and are independently monitored and audited for business adherence to the set policies.

Results and outcomes

Outcomes and results include, but are not limited to:

1. **Product formulation/design** - modification of a drug/device combination product in development based on patient insights, resulting in more correct dosing.
2. **Modification of multiple clinical trials** based on direct patient insights, including reversing the order of tests/tasks (protocol modification), providing transportation to the site, and reducing length of visits. In one clinical trial, there were no dropouts for 7 months.
3. **Developing medicines that better meet needs:** decision to modify a target product profile to pursue a biomarker or faster acting medicine, based on feedback from a patient advisory board.
4. **Measuring what matters to patients:** decision to measure fatigue and sleep patterns, working with patients to develop a new symptom diary.
5. **Co-developing disease educational materials.** These examples are from multiple disease areas within the company.

Positive impact for specific medicines development phases

- Better recruitment & retention in clinical trials
- Better correct use leads to better outcomes
- A more aligned product to patient's needs and wants

Direct or indirect positive impact for patients

- A better experience in Clinical Trials
- Better correct use of a product
- Products that better align to their needs and wants

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

- Redesigned Device - outcome: better correct use
- Modifications to Clinical Trial - outcome: no dropouts
- Updated target product profile - outcome: pursuing ways to know sooner whether a therapy will work

Lessons learned

Learning is that patient engagement requires time and commitment.

It is important to have the support of senior leaders, to have tools and expectations and to embed patient engagement into the various departments, such as R&D, supply chain, commercial etc. It is also helpful to hold employees accountable by measuring progress over time.

The Dashboard has been in place since 2016. Significant progress has been made and there is still more to do. To operationalize patient engagement, it is important to get support from all functions of the company to embed patient engagement as a way of working. We have also found it helpful to start with "service models" whereby a dedicated resource or department in the company initially conducts the engagements – this can accelerate progress. We have also learned that budget constraints and time can be limiting factors for patient engagement, but as long as everyone is asking what does the patient want and need and as long as patients are directly involved in the development of solutions, ultimately needs should be better met and research should be accelerated.

Appendix 1

Dashboard Objectives:

Drive Organisation's behavior to incorporate direct patient voice early in drug development

Understand barriers to incorporating patient voice

- High Value Compound
- Timing: Early, approx. ph 2
- Patient Role: in selecting, paying adhering

Patient Engagement Dashboard

Disease Area Strategy:

- Is an Integrated Patient Journey (IPJ) complete?
- Is there direct patient input into disease area strategy?

Compound Strategy

- Are Patient and Self Pay sections of the Target Product Profile (TPP) complete, with insights derived directly from patients?
- Did patients provide direct input into compound indication/s and end points?
- Have patients provided input into product formulation / delivery per Customer Value Framework?
- Does the compound have a pre-approval patient access strategy?

Clinical Development Plan:

- Did patients provide direct input into the clinical development plan prior to the start of pivotal?
- Are protocol designs and operational strategies informed by direct patient insights?
- Are the Patient Reported Outcomes needed for evidence at launch incorporated into the pivotal?

Integrated Patient Journey - Template

Key terms	Phase 5	Phase 6
Rational Title	Remission or Relapse	
Emotional Title	Waiting for the ball to drop	
Age & Time in Phase	Months	
Essence of the Phase	Attempting to return to life without cancer. Challenging as faelents are unsure of what to do with many feeling stuck in a holding pattern	
Pt/Caregiver's Emotional & Psycho-Social Experience	Patient: Optimistic that they will remain cancer free, though an underlying feeling of anxiety as patients worry about it coming back. When it does return. patients feel devastated Caregiver: supporting the patient has needed. attempting to return to their normal routine	
Pt/Caregiver's Physical & Medical Experience	Patient feels better than they have in the past with CT scans every 3 - 6 months reminding them of their cancer	
Moments of Meaning (includes unmet needs)	Physically, patients feel better in remission, emotionally it can be a tough time with many unsure how to move forward	
Stakeholder Experience	Patient, caregiver, oncologist, radiologist	
Business Leverage Point	Provide / direct patients and caregivers to support groups to help them emotionally	
Facts and Data	Even in the NCCN guidebook, remission or relapse is not discussed Treatment is discussed as cure or controlling	
Knowledge Gaps	How can patients / GGs best deal with remission? Are there things that they can do to help them find their 'new normal'?	

Appendix 1

Introduction I Purpose of Patient Engagement (PE) Playbook

Chapter 01 I Foundational Understanding of Patient Engagement

Chapter 02 I Engagement Mapping

Chapter 03 I Best Practices for Strategic Excellence

Chapter 04 I Tools, Templates, and Cross Functional Application

Chapter 05 I In Patients' Own Words

Chapter 06 I Glossary

Patient Engagement defined:

Patient engagement is integrating the voice of the patient throughout the lifecycle of the disease area and item (i.e. drug, device, diagnostics, solutions) development and commercialization.

It is defined as a purposeful dialogue with patients for improving lives and outcomes. It involves:

- Systematically Listening, Understanding, Co-Activating and Collaborating with patients directly or indirectly in one-way or two-way participation.
- Activating patient insights by informing Company's product, service and solution development early on, and by creating meaningful value proposition through innovative ideas that are empowering for the patients and their care community.

@JanssenGlobal

About the organisation

Who is Janssen? We're more than 30,000 people working hard to prevent, treat, cure and stop some of the most devastating and complex diseases of our time. From heart disease to HIV, Alzheimer's disease to cancer, we are committed to issues that touch everyone's lives.

Our mission and vision

Our mission is to transform individual lives and fundamentally change the way diseases are managed, interpreted, and prevented. We believe that challenging something is the best way to change it. So every day, in more than 150 countries, we bring cutting-edge science and the most creative minds in the industry together to think differently about diseases. We aim not only to innovate but also to empower people with the tools they need to make informed decisions and achieve the best possible results for their health.

We are looking at a future where the world of healthcare will be challenged by informed and empowered patients. We work for change that will improve access to medicines: the best available treatment at an affordable price. That's why we at Janssen strive to provide access to effective and affordable medicines and related healthcare services to the people who need them.

KATHERINE CAPPERELLA – VP, Global Patient Engagement Leader, Janssen Pharmaceuticals

As global head of patient engagement for Janssen, Katherine leads a cross-functional team responsible for developing and driving patient engagement strategy and measuring progress over time. She and her team are focused on leading Janssen to incorporate direct patient voice early and throughout the entire product lifecycle, beginning with disease area strategies and including target product profiles, formulations, clinical trials and post-marketing activities. Katherine has over 20 years of experience at Johnson & Johnson in roles of increasing responsibility.

Katherine began her career in women's health and has demonstrated success in launch, turnaround and mature businesses in a variety of disease areas. Katherine holds a Bachelor of Science in Business/Marketing from the Kelley School of Business.

PATIENT FOCUSED
MEDICINES DEVELOPMENT

Pfizer

Building patient centricity into process: clinical development - HILFE FÜR MICH (Patient Navigator)

Organisation: Pfizer

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PATIENT FOCUSED MEDICINES DEVELOPMENT

Building patient centricity into process: clinical development - HILFE FÜR MICH (Patient Navigator)

Organisation: Pfizer

Basic Information

HILFE FÜR MICH (Patient Navigator) is an online tool, created for patients as well as their relatives to inform, educate and explore people's needs. The Digital Patient Navigator, first in navigating patients and caregivers through the complex HC system for diseases, initially metastatic breast cancer, stroke and kidney cancer, is patient-led and developed in concept, content and design, leveraging synergies across therapeutic areas in co-creation with external partners.

In a pilot project, a digital service platform that offers quality-reviewed information and patient pathways for people living with illness and their families was developed. With a clear structure with questions, answers, and links, those affected should quickly find the information they need.

- What's unique about this: Through the development of personal "patient pathways" patients also get individual help in dealing with their disease. The patient pathway helps them outline and understand their options and what they can do to stabilize or improve their health. Thus the platform may help the people affected, as well as their families and other members of their support communities, to cope better with their new situation and their own emotions, to deal with the plethora of information, and to make necessary decisions with a sense of confidence and empowerment.

The central impulse for the creation of this internet portal was the idea of a board of patient experts to develop a new information offering for patients with severe chronic disease, which would offer them support and comfort on their path and would help them deal with their new situation, their emotions, and the plethora of information, as well as empower them to make important decisions and to cope better with the disease in their lives.

Patient Navigator is based on patient, physician and the Pfizer expertise:

The experts are represented for metastatic breast cancer (14 experts), stroke (8 experts) and kidney cancer (6 experts) according to their professional background. In these categories, Pfizer colleagues are involved and participate at eye level with the external specialists that support the project. Pfizer‘s experts on www.hilfefuermich.de are Peter Albiez, CEO Pfizer Germany, and Christina Claussen, Director Alliance Management & Patient Relations Pfizer Germany.

Users find information through more than 250 questions and answers that include links to medical associations, treatment guidelines or journals to make external knowledge available for the users. HILFE FÜR MICH provides links and short descriptions of more than 50 patient organizations and other platforms that offer information and help.

All content goes through a multi-level review process before publication. Checks are performed on the factual correctness of the information, the relevance and currency of the content, and whether the selection and presentation of the topics is suitable for the patient audience. Experts who provide different specialist competencies, such as in the medical, legal, and editorial fields, are involved in the different review processes. Patients also ensure that the patient orientation of the topics and content is guaranteed. Medical specialists check the accuracy of the health topics, while communication experts ensure that the text is easy to read and flows well.

The protection of personal privacy and private data is important to us. We capture, process, and use personal data in compliance with the German data protection laws and its data protection declaration.

Initiative update in August 2019:

- the possibility to create an individual patient pathway has been removed three months ago;
- the number of indications went up from 3 (mBC, stroke, kidney cancer) to 8 (lung cancer, pain, rare disease, ulcerative colitis and smoking cessation) and
- due to this expansion, the number of experts increased as well (lung cancer: 8, pain: 9, rare disease: 13, ulcerative colitis: 9, smoking cessation: 8), and in addition
- the number of questions and answers went up to over 1.050 as well.

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

Medicinal product agnostic, generally intended for newly diagnosed people with illness with potential utility at other times in the journey with that illness.

Other: Care journey (disease/care management)

Which stakeholders does this PE project involve?

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

The quality of patient engagement

1. Shared purpose

The concept for the Patient Navigator originated from patients attending a regularly occurring Pfizer-Patient-Dialogue in Germany with patient advocacy groups. Discussions were continued among a smaller group of patient representatives, IT experts, health care professionals, and Pfizer colleagues to co-create the Navigator.

The working group confirmed the appropriateness and progress at regularly occurring update discussions.

2. Respect and accessibility

The program was shaped from the start with guidance from patients and in an iterative manner through its development.

Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.

3. Representativeness of stakeholders

Patient advocates and healthcare professionals involved in the project interact on a regular basis with a diverse group of patients within Germany and strived to integrate those diverse experiences into the project. However, we recognize that individual patient experts cannot be fully representative of all patients who may use the Navigator.

Information is collected by the people using it to help them navigate their care including and not limited to appointments, family and patient resources, medicines, professional and social issues.

4. Roles and responsibilities

The point of contact for the patients involved was a skilled and dedicated Patient Relations Manager (well-versed in Pfizer, EU and Country regulations for interactions with patients) who has developed and nurtured relationships with patients and patient organizations involved.

Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.

5. Capacity and capability for engagement

The patients involved had the requisite expertise as established advocates and included people with health communications expertise as well as experience as patients.

The selection of the team included patients from the start as well as Pfizer colleagues to ensure the alignment of capabilities with the goals of the project.

6. Transparency in communication and documentation

A transparent communication plan was part of the design and release of the Navigator to the public. In addition the quality process is outlined below:

1. Concept: Co-Development of Concept, Topics and Content together with Patient Organisation
2. Editorial: Defined quality criteria
3. Review I: Check by patients and caregivers
4. Review II: Check by medical and legal external experts

At a glance: How was the content for this website created?

Conception	Editorial Team	Review I: Patients	Review II - Scientific experts
Selection and concept of topics and content in collaboration with experts in patient work	Creation and editorial review of content based on defined quality criteria	Review of the content by external patients and relatives	Review of the content by medical experts on the scientific advisory board

Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.

7. Continuity and sustainability

The plan included launch in two illness areas with the intent if successful for expansion beyond those areas and in additional geographies beyond Germany. The team including patient experts has been involved in those discussions. A third illness area was launched and more are planned.

Feedback from patients at regularly scheduled discussions verified that these actions were meeting their expectations of engagement.

Results and outcomes

Positive impact for specific medicines development phases

Pfizer's "patients first" approach across the medicines development phases includes creating new alliances and partnerships among stakeholders. Responsiveness to suggestions meeting a need identified through the Pfizer-Patient-Dialogue is but one way Pfizer is demonstrating this commitment.

We are working with other country and global teams to implement this in other countries and adapt it in ways patients feel it will be useful for them.

Direct or indirect positive impact for patients

The Patient Navigator program may help people identify their needs and assist them with navigation through the healthcare system as well as a better understanding of patient health condition and expectations. Metrics below about initial reach and average session duration are encouraging.

Total Number of users (launch 8th May 2017 until 30th August 2019)

	Users total	Session Duration	New Visitors	Returning Visitors
May 2018	221,000	00:00:59	219,600	17,400
2019 update	642,735	00:01:05	641,565	57,992

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

The impact for non-patient stakeholders is the experience of co-creating a tool with patients as experts on the same team. This has not been the traditional approach in the development of healthcare tools for patients.

Lessons learned

Key Learnings:

1. **A "Patient First" approach is key:** the patient need was at the core of the project and a group of patient experts were involved in developing the program.
2. **Strong sponsorship and involvement of sponsors along the journey:** sponsorship at senior organizational levels, with involvement throughout the project and at key strategic points.
3. **Plan carefully with the flexibility to be responsive to potential challenges:** create a project plan, look at all the tasks and dependencies early and get input from all external and internal experts along the way.

@pfizer

Pfizer's purpose

Our purpose is grounded in our commitment to fund programs that provide public benefit, advance medical care and improve patient outcomes. Our belief is that all people deserve to live healthy lives. This drives our desire to provide access to medicines that are safe, effective, and affordable.

PATIENT FOCUSED
MEDICINES DEVELOPMENT

AKU
Alkaptonuria Society

Develop AKUre - Patient-led clinical trial

Organisation: AKU Society

The PFMD
Book of Good Practices

1st edition I 2018

PATIENT FOCUSED MEDICINES DEVELOPMENT

AKU Alkaptonuria Society

Develop AKUre - Patient-led clinical trial

Organisation: AKU Society

Basic Information

DevelopAKUre

AKU Society led a project to start clinical trials on repurposing an existing medication to a rare disease. “It aims to study a potential new drug, called nitisinone, and assess its potential effectiveness in treating the rare disease, alkaptonuria (AKU)”. The project started in 2003 and got European Commission’s funding in order to develop and run the clinical trials. The project is ongoing until 2019.

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

Research and discovery phase | Pre-clinical phase | **Clinical study phase 2-3** | Health technology assessment | Regulatory review and approval or registration phase | Post-registration/ -launch activities | Other

Which stakeholders does this PE project involve?

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

The quality of patient engagement

1. Shared purpose

AKU Society aimed to develop AKU re-discussion with patients (who were involved a long time) before the clinical trials (CTs) started; AKU meetings, communication towards patients. After visits, personal experience patient feedback is aggregated and presented to consortium members. Feedback is generated through a google sheets survey. (Attached is an example of the survey and how it is presented to consortium members).

Workshops are a valuable tool to report back to patients about the progress of the trial and enables the AKU Society to address any issues if they arrive. International workshops are attended by the majority of consortium members who can address patients directly.

Communication with patients happened naturally, listening to their concerns in these discussions helped in the design and planning of the CT and making it adapt to patients' needs. A few things were changed in the design, some were compromised and some couldn't be changed based on the feedback (E.g. non treatment group).

2. Respect and accessibility

Patients' needs were taken into consideration in the design of the CT. This includes reducing the amount of time participants spend in each of the trial sites. Based on feedback from the project's first clinical trial, we explained to other stakeholders the importance of patients having 'down time' and not staying in hospital as if they were unwell. Due to this, for the second phase of the project's clinical trial, patients stayed in a nearby hotel. Feedback from this move was overwhelmingly positive and has led to patients from across Europe seeing their visit as a holiday. They take ample opportunity to use the trip as an opportunity to explore Liverpool and its tourist sites, as well as extending their trip to holiday in the UK (Feedback is attached here).

It was also noted that due to the nature of the disease, patients may have severe mobility issues. As a reflection of this, and with feedback from the patient group, it was decided that those patients will be accompanied by a chaperone who is fully reimbursed. Normally this is a family member who acts as a carer. This is vital, as patients communicated they would not come without one and allows for continuity of care.

Recruitment was efficient because patients were helping in the discussions as well (finding patients outside of AKU Society's network). Understand that patients can be very motivated and a useful partner in recruiting and finding new patients. Patients understood the benefit of the trial for themselves and the patient community now and in the future. This was shown to them with reference to existing research and by a simple and accessible explanation on the trials website.

Researchers and doctors across Europe who may have patients affected by the disease were made aware of the trial by a series of email campaigns aimed either directly at them or through membership societies. Informed patients, however, would often tell their physicians about the CT. Due to the relatively low number of patients globally, these doctors are often highly specialised and have other patients with the disease who were then informed in turn. These would become 'patient champions' who would go onto disseminate to their own national patient groups.

Ongoing surveying allows us to track how patients are feeling in regards to all aspects of the trial. Monthly individual contact also adds to ongoing changes in areas that patients are concerned about based on their responses.

For example, the AKU Society took over the paying of personal and travel expenses and booking flights for patients enrolled at the city trial site, in the middle of the trial based on feedback in regards delays in reimbursement and issues over flights. The vast majority of patients when contacted directly saw this as positive and have commended the consortium on the positive impact this has had for them.

Almost half the patient population was recruited using the steps above. Due to the nature of the recruitment, it was integral that they keep the conversation with those enrolled open at all times throughout the trial. This is reflected by the decisions taken, which are highlighted above, and by their continuing high level of retention, which is almost 95%. Very high for this type of CT.

3. Representativeness of stakeholders

Aiming to get everyone possible - eligible over 16 yrs old, able to travel to the site.

Some patients didn't attend due to other conditions. Approx. 60 patients from the UK couldn't join the CTs because they had taken the drug previously as unlicensed drug (National Specialised Services) >> however, they did participate in the design of the study.

Representativeness of data:

- Benefit: two different data sets (60 patients from NAC) and 140 from the CT.
- As per the majority of other CT's, pregnancy or imminent pregnancy was an automatic bar to enrolment due to lack of scientific understanding of how the drug affects unborn children.

The AKU Society knows of only 500 patients throughout Europe. This meant diversity was almost guaranteed. All study materials were professionally translated and the website was installed with a translation service so that it was accessible to everyone.

4. Roles and responsibilities

- AKU Society was accountable to the patients > workshop actions were followed up by patients
- Getting EU Commission funding made the Society accountable to proceed with the application process> having the actual EU funding held us accountable
- Within the project, partners held each other accountable (12 partners: 2 PGs, 1 Industry, 3 hospitals, 1 CRO, rest research teams >> 5 different countries represented)

Regular project meetings by teleconference, face to face meeting once a year, progress reports every six months.

These meetings and continued teleconferences were used to ensure that each member of the consortium knew what was expected and were accountable to everyone else.

As a patient group, we are tasked with the dissemination of all the project's ongoing achievements and how it was reaching its goals. This meant that all partners had to feed back to them in regards to how these goals

were being met. This was then disseminated to the patient group via our website. If these weren't being met, we held the relevant partners to account based on patient questions generated at face to face meetings.

Each partner was involved in the trial design at earlier stages and throughout. This involvement wasn't limited to the areas that they would be responsible for. This inclusivity has led to mutual ownership of the trial and where members are responsible to each other and the wider patient group.

5. Capacity and capability for engagement

Capacity was built almost by accident over time - AKU Society learned about the CT process alongside with patients.

Big questions from patients:

- Patients had their own explanations about the randomisation/group
- Placebo group problem - what's the point in it if the drug is known to already work
- CT phases/ process clarified

Suggestions from both researchers and patients were discussed openly and agreed mutually. A few workshops together, having a statistician helped in clarifying some of the questions patients had.

* The EUPATI programme is good for patient education. Educating patients on clinical research and how they are involved- 3 representatives did a EURORDIS summer school.

6. Transparency in communication and documentation

Communication:

- Comms (related to CT) to patients has to go through the ethics committee
- Advice received from CRO on transparency and communication with patients (documents needed, what can be said and not, how to explain things)
- Ongoing discussions about patient support after the trail is over

Documentation is part of the EC grant requirements - every 18 months.

- Making sure patients know what's going on to ensure retention (interim communications after phase 2, midway phase 3)
- Every month all patients emailed for questions or update with information.
- For the CRO, this level of communication was unusual.
- Partners happy to have discussions about how much can be shared.
- Need to understand different stakeholders' motives to keeping some of the information confidential.

7. Continuity and sustainability

Continuity:

- EC project requires study reports, documentation for licensing
- Patient survey for patients taking the drug on why they're taking this drug should help in discussion with payers

As a patient group, we have a vested interest in the continuation of the search for an effective treatment for alkaptonuria. If this trial is successful we will liaise with all concerned in regards to licensing of the drug and reimbursement across the world. We will assist patients enrolled on the study to find healthcare and treatment after the study. Any further discussion on this isn't appropriate at this stage.

Results and outcomes

Positive impact for specific medicines development phases

Discovery and research: identifying unmet medical needs, prioritise research agenda.

Clinical development phases: improving study design (e.g. fewer protocol amendments), accelerating patient recruitment, improved retention of the patients during clinical studies, reduced time of the clinical development of the medicine, financial impact.

Registration: quality of registration dossier, timing to registration.

Post launch - life cycle: patient solutions increasing patient adherence to medication, extension to new patient groups, adapted formulation, enhanced quality of AE monitoring and reporting, etc.

- First treatment to AKU
- No major amendments to the study protocol.

Efficiency: Faster recruitment (under 1 year vs. 2 years) due to prepared patients

Direct or indirect positive impact for patients

Finding treatment for AKU patients.

- Recruitment rate 50% of eligible patients across Europe
- Retention rate over 95%

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

Researchers: understanding patients' unmet medical needs, PE helps in focusing and prioritising research efforts, research funding process is empowered with patient insights.

Patients/ Carers: influence/ impact to research prioritisation agenda, easier access to novel therapeutic options (e.g. new medicine in clinical trials phase), influence on outcomes of clinical trials, enhanced understanding of disease conditions and treatment options, better compliance to treatment.

Wider community: more effective medication leading to reduced health services, health/ condition related education of one individual is shared beyond that individual.

Patient Advocates/Patient Organisations: increased knowledge about being involved in collaboration with other stakeholders.

Industry: more effective research prioritisation efforts, acceleration of clinical development, faster registration process, etc.

HCPs: better understanding of patients' health conditions and expectations, quality of clinical trials, adherence to medication is increased, etc.

Regulators: patients' voice is embedded is decision-making, ensuring the quality of the regulatory file etc. Reducing the time that patients had to spend at the site

Funding: all partners received funding from EC in this project. The pharmaceutical company hopefully will end up with an approved product at the end of the study

Hospital site: Came up a new method to measure HGA (chemical in AKU patients) and therefore monitor progression of AKU → Papers/ research articles published

Lessons learned

Since the beginning of the project, we have shared what we have learnt across a myriad of media, from articles to talks. This is due to the rarity of projects like this. We were learning every step of the way. Luckily with the help of the pharmaceutical company the clinical sites and the CRO, along with our drive to represent patients, we believe we have designed a remarkable CT.

As AKU Society lead on patient identification, recruitment and support for all patients enrolled at the three clinical sites across Europe and also leads on dissemination of the project, we were in a unique position to give advice to others in a similar situation.

- Relationships: it's integral to build relationships with patients, to help them understand everything about the trial and feel able to ask the difficult questions. This is the key reason for including patient groups in clinical trials. Pharma companies have to keep their distance from patients, and so patient groups can fulfil that role.
- 61% of patients said feeling part of a community was an important part of deciding to take part in a trial. Rare disease patients are so spread apart, communities have to be online to get the discussion going with the most patients.
- 30% of clinical timelines is to recruit patients for research and 90% of clinical trials are delayed because of patient recruitment problems; this is made even more difficult in rare diseases. However, in this project they reached target of almost 150 patients in under 9 months. Through hard work, and collaboration, media campaigns across Europe, attendance at scientific conferences, and email campaigns to 7,000 specialist doctors.

References

1. http://patientfocusedmedicine.org/wp-content/uploads/2018/05/Attachment.xlsx

Appendix 1 (2016 Feedback -Liverpool)

Clinical trial 2

Overall experience? Average of 4.5

The travel organisation? Average of 4

The accommodation? Average of 4

The interpreter? Average of 4.5 (four answered this question)

The organisation of tests and Assessments? Average of 4

Communication with staff prior to visit? Average of 4

During visit? Average of 4.5

After visit? Average of 4

Comments

Negative

- Patients prefer the Liner over other hotels.
- Some patients wish to go home on early and feel like they are not needed for the whole time.
- Taxis being late/not turning up in Liverpool.
- Expenses issues.
- Having to go back to departments due to mistakes.
- Mixed reviews of interpreters in Liverpool.

Positive

- The staff in Liverpool are widely praised by patients, especially Emily and Ranga.
- High score for overall experiences staff and organisation
- High praise for communication before during and after visit.

Observational study *

*Only three patients responded.

Overall experience? Average of 4.5

The travel organisation? Average of 4

The accommodation? Average of 4.

The interpreter? N/A

The organisation of tests and Assessments? Average of 4

Communication with staff prior to visit? Average of 4

During visit? Average of 4.5

After visit? Average of 4.5

Comments

Negative

- Ear Biopsy did hurt. One patient said it hurt a lot, the other two thought it was an acceptable level of pain. Suggestion strong painkillers are provided.
- Slight delay in expenses.

Positive

- High score for overall experiences staff and organisation
- High praise for communication before during and after visit.
- Liverpool staff and Patient Organisation are highly praised
- “Everyone was great! Which eased a lot of my concerns.”
- “Thanks you guys, seriously. It means a lot to be treated like a human being especially during trials.”

@AKUSociety

About AKU Society

The AKU Society is a patient led charity that offers life-changing support to AKU patients and their families. We provide patients with personalised care and home visits along with the latest information from specialist workshops, our website, social media and online patient communities. We have also pioneered and funded research into the diseases whilst looking for an effective treatment: a drug called nitisinone is working, drastically lowering the build-up of the harmful acid.

The DevelopAKUre programme was a series of three major international clinical trials, run by a consortium of 12 European partners. It aimed to study a drug, called nitisinone, and assess its effectiveness in treating AKU. Nitisinone is not licensed for AKU but is being used by many patients off- label and at the National Alkaptonuria Centre (NAC) in Liverpool.

DevelopAKUre ended in January 2019, and the consortium then worked on analysing the data. SOBI announced in July that the data was positive and that they would apply to the European Medicines Agency (EMA) for a marketing authorisation license for nitisinone for AKU.

Picture of the consortium

PATIENT FOCUSED
MEDICINES DEVELOPMENT

PARKINSON'S UK
CHANGE ATTITUDES.
FIND A CURE.
JOIN US.

Supporting patient involvement in the development of new treatments

Organisation: Parkinson's UK

The PFMD
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Supporting patient involvement in the development of new treatments

Organisation: Parkinson's UK

Basic Information

Recruitment of patients with Parkinson's UK to work with a research team: stem cell therapy; exploring the level of understanding/benefit vs. risk.

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

- **Research and discovery phase**
- Pre-clinical phase
- Clinical study phase 1-3
- Health technology assessment
- Regulatory review and approval or registration phase
- Post-registration/ -launch activities
- Other

Which stakeholders does this PE project involve?

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

The quality of patient engagement

1. Shared purpose

Values and common purpose include:

1. **Exploring the level of understanding of stem cell therapy among people affected by Parkinson's and attitudes towards stem cell therapy as a potential future treatment in Parkinson's**
2. **Understanding minimum benefit vs risk when considering stem cell therapy treatment**
3. **Recruiting people affected by Parkinson's to work as part of a research team in future work**

By outlining clear goals and shared purpose, each stakeholder group was aware of their responsibilities to the session and to each other. Our Research Involvement team worked with stem cell therapy research team to develop objectives and goals for involvement. These were then communicated with people affected by Parkinson's prior to the focus group meeting.

Stage 1: We worked with a UK based University research team and people affected by Parkinson's to create a survey to consult a large patient population about attitudes to stem cell therapy. The 548 survey participants self-selected from a group of over 3000 people affected by Parkinson's interested in research. The discussion group of 16 participants were selected as they had completed the "Patient Involvement in research training".

Stage 2: Following the survey, we organised a discussion session between a group of 16 people affected by Parkinson's and the stem cell research team at the University. The purpose of the session was to explore and understand the survey responses in greater depth.

Stage 3: The attendees at the discussion session received feedback about the survey and discussion session and were then invited to work as part of the research team to further develop this work and to apply for funding. We evaluated these interactions to ensure that all stakeholder's needs and goals were met.

2. Respect and accessibility

This work was completed as part of Parkinson's UK's Research Involvement Awards which brings together and facilitates partnerships between people affected by Parkinson's and the research community. Parkinson's UK's research involvement team act as the broker/neutral party, ensuring that the needs of people affected by Parkinson's are considered at every stage, that information and opportunities are timely and appropriate and that conversations and output are always respectful and reciprocal.

Involvement was sought from both people with Parkinson's and people affected by Parkinson's in other ways (family members, carers, partners, friends of people with Parkinson's). Offering several involvement opportunities (the survey and the meeting) enabled some of the barriers to involvement to be addressed and ensured that the involvement opportunity was open to people across our research community.

3. Representativeness of stakeholders

It was important as part of this project to ensure that there was representation in both the survey and the discussion group related to:

- Age
- Sex
- Years since diagnosis
- People with Parkinson's and carers

This was achieved but it is a continuing challenge for us to ensure that ethnicity and socioeconomic status are also appropriately represented.

The 548 survey participants self-selected from a group of over 3000 people affected by Parkinson's interested in research. The group of 16 participants were selected as they had completed the training. There was a good selection in terms of male/female, years from diagnosis and experience of the condition and research. But in terms of ethnic and economic/social diversity this wasn't something the we measured. A diversity project will be launched this year to ensure that we have a more diverse pool of patient contributors, but this is challenging for everyone.

4. Roles and responsibilities

By outlining clear goals and shared purpose, each stakeholder group was aware of their responsibilities to the session and to each other. Parkinson's UK's Research Involvement team worked with stem cell therapy research team to develop objectives and goals for involvement. These were then communicated with people affected by Parkinson's prior to the focus group meeting.

As part of the Research Involvement Award, we monitor this accountability and have processes in place to:

- Follow up with all stakeholders
- Feedback to all stakeholders
- Evaluate stakeholder experience
- Give further support for partnership working if required

5. Capacity and capability for engagement

Both the researchers and people affected by Parkinson's involved in the discussion group had completed our Patient and Public Involvement training. This training ensured that all stakeholders understood the importance of partnership working, the goals, as well as fully understanding their respective roles.

People affected by Parkinson's were also given access to information on stem cell therapy to read before attending the discussion session and given an opportunity to contact Parkinson's UK if there were any questions related to the pre-read material before the session.

Parkinson's UK delivered training to all stakeholders involved (the lead researcher and the people affected by Parkinson's) to ensure that everyone was appropriately prepared to take part in this session. We worked with people affected by Parkinson's and the researchers to plan the session. We spent lots of time explaining the research and answering questions to ensure a full understanding of the subject and evaluated the session from both the researcher and patient perspective.

As part of the session, the lead researcher introduced stem cell therapy to begin the session - in case the attendees had not had the opportunity to read the document or had not understood it. This was then followed by a Q&A session which was given extra time if needed.

6. Transparency in communication and documentation

Prior to recruiting people affected by Parkinson's for involvement, Parkinson's UK worked with the stem cell therapy research team to develop an Involvement Plan for the project to ensure involvement was well planned. Other documentation shared between all stakeholders included:

- Pre-read information on stem cell therapy as well as detailed information on the agenda and access to facility and expenses claims form and policy
- The slides to make notes
- An immediate follow up email detailing next steps
- An evaluation of experience
- An intermediate follow up document with interim findings
- An invitation to join the research team to further develop the project
- Further documentation will include long term follow up (6-12 months)

7. Continuity and sustainability

To ensure transparency, we:

- Provided updates/feedback on the outcome(s) of involvement including where suggestions were implemented and how the project has been shaped as a result of involvement
- Evaluated patient and researcher experience and feedback
- Offered in depth follow up (immediate, mid-term, long-term)
- Recommend that further partnership working results in patients becoming part of the research team, co-applicants and co-authors

Results and outcomes

1. Parkinson's UK's perspective
   - Gaining informed consent from survey participants
   - Have separate facilitators and note takers to capture all comments and information
   - We also changed our processes in terms of conditions of support to a signed agreement from researchers about feeding back to patient contributors as well as having a more robust plan/timelines in place for things like feedback.
2. From a researchers' perspective - the researchers had originally wanted people affected by Parkinson's to comment on the more methodological aspects of their work (lab based research) but at the discussion session the patient contributors were reluctant to do that. More preparation and support needs to be given for patient contributors to contribute this way and for researchers to ask the right questions.

   Conducting a consultation (survey) to shape and inform the discussion group was an excellent way to gain a wider patient perspective along with the complementary real-life stories and in depth answers.

Positive impact for specific medicines development phases

- Understanding unmet medical need
- Understanding benefit vs risk
- Informing clinical trial development/design

Direct or indirect positive impact for patients

- Increased awareness of stem cell therapy as a treatment for Parkinson's (past trials/research, current research and future/potential trials)
- Influencing stem cell therapy trial development, making them more relevant and likely to succeed
- Increasing likelihood of new treatment for Parkinson's

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

- Better understanding of patient perspective, acceptability and expectations
- More likely to develop relevant future work
- Publication opportunity as unique area of work
- Increased chance of funding
- Empowerment for patients/public who are involved

Lessons learned

Learnings from the logistics of discussion group:

- Difficult to get enough disability parking close enough to venue
- Difficult to give good directions as the campus/venue was complicated
- Most of the group did not read the pre-read documents, but as we went through an introduction, this did not significantly affect the day
- It would have been good to have additional members of staff to help with escorting patients to room/venue
- Discussions were rich but the time consuming and resource intensive nature of using recording was not fully appreciated – it may be worth paying for a scribe to attend future sessions

Authors are currently experiencing some questions around being able to use the survey responses in a publication. Advise to get consent to publish as a specific survey question.

PARKINSON'S UK
CHANGE ATTITUDES.
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JOIN US.

@ParkinsonsUK
@_nratcliffe
@dyskochick
Dr Emma Lane

About Parkinson's UK

Parkinson's UK is the largest charity funder of Parkinson's research in Europe. Our vision - our ultimate ambition - is to find a cure and improve life for everyone affected by Parkinson's.

Parkinson's UK influence and fund groundbreaking research aimed at finding better treatments and advancing understanding of Parkinson's and its causes. Everything we do is driven by people affected by Parkinson's. We are an international leader in research, driving progress through collaboration with organisations from around the global and people affected by Parkinson's to accelerate progress towards new and better treatments.

Alongside our research activities, we provide expert information and support to people with Parkinson's, their families and carers to help people manage the condition and live life to the full. We also raise awareness of Parkinson's, change perceptions of the condition and work in partnership to drive better services.

DR NATASHA RATCLIFFE
Research Involvement Manager, Parkinson's UK

Natasha is Research Involvement Manager at Parkinson's UK, leading the charity's patient and public involvement programme and supporting researchers across academia and industry to work with people affected by Parkinson's to improve research outcomes. Natasha consults on methods and approaches to involvement and facilitates partnership working to help ensure that the expertise of people affected by Parkinson's is incorporated meaningfully throughout all stages of the research process. She also works closely with other charities and external organisations, contributing to national and global initiatives to help drive forward the field of patient and public involvement in research.

DR EMMA LANE B.SC. PH.D. FHEA
Senior Lecturer in Pharmacology

Director of Postgraduate Research Studies School of Pharmacy and Pharmaceutical Sciences Cardiff University

Completing a PhD in Pharmacology at King's College London in 2004 focused on Parkinson's disease, I then worked with world leaders in the field of cell transplantation and L-DOPA induced dyskinesia at Lund University, Sweden, to understand the generation of side effects of cell transplantation as a treatment for Parkinson's disease. I then moved to Cardiff University, first as a post-doctoral researcher and subsequently securing a position as a Lecturer in Pharmacology in the School of Pharmacy and Pharmaceutical Sciences. Since then my group has continued to focus on laboratory based research into cell and gene based treatment for Parkinson's disease but with a growing parallel interest in understanding and involving the patient voice in both preclinical and clinical research through Public Patient Involvement and engagement activities. This has led to ongoing work in leading a PPI group supporting and guiding studies in South Wales on surgical interventions for neurological disorders BRAINInvolve.

PATIENT FOCUSED MEDICINES DEVELOPMENT

Sant Joan de Déu Research Foundation

Kids Barcelona - Young Person's Advisory Group

Organisation: Sant Joan de Déu Research Foundation

The PFMD
Book of Good Practices

1st edition I 2018

Kids Barcelona - Young Person's Advisory Group

Organisation: Sant Joan de Déu Research Foundation

Basic Information

Background

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

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

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

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

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

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

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

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

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

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

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

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

- **Launch of the European YPAG Network (eYPAGnet).** In May of 2017, the eYPAGnet achieved the recognition of EnprEMA and was officially launched. The specific European regulatory environment of paediatric clinical trials and the international and multicentre methodology to perform the studies, encouraged the creation of this network. Sant Joan de Déu Research Foundation is founder member and part of the Steering Committee of eYPAGnet as well is the host institution of the network.

  The goals of eYPAGnet are:

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

Initiative update 2019

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

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

Which stakeholders does this PE project involve?

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

Other: CRO

The quality of patient engagement

1. Shared purpose

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

2. Respect and accessibility

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

3. Representativeness of stakeholders

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

4. Roles and responsibilities

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

5. Capacity and capability for engagement

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

6. Transparency in communication and documentation

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

7. Continuity and sustainability

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

Results and outcomes

Positive impact for specific medicines development phases

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

Lessons learned

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

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About Sant Joan de Déu Research Foundation

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

Picture: Kids Barcelona coordinators Begonya Nafria and Joana Claverol

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

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

PATIENT FOCUSED
MEDICINES DEVELOPMENT

gsk | ViiV Healthcare

Patients and community feedback on PLSs (Plain Language Summaries)

Organisation: Viiv Healthcare/ GSK

The PFMD
Book of Good Practices

1st edition I 2018

PATIENT FOCUSED MEDICINES DEVELOPMENT

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Patients and community feedback on PLSs (Plain Language Summaries)

Organisation:
Viiv Healthcare/ GSK

Basic Information

Our company has been integrally involved in the cross-sectoral (industry/academia/regulators/patients/HCPs) initiatives coordinated by the TransCelerate and Harvard MRCT (Multi Regional Clinical Trials) Center, aimed to improve communication of study results to participants and the wider community through the development and dissemination of plain language summaries (PLSs). We also served on the Health Research Authority Task Force, a multi-stakeholder working group responsible for developing the EU Regulatory Guidance on Layperson Summaries (release August, 2017). These initiatives will address the requirements of the upcoming EU regulation (2018) to publish a PLS within 12 months after LSLV (last subject last visit) of a study and support efforts to provide study result to participants.

In 2014, we started their internal work to ensure good preparedness and smooth delivery of this initiative within the company, reach the PLS quality criteria in line with the requirements, and address their patients' expectation to have results communicated as early as possible after a study completion. This early work, well ahead of the applicability of the EU CT Regulatory requirement*, enabled us to understand the challenges and identify pragmatic solution to help inform external work in this area. We also wanted to ensure that the developed PLS would be understandable and clear for patients and the general public (all of whom are patients as well).

The following are two examples of feedback sprints authors conducted:

- Pilot PLS (COPD) reviewed by EUPATI trainees (2015) and
- 2 PLSs review (COPD study 113108 and study 115151 SLS) via Crowd sourcing Amazon Turk online platform (2017).

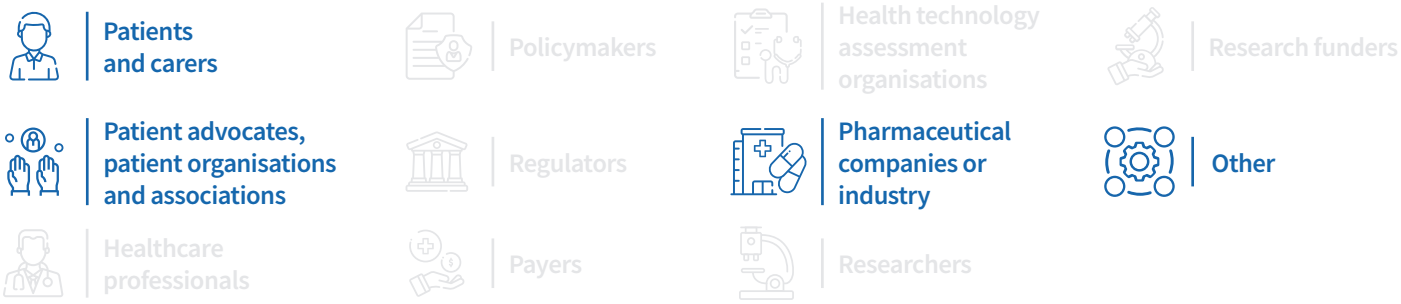
Detail on the methodology:

1. Summaries of Clinical Trial Results for Laypersons released June 2017
   https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-10/2017_01_26_summaries_of_ct_results_for_laypersons.pdf
2. Layperson Summaries of Clinical Trials: An Implementation Guide (Download here)
3. Clinical Data Transparency
   http://www.transceleratebiopharmainc.com/assets/clinical-data-transparency/

   * EU Regulation No 536/2014, see https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-1/reg_2014_536/reg_2014_536_en.pdf

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

Which stakeholders does this PE project involve?



Other: Wider community: caregivers, media and treatment activists

The quality of patient engagement

1. Shared purpose

The Harvard MRCT workgroup was co-led by a patient advocate. The multi-stakeholder discussions and ultimately the guidance and toolkit released, represented a balanced and comprehensive approach based on significant input and discussions among the many stakeholders. This work then formed the basis of the HRA Task Force's guidance which was further vetted and refined specifically for alignment and clarity to enable compliance with the EU Clinical Trial Regulation as to Layperson/Plain Language Summaries (PLSs) shared with patients and community representatives to ensure good understanding of key study points and addressing all unclear stuff prior final PLS approval and publication.

The first activity in 2015 helped us refine formatting and content to improve clarity. This activity also provided further feedback as to the importance of providing clinical trial results in plain language from the patient perspective.

Pilot 2015: EUPATI interviews - Initial Patient feedback
(feedback on version 1 helped refine contact and improve clarity)

"It is essential and respectful to volunteers and patients who have taken part in a clinical trial."

"Surely it will build up good relationships between patients, the public, and researchers."

"A graph would be useful. I'm very visual."

A patient who spoke English very well as a second language misunderstood the distinction between "inflammation" and "infection".

"feeling of partnership [not] merely a patient to test on - a guinea pig"

"There are three really important sentences, but they are hard to search out [Maybe] if there were headings?"

The second activity in 2017 helped us to understand patients/wider community preferences and expectations from a communication on study results as well as common understanding of the pilot PLS:

"Really can't stress enough- this was incrediblywell compiled & easy to process. I started this under the impression that it would ultimately be mind-bendingly tedious given the subject matter, and was very pleasantly surprised to find the exact opposite. Thanks!"

"I enjoyed this study very much. I'm always interested in finding out whether the medicines big pharma companies churn out are worth the price or side effects."

"It was very interesting. Especially so since I may have the beginnings of COPD but have not yet been diagnosed."

"This is important if the manufacturer wants it to be read by this group [lay public] which apparently it does."

"I think it's a great idea because far too much information provided to patients (especially package inserts) is practically unreadable unless the patient has a medical background."

"As someone who has a relative who suffers from COPD this is a very interestingtrial to read."

"Thank you, this is important research because it is important that people understand this information and that is it not written in a way that scares people away..."

"It was understandable; plus, it was interestingto me since I have COPD."

"It's a shame it wasn't effective. Regardless, this is why drugs we so expensive by the time they get into the consumer market."

2. Respect and accessibility

At the pilot stage, we invited the EUPATI trainees (patients who completed the EUPATI course on R&D procedures and medicine development continuum) to have a preliminary feedback on the approach used. At the stage of the review via the Crowd-sourcing Amazon Turk online platform we assessed feedback on understandability, likeability and areas for improvement from patients and other stakeholders: advocates, media and treatment activists, caregivers and others (118 participants for the COPD Study 113108 and 115 for the SLS study 115151). We focused on the US geography, three age brackets, varying education levels and all had English as their primary language.

Overall, the PLS development process was highly appreciated by patients and community representatives; the response rates exceeded our expectations.

3. Representativeness of stakeholders

The feedback received on the developed PLS drafts has reflected the interests of several stakeholder categories: first and foremost – patients, caregivers, media and treatment activists, members of patient organisations and other community representatives. The only limitation was around the English-speaking audience in Europe (for the pilot stage) and in the US (for the Amazon Turk platform), as all drafts were written in English. We envision opportunities to obtain feedback on PLS in other therapy areas, clinical trial phases and on discrete issues (e.g. whether a particular graph or chart is understandable etc.)

The diversity of views/opinions was reflected in the following statements from participants regarding possible PLS improvement:

"I would like to see a little more explanation about a few points such as pulse wave velocity and the m/sec value."

"Improving it would make it longer more complicated, and then it would lose its ease of use. I'd leave this way."

"No, the document seems to provide the minimum amount of information while still serving is intended purpose which appears to me to be the goal."

"If the studywas a success, not a success or if it needed more data to be determined successful or not successful. It showed results but never uite stated that I saw if it was worth it."

"I thought the paper could be longer and more involved but then it would be complex and not as readable to the average person."

"Add a glossary to explain terms used in the summary."

"I would like to see the results for smokers vs. non-smokers"

"It would have been better if the medicines tested were useful."

4. Roles and responsibilities

All participants were instructed prior to PLSs review and accepted the terms and conditions (at the pilot stage and prior starting the Amazon questionnaire). Participants from Amazon Turk crowdsourcing were included if they were rated as high-quality responders within the Amazon Turk environment. Results for Amazon Turk were assessed across 3 discrete age ranges to get acceptable representation as well as for two different PLS (one we internally rated as "easier" to understand and the other "harder").

Internally, questionnaire was developed to assess the understanding of and how well the PLS are received. We assessed understanding by asking responders to provide 3-4 main points from the PLS in open response and then scored as to whether or not the statement was accurate. Thus, we allowed for individual differences in what stood out as important to each but could nonetheless measure if accurate or not. We found that respondents were 94-96% accurate on the 2 PLS selected for feedback (one was simpler and one more complex). We also measured whether there was anything they liked or disliked about PLS and if so, what, and whether there was anything confusing and if so, what.

Feedback received was assessed for quality of responses and completeness of questionnaire and found to be acceptable by internal team for the intended purpose.

5. Capacity and capability for engagement

Having in mind the key target audiences for PLSs as study participants (patients, including expert patients) and community representatives (caregivers, advocates and activists) we asked them to review the prepared drafts and provide feedback on wording/terminology, visibility, format, understandability and the ways data presented.

PLS development/review projects have made us understand that patient and general public input is needed to assess [the reader's] understandability. Extrapolation and repeated assessment in different populations will likely be needed to assess how well-received the PLS are for a given population. We strive for understandability at a 12-year old reading level and use health literacy and numeracy principles but in addition, stakeholder feedback is critical to ensure we are achieving a quality PLS that is fit for purpose.

6. Transparency in communication and documentation

The received feedback was documented and presented internally. As we take part in the cross-sectoral initiative as an industry representative, including at the Harvard MRCT, the findings were presented and discussed with broader stakeholder groups (including patient and patient representatives such as at the EFGCP-EFPIA Workshop, Brussels on May 2. 2017). This case study was also presented at the PFMD Task Force meeting on May 9, 2017.

Due to the tight timelines for developing and disseminating PLS, we would not recommend obtaining individual feedback before dissemination. However, obtaining feedback generally and on specific issues and

incorporating lessons learned and best practices moving forward is quite important. Crowdsourcing is useful for feedback from a general audience. Feedback from study participants is also important, keeping in mind that perspectives can differ by therapeutic area, geography, phase of study, age of participants, etc.

Written materials, documents and records are available internally and/or externally.

7. Continuity and sustainability

We have developed a standard operating procedure (SOP) for development, translation and distribution of PLS with particular consideration of a mechanism for getting feedback from patients (study participants) and the wider community on developed PLS to improve their quality.

The PLS development strategy as an essential part of our commitment to R&D transparency and disclosure of study results has been substantiated and approved for the period of 2018-2021.

Results and outcomes

Building in quality by strategic writing and review process which help establish roles and responsibilities as well as actual process for writing and reviewing by each involved stakeholder. This well-defined writing and review [process] provided clarity around who is reviewing for plain language, who for scientific accuracy and who for technical review, for example. Often there is a need to go back and forth between plain language and subject matter experts to assure that accuracy remains, however, subject matter experts are often not adept at writing in plain language.

Another learning has been the benefit of our internal work in developing a PLS template and detailed instructions so that we can hand to our selected external vendor now in 2018 and help develop their capability and understanding of what we want.

Rollout plans are to post PLS to GSK Study Register along with translation to local languages. We are scaling up this capability and will expand beyond the EU CT Regulatory requirements for PLS.

Positive impact for specific medicines development phases

Although this case study reflected the post-phase IIIb experience, PLS may be reviewed after any phase study/ clinical development milestone (of course, with the biggest consideration of phase II-IV interventional studies) and we are currently seeking feedback from study participants. This approach contributes to our values:

- Be focused on the patient;
- Respect for people;
- Act with integrity;
- Operate with transparency

Direct or indirect positive impact for patients

- Explore and/or pilot co-creation of PLS to ensure clarity and consistent understanding by several groups of patients and community representatives;
- Awareness of study results as early as possible;
- Satisfaction of study participants (if selected as PLS reviewers) making them proud of any to have contributed to the study and any knowledge gained as a result.

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

- PLS team within the company: lessons learned and areas for improvement;
- Cross-sectoral project teams (other companies, (in the development of an Implementation Guide and Recommendations for Drafting Non-Promotional Layperson Summaries – both through the R&D Organisation, regulators contributed input toward an aligned PLS guidance from FDA, EFPIA/industry associations (Reflections paper), HCPs and investigators);
- Public health benefits: increase transparency and openness within healthcare systems. The public (as patients) may feel empowered with increased knowledge and understanding of clinical trials and are better able to discuss their condition. Also, they may develop greater trust in the drug development process.

Lessons learned

After concluding the Amazon Turk second sprint, the assessments are the following:

- There is a right direction;
- This Amazon Turk system offers a fast way to see if specific portions of a PLS are understandable;
- Great method for quick check where needed;
- Could be used to test for specific issues (e.g. are more graphics/wording/glossary preferable?)
- Is there benefit in considering a head-to-head comparison with scientific summary or publication abstract?) or clarity/confusion of part of a PLS as an adjunct?
- Significant expertise has been gained, which can help with vendor selection and onboarding

Methodology, involvement of patients and the general public for feedback.

To seek feedback from study participants as a specific target audience.

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PATIENT FOCUSED
MEDICINES DEVELOPMENT

Understanding patient and stakeholder needs alongside the patient journey*

(*Update in Jan 2018: This project has been discontinued due to internal matters)

Case from a Pharma Company ("Company")

The PFMD
Book of Good Practices

1st edition I 2018

Understanding patient and stakeholder needs alongside the patient journey*

(*Update in Jan 2018: This project has been discontinued due to internal matters)

Case from a Pharma Company ("Company")

Basic Information

The Initiative had the goal to understand the needs along the patient journey and to co-develop a solution that helps patients managing their daily life covering the most important needs. As early as possible, stakeholders' needs alongside the patient journey were collected and matched, so that the most important needs could be identified. As a second step, based on the prioritized needs, a solution gets co-created and tested. The solution will get implemented after launch. The project itself got stopped due to prioritization of another digital health solution, which was more advanced. However the insights could be used for the other patient solution.

The assessment of the quality of PE practice

Steps towards the Initiative's solutions in a non-oncology disease setting:

1. Multi-stakeholder advisory board with patients, patient experts, nurses and physicians has been conducted by asking their challenges, needs and feelings alongside the patient journey (please see details on the next page).
2. Results from advisory board have been confirmed with market research and had been matched to internal strategy.
3. External landscape analysis has been conducted to check what solutions are available to patients with the disease.
4. Potential solutions with patients, patient group representatives, nurses and physicians, that were present in the first advisory board, have been co-created. Three ideas were defined to move forward.

1. The solutions have been tested with other patients and physicians and revised.
2. The solution is going to be implemented within test countries and in collaboration with local patient groups.

Overview of working sessions

Session	Groups	Exercise	Outcome
Working Session I (75 min. plus 15 min. walkthrough during coffee break)	2 mixed groups (collaging is an individual task therefore group composition does not matter)	**Collage:** select picture and create a collage that reflects how you experience life with the disease **(For HCP: create a collage that reflects how you feel about treating patients)**	Emotional understanding of: • The patient's feelings, problems, needs, state of mind • The HCP's feelings and emotions when treating patients
Working Session II (60 min. plus 10 min. per group for sharing of key points in plenary)	Separate groups (Patients/PAGs and HCPs)	**Needs along the patient journey**	• Talking through patient journey phases (awareness, diagnosis, treatment and adherence) • Mapping of needs along the entire patient journey
Working session III (60 min.)	Patient/ PAG group HCP group	**Focus - Life with the disease:** impact on social life and living with the disease and symptoms **Focus - Interacting with patients:** typical questions, concerns, dealing with patients' emotions	In-depth understanding of daily challenges, struggles and needs in focus areas that most relevant and actionable for Pharma Company (basis for solution development)
Working session IV (30 min. plus 10 min. per group for results sharing in plenary for III and IV)	Same groups continue from working session III	**Solution brainstorming** for focus areas: **information needs and other solution ideas**	• Longlist of ideas that addresses identified needs in the focus areas • Voting and definition of 3-5 ideas

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

Research and discovery phase | Pre-clinical phase | **Clinical study phase 1-3** | Health technology assessment | Regulatory review and approval or registration phase | **Post-registration/ -launch activities** clinical study phase 4 | Other

Which stakeholders does this PE project involve?

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

*HCP who were considered as local and/or global TAEs

The quality of patient engagement

1. Shared purpose

Company involved patients and patient representatives as well as other stakeholders from the start until the end of the project, by understanding the needs, co-creating the solution and by implementing it together.

Examples of the questions and topics planned for the advisory board to get discussions started.

1. Select pictures and create a collage that reflects how you experience life with MS or how you experience to treat MS patients:
   - Why did you use this picture?
   - Why is … important to you?
   - How do you deal with …?
   - What is the impact of … on your daily life?
2. Patient Journey:
   - What was it like for you to go through theses steps?
   - What were particular challenges you faced?
   - Why were certain things difficult and what are the implications of that?
   - What are things that went well?
   - What support did you receive?
3.

   a) What are your (an MS patient's) biggest concerns when getting up in the morning?
   b) Where do you feel the biggest challenges regarding your social life being an MS patient?
   c) Where do you struggle most in daily life with the MS disease and the related symptoms?

   - Contracts and track records are in place to meet Company's compliance and legal requirements.
   - After each of the meetings the Company have assessed the quality of interaction with participants.
   - The final solution still needs to be implemented and needs to include metrics to measure the success. (Edit Jan 2018: initiative has been disconnected)

2. Respect and accessibility

- In the workshops a way of verbal and nonverbal communication was used with (for example) pictures, as feelings may be better expressed with images.
- Very strong listening skills as well as moderators who are having the empathy to accommodate all stakeholders have been used.

- There was a follow up in 1:1 meetings to clarify all individual perspectives and to understand if anything in the group was missing .
- HCPs and patients were first split in the workshop. However, the participants wanted to listen to the other perspective, so they handle a joined meeting the following time.
- Participants' surveys helped to improve the project and the way of working with the stakeholders.

3. Representativeness of stakeholders

The most difficult task was the identification of the right participants*. They were identified via their affiliates and patient group relations people. Qualitative market research was used to complete their findings.

*1) Patients a) just diagnosed and b) living with the disease for longer time from the 3 pilot countries

2) HCPs who were considered as local and/or global TAEs

Company could match the outcomes of the advisory board and the market research very well.

4. Roles and responsibilities

Follow up meetings 1:1 as well as updates via emails informed about the progress. Once the final implementation is done, the feedback loop will be ensured.

This PE project owners used feedback from participants to check if what they did was appropriate to achieve this criterion.

The feedback of the workshop participants was always very good and didn't have a lot of feedback for improvement, other than mixing the stakeholder groups more.

5. Capacity and capability for engagement

In this project, the focus was on questions and solutions for daily life. Therefore, no further skills or knowledge from the participants were necessary. The stakeholders were informed alongside the process of the questions and tasks.

Stakeholders did not look for clinical trial experience, knowledge of drug development. They selected participants on the basis of the following criteria:

- How long they were living with the disease
- Geographical scope
- Female and male ratio

This PE project owners used participants surveys to check if what they did was appropriate to achieve this criterion.

6. Transparency in communication and documentation

Contracts and track records are in place to meet Company's compliance and legal requirements. As per Company's guidance, it is mandatory to document any activity with external engagement, e.g. advisory boards. There is a need to work on the share best practice in house and get local approvals for the engagement.

Documents were shared with the workshop participants before, during and after the project. Before the meeting only the objective was shared. After the meetings the outcome and the next steps including the final testing were shared.

7. Continuity and sustainability

The scope of the project as well as the role of each individual involved was always very clearly communicated via the contracts, as well as in the meetings. Whenever questions occurred, they were appropriately addressed.

Results and outcomes

PE practice led to the following measured outcomes:

- Profound understanding of patient and stakeholders needs alongside the patient journey.
- Potential solutions that may impact the life of the patients.
- This Initiative showed Company that there is a very high unmet need in giving the power to patient to live their lives.
- With the co-created solution, which is still in iterations, Company believes that it can help patients to achieve their goal to be proactive in living a normal life.
- The process Company has chosen, is a very solid approach, however, final implementation seems to be more challenging than anticipated due to cost and compliance considerations.

Positive impact for specific medicines development phases

- Improvement of self-empowerment and therefore also clinical care.

Direct or indirect positive impact for patients

- Living a normal life.

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

- Helping HCPs.

Lessons learned

The project was stopped, however the way of working to assess the needs along the patient journey and how to co-create solutions already has been used by other disease areas.

It was very difficult to find the right participants with the right profile and without compliance/legal restrictions. However, once Company had identified the stakeholders, the biggest learning was, that a multi-stakeholder approach gives the best overview to the questions Company had providing the variety of different perspectives. This made it easier for Company to prioritize the key findings.

Involvement of the stakeholders alongside the project including the multi-stakeholder perspectives.

It's important to give enough time to plan and identify the participants.

Annex 1: How to read the Book of Good Practices

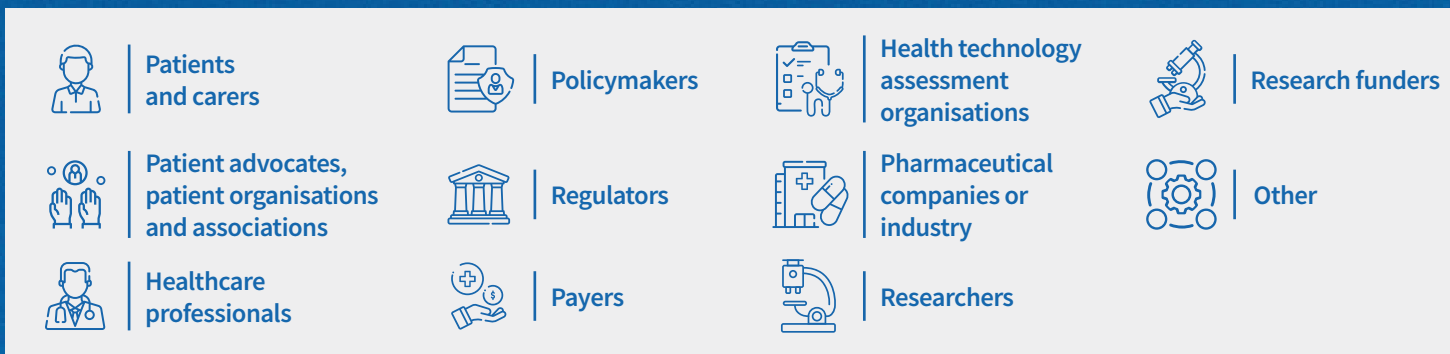
The Book of Good Practices cases are all structured in the same way as the Patient Engagement Quality Guidance. You will find that each case has a basic description, followed by icons to show in which phases of medicines continuum they fit in and which stakeholders they have involved in their work (see description of icons below). In section 2 these cases will describe how they reached each of the 7 Quality Criteria. You will see from the wheel in the beginning, which of the Quality Criteria they exemplified in (judged by an external group of reviewers). Finally, you will find the results and outcomes of each case and the lessons learned.

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

Research and discovery phase | Pre-clinical phase | Clinical study phase 1-3 | Health technology assessment | Regulatory review and approval or registration phase | Post-registration/ -launch activities | Other

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

Which stakeholders does this PE project involve?



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

Annex 2: Descriptions of the Patient Engagement Quality Criteria

1. Shared purpose

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

2. Respect and accessibility

This refers to (1) respecting each other, and respectful interactions within the project to be established among partners, and (2) openness to and inclusion of individuals and communities (to the project) without discrimination. Considerations to ensure good conditions to implement the project should be made from the beginning. For example:

- simplification of wording
- budget and payment considerations
- cultural adaptations to procedures
- practicalities such as meeting timing, location and format
- accessibility of project materials
- written co-developed rules of conduct

Accessibility to participate may be facilitated by enabling multiple ways to involve stakeholders who could benefit from and/ or contribute to the project. For example, patients with cognitive impairment might need more time to go through project material, or need printed versions rather than electronic documents or PDFs for easier reading.

3. Representativeness of stakeholders

This refers to the mix of people you involve, which should reflect the needs of the project, and the interests of those who may benefit from project outputs (for example, target population). Consider diversity in expertise, experience, demographics, and other relevant criteria for inclusion. When selecting PE stakeholders, patients, attention will be given to awareness of the diversity required to achieve visible representative voice.

4. Roles and responsibilities

This refers to the need for clearly agreed, and ideally co-created roles and responsibilities, in writing, addressing that all aspects of project needs will be established upfront and revisited regularly.

Annex 2: Descriptions of the Patient Engagement Quality Criteria

5. Capacity and capability for engagement

This refers to (1) capacity as having relevant and dedicated resources from all stakeholders (for example, providing a dedicated point of contact by the sponsor and having allocated sufficient time by all stakeholders to allow genuine engagement); and (2) capabilities for all stakeholders to enable meaningful engagement. (For example, the level of knowledge, expertise and training stakeholders might need to deliver PE activities throughout the project).

Consider supporting stakeholders to build the required capacity and capabilities for this project in different forms of training both with sponsor organisations and with each stakeholder (for example, helping to understand the context, processes, involved terminology etc.).

Both capacity and capability building are intended to facilitate participation and lower barriers to collaborate. Stakeholders can be given access to learning resources and given dedicated support (if needed). Capability needs may vary depending on the project needs, but also e.g. personal circumstances of PE representatives.

6. Transparency in communication and documentation

This refers to the establishment of communications plan and ongoing project documentation that can be shared with stakeholders. Communication among stakeholders must be open, honest and complete.

In addition, adequate up-to-date documentation must facilitate communication with all stakeholders throughout the project. Consider proactively and openly sharing progress updates throughout the project externally. In addition, communicating outcomes of the project to all stakeholders and how their contribution was of value to the success of the project is critical.

7. Continuity and sustainability

This refers to the smooth progression of the project, as well as efforts to maintain ongoing relationship with stakeholders. Consideration should be given for the role of stakeholders beyond a single project. When starting the project, consider including in your project plan the actions needed for maintaining expected flow of the project from beginning to end.

Create a plan to nurture relationships with your partners and stakeholders involved during the project, and when needed and requested, beyond the project as well. For all stakeholders successful planning and personal and organisational resilience should be anticipated.