



PATIENT FOCUSED
MEDICINES DEVELOPMENT



Patient Engagement
Book of Good Practices



The PFMD Book of Good Practices

3rd edition | 2020

 @PFMDwithPatient

#PEQualityGuidance
#PEQualityCriteria
#BoGP

 www.PatientFocusedMedicine.org
www.PEMSuite.org

Message from PFMD

Dear reader,

PFMD began its Framework Building workstream in 2016 to respond to a need for a practical and actionable patient engagement framework that helps relevant stakeholders to start or improve their patient engagement activities. One of the first outputs — the [Patient Engagement Quality Guidance \(PEQG\)](#) — was a co-creation effort of more than 100 experts globally, representing over 50 organisations.

In addition to a standardised patient engagement framework, there was also a need to see examples of what “good patient engagement” looks like. In response, PFMD created the **Book of Good Practices** to accompany the practical framework in the Patient Engagement Quality Guidance.

You are now exploring the third edition of the Book of Good Practices. Throughout the past two editions, we have seen a tremendous increase in patient engagement activities as well as the increasing quality and care in involving patients as true partners. If you are curious to find out how these initiatives were selected for the Book of Good Practices, you can read more about the review method in our publication in [DIA Global](#).

These and all PFMD’s practical tools for patient engagement are integrated in the [Patient Engagement Management \(PEM\) Suite](#) - a comprehensive global hub for practical tools to plan, assess and execute any patient engagement initiative.

We hope this book will inspire and help you in your patient engagement journey and we encourage you to explore all the tools at your disposal within the PEM Suite.

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

PFMD Team

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THE BOOK OF GOOD PRACTICE INITIATIVES ARE ALSO AVAILABLE IN [SYNAPSE](#).

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<https://pemsuite.org/bogp/>

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MEDICINES DEVELOPMENT



Patient Partnership Program

Organization: AstraZeneca

The PFMD Book of Good Practices

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Patient Partnership Program

Organization:
AstraZeneca



Basic Information

Project Description

At AstraZeneca, we recognise patients as people first and putting them at the heart of what we do. We believe it is crucial to get input from real patients, for real patients, to ensure that their voices are heard at every step of the product discovery and development process. Through the **Patient Partnership Program (PPP)**, AstraZeneca has fundamentally changed the way it thinks about researching and developing new, life-changing medicines.

Start Date:

November/2016

End Date:

Ongoing

Geographic focus

- Global
- Continental/Regional
 - Europe
 - North America
 - South America
 - Asia
 - Africa
 - Antarctica
 - Oceania
- National

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

- Research
- Access
- Care Delivery
- Education
- Advocacy
- Gap analysis
- Organization/
System development
- Policy











Which phase(s) in the patient care journey does the initiative align with?

- Prevention
- Newly Diagnosed
- Clinical Trial
- Other
- Onset of symptoms
- Treatment
- Long Term Management

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



Which stakeholders does this PE project involve?

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

The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

Together with a cross-functional governance team comprised of patient engagement, R&D, patient centred science, compliance, data privacy, legal, nominated signatory, procurement and patient safety, we have developed an approved, streamlined operational process for the PPP with a 4-year proven track record across 11 disease areas providing a scalable and sustainable model for global success. Patients from around the world interested in the program are first screened via the Patient Partnership Program website. A third-party company then interviews and recommends patients for the program based on a mutual fit for both the patient and AstraZeneca. PPP members are contracted for a period of one year to ensure that engagement with the patient experts is effortless and seamless. This has driven utilization of the program across AstraZeneca in all functions from early development to commercialization, ensuring that the patient voice is at the forefront of everything we do.

What is your stated “shared purpose”?

Each project lead completes an engagement form to clearly state outline the activities for the project, the benefit from the activity from the AstraZeneca perspective and the benefit from the activity for the patient

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

This is confirmed and agreed when the engagement form is completed.

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

During the initial interview process, potential PPP members are asked about their goals for joining the program and are trained on our vision and purpose for the PPP, its value and desired outcomes.

At what time points?

The shared purpose of each activity is reviewed at the beginning and end of each engagement to ensure the teams action the input from the patients and then it is shared with the patients involved.

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

We take a patient-centered approach by first listening to the words and phrases patients tell us they like or don't like us to use, and what resonates most with them. This allows us to communicate in patient friendly language, eliminating acronyms and industry/company jargon as much as possible. We treat patients with care and empathy,

mindful that they may be experiencing pain, discomfort, or fear. We recognise that patients are not working to our timeline or schedule, so we are conscious and understanding about response time, ability to commit to activities, and potential for them to need to cancel or reschedule appointments based on how they feel on any given day.

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

During the first contact with patient at initial interview potential PPP members have an opportunity to get better understanding of AstraZeneca vision, purpose and goals of this program. At the same time, we get to know the patient as more than their disease, learning more about their values, strengths and interests. These efforts are continued throughout the program. In our pre-read materials, we include information about the activity and what key questions and topics the teams want to cover during the discussion, but we also include an opportunity for patients to share any other comments to bring to our attention.

3. Representativeness of stakeholders



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

PPP members are recruited from around the world to ensure that we have representation from both men and women, in a variety of countries, cultures and healthcare systems. Currently PPP members must communicate well in English to interact with our Global teams, but we are expanding the program into local markets to ensure that the patient voice is heard irrespective of language.

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

Our PPP patients are from around the world (>130 patients) with expertise across 11 disease areas [Asthma, COPD, Nasal Polyposis, Lupus, EGPA (Eosinophilic Granulomatosis with Polyangiitis) , EoE (eosinophilic esophagitis), HES (hypereosinophilic syndrome), T1D (type 1 diabetes), T2D (type 2 diabetes), Ovarian cancer, Lung Cancer]

4. Roles and responsibilities



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

As the various PPP partners are engaged on a very regular basis by teams from clinical development, medical affairs, marketing, market access and digital health, the patient insights we have gathered have led to numerous positive changes and innovative solutions over the last 4 years.

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

Before PPP partners decide to work on a project, they are provided the opportunity to review project details in a project agreement e.g., PPP partner’s role and responsibilities and time commitments. Upon agreement of the project details and willingness to participate, PPP partners inform the AstraZeneca Patient Engagement lead, who will in turn inform the team executing the engagement to begin.

At the end of every year, we publish a “PPP Year in Review” newsletter for our Patient Experts and internal teams to describe the results of these projects and ensure transparency of the value our PPP Patient Experts bring to the development of AstraZeneca’s products and solutions.

At what frequency have you checked this in?

The shared purpose of each activity is reviewed at the beginning and end of each engagement to ensure the teams action the input from the patients and then it is shared with the patients involved.

5. Capacity and capability for engagement



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

Together with a cross-functional governance team comprised of patient engagement, R&D, patient centred science, compliance, data privacy, legal, nominated signatory, procurement, and patient safety, we have developed an approved, streamlined operational process for the PPP with a 4-year proven track record across multiple therapeutic areas (TA) providing a scalable and sustainable model for global success.

With the goal to improve patient experience across all stages of the disease, from prevention and awareness to wellness there is an increased need for patient collaboration across product lifecycle, with involvement of cross-functional teams. Hence, we are planning a PPP extension to cover more disease areas and broader patient population with inclusion of multiple countries.

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

The Patient Engagement Lead supports all discussions that AstraZeneca teams have with the PPP. This ensures that all material shared with the PPP members is in a patient friendly format and all participants have an opportunity to contribute to the discussion.

6. Transparency in communication and documentation



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

As an example, when conducting a discussion for our clinical study protocols we provide patient friendly pre-read materials at least 1 week ahead of the webex discussion so the PPP participants are prepared for the call.

In the pre-read we include information about the study design, study visit schedule and procedures included at each visit. We also include the list of questions and topics the team at AstraZeneca would like to discuss, but encourage the PPP partners to include any comments they have.

With the global study team (physicians, clinical scientists and operational team members) on the webex with the PPP participants it is an opportunity to discuss with the patients first hand where they see the challenges with the study design, where they suggest making modifications and what in addition they would like us to consider.

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

At the end of every year, we publish a “PPP Year in Review” newsletter for our Patient Experts and internal teams to describe the results of these projects and ensure transparency of the value our PPP Patient Experts bring to the development of AstraZeneca’s products and solutions.

7. Continuity and sustainability



What did you do to achieve this criterion?

As the various PPPs are engaged on a very regular basis by teams from clinical development, medical affairs, marketing, market access and digital health, the patient insights we have gathered have led to numerous positive changes and innovative solutions over the last 4 years.

How did you gather feedback on what you have done?

Upon completion of each project, the team involved sends a project report to the Patient Engagement Director outlining any changes/ improvements that have been implemented following patient input. Furthermore, at the end of every year, we publish a “PPP Year in Review” newsletter for our Patient Experts and internal teams to describe the results of these projects and ensure transparency of the value our PPP Patient Experts bring to the development of AstraZeneca’s products and solutions.

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

PPP partners are not obligated to participate in an activity if invited to do so. They have the right to terminate participation with the Patient Partnership Program at any time. Each PPP participant is contracted for a year at a time and 99% of our members return year on year. We are also constantly recruiting new members to the PPP to ensure that we have long term growth of the program across our teams and markets.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

Example 1 - Clinical Trial Optimization project

Protocol Design

- Patients no longer have to wait 2 days for symptoms to be present before contacting the site; they are encouraged to reach out to the Investigator to avoid potential worsening
- Patients provided input to the team on study materials that they planned to use for recruitment and retention, which resulted in the team refining the materials (symptoms definition, screening visit, managing exacerbations etc.)

Improve study experience for Patients (Local Regulations may apply)

- Team is checking with vendor that manages e-diary to see if they can have a read aloud feature
- Team is evaluating what is possible in terms of support during long visits (food)
- The site will be educated that at long visits patients can use nebulizer and eat/drink if necessary
- Team is looking into vendors for Patient transportation support
- Team is looking into other options for eDiary and phone apps after the 56 week treatment portion

Sub-Study

- Patients provided comments to enhance Clarity of Sub-study including all the assessments
- Patients commented that 56 weeks is a long time and wanted to understand how could they participate and still be able to go on vacation – Team will ensure there are clear instructions so patients know what they need to do
- Nasal brushing - Team explained the procedure to the patients. Patients wanted to see a video-a video option was sent to the patients after the discussion. Patients felt viewing video was helpful and re-assured concerns. Team will work to provide a video for the sites to use with patients
- Patients expressed frequent weekly sampling at home for Biomarker collection was burdensome – Team has taken feedback onboard and revised Sub-Study protocol to collect samples every two weeks being mindful of patient concerns.

Example 2 - Patient-centric physician education

Objective

Provide patient perspective on content for the development of a physician education program to advance clinical practice and new treatment options.

Outcomes

Based on patients' feedback incorporated practical components into the training, added questions that patients want to ask their physicians, included information that patients need to hear when diagnosed with a severe condition. This optimised training program supports improvement of patient-physician communication and leads to more positive experiences for patients and physician.

Example 3 - Optimizing Patient Support Programs

Objective

Understand the role that PSPs play to enhance the patient experience, improve patient outcomes and increase adherence to treatment programs.

Outcomes

Patient input significantly improved content and optimized delivery of Patient Support Program to ensure it is meeting patient needs.

Positive impact for specific medicines development phases

In Clinical Trial Optimization project the discussion with the PPP provided an improved study design based on modifications as described above. In addition, the materials and operational input provided allowed the team to have discussions with the various vendors supporting the study to modify prior to final development. Timelines within drug development are always being pushed. The PPP provides clinical study teams with access to patients for immediate discussion about the protocol design. Developing the patient friendly material is typically the rate limiting step in the process, but once the teams follow the template provided they are able to easily put the material together so we are able to provide the patients with an understandable format of the study to discuss.

The study teams value the activity as it is an opportunity to listen to what the patients need. Very often the elements of the study that the study team are most concerned about are not the elements the patients find to be a concern.

Direct or indirect positive impact for patients

In Clinical Trial Optimization project, the PPP patients were able to provide their perspective of how living with the disease impacts their ability to participate in the study as we had originally designed it.

In Patient-centric physician education the optimised training program supports improvement of patient-physician communication and leads to more positive experiences for patients and physicians.

In Patient Support Program patient are experiencing customized program to their needs with optimized delivery.

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

In Clinical Trial optimisation project the study team participated in the discussion with the patients and was able to hear the feedback directly from the patients on where they saw challenges to their daily life if they were to participate in the study.

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?

Yes

No

Lessons learned

The PPP is not a transactional project, but rather a long term, established program for obtaining patient insights, embedding these in our strategy and enabling co-creation with patients for patients. We learn from this year on year by listening to what worked and what didn't via patient feedback loop.

About AstraZeneca

AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development and commercialisation of prescription medicines, primarily for the treatment of diseases in three therapy areas - Oncology, Cardiovascular, Renal & Metabolism, and Respiratory & Immunology. Based in Cambridge, UK, AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. Please visit astrazeneca.com and follow the Company on Twitter @AstraZeneca.

About the team



Ana Marija Gjurovic

Head Patient Centricity, AstraZeneca
BioPharmaceuticals Medical

Ana Marija brings over 20 years of extensive experience in the international pharmaceutical environment across multiple geographies (Eastern Europe, Switzerland, South Africa, Middle East). She held leadership positions in Clinical research, Medical Affairs and Patient Centricity in cardiovascular and metabolic, respiratory and oncology therapy areas on local, regional and global level.

She is an enthusiastic and energetic leader with enhanced passion for patients. She strongly believes that only a collaborative work of all healthcare stakeholders based on complete understanding of patients' experiences and needs can bring significant advancement in therapeutic and disease management and deliver best possible patient outcomes and experiences that they care about most.

Ana Marija holds a Medical Doctor degree from the University of Zagreb, Croatia and an MSc in Pharmacotherapy from Witwatersrand University, Johannesburg, South Africa.



Petrina Stevens

Patient Engagement Director CVRM,
AstraZeneca BioPharmaceuticals Medical

A healthcare professional leader for the past 15 years across international geographies (Australia, UK and Switzerland) within medical device and pharmaceutical industries. Petrina's experience and expertise encompasses local and global strategic global patient services and patient engagement.

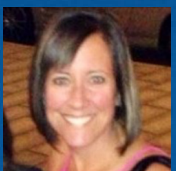
Petrina brings true patient centric thinking into global strategy development, with a view to enhance patients' experience and ultimately measurable outcomes. To this end, she is passionate in the partnering with patients and other critical stakeholders as they increasingly demand and play an active role in managing their own disease.



Marie Eckerd

Patient Engagement Director Respiratory and Immunology, AstraZeneca BioPharmaceuticals Medical

Marie Eckerd has more than 30 years in the pharmaceutical industry across biopharma and biotechnology companies. She began her career at Wyeth-Ayerst then IBEX Technologies where she held leadership positions in Clinical Data Management and Project Management over the course of 15 years. For the past twenty years, Marie has been at AstraZeneca first as a Director of Clinical Development for cardiovascular, respiratory, and anti-infective programs, then as a Feasibility and Recruitment Partner. In that position she was responsible for leading end-to-end study feasibility using data-driven predictive models to support the global allocation of oncology studies, particularly in the lung cancer indication. Currently, as the Global Patient Engagement Director for Respiratory and Inflammation, Marie brings a deep commitment to the patient experience and has brought these insights forward across many indications. She is committed to partnering with patients, physicians and the broader healthcare community to embed the AstraZeneca philosophy to put patients first.



Michele Teufel

Patient & Site Engagement Lead, Patient Insights & Solutions, AstraZeneca BioPharmaceuticals R&D

Michele has been working over 20 years within Clinical Operations in various roles where she delivered early and late phase clinical studies across various therapeutic areas. She was responsible to ensure project standards and scientific requirements from study design concept through study closeout.

Currently, Michele is working within Patient Insight and Solutions team which supports teams across Clinical Operations within AstraZeneca. In this role, she works with clinical development teams to gather patient and site input to our programs as we are designing. This work is allowing teams to directly hear the feedback and influencing changes to protocols.



Rosie Tremlett

Associate Global Patient Affairs Director – Oncology Patient Affairs, AstraZeneca Oncology Business Unit

After graduating from The University of Glasgow Rosie joined the Pharmaceutical Industry & gained expertise in a number of roles over the past 30 years.

She has always work in the field of oncology & her passion is making a difference to patients & families lives both in terms of living with the disease & quality of life. She is committed to eliminating cancer as a cause of death in the future . We must listen & act on what people living with cancer tell us. Its also critical that we engage with patients holistically ,not just a cancer diagnosis.



Kim Williams

Medical Network Head, AstraZeneca
BioPharmaceuticals Medical

After graduating from the University of Birmingham with a Law degree, Kim has had a career in multiple industries including Pharmaceutical, Healthcare service, FMCG, Engineering and manufacturing sectors.

She is an experienced business leader working in strategic planning and operations and procurement roles. She is an effective communicator with a track record of driving agendas to deliver business priorities, a successful builder of multi-disciplinary teams and a strong people manager with experience of developing teams to maximise talent. She believes sustainability, collaboration and helping people reach their potential are at the core of successful delivery.



Lynn Hagger

Experience Design Lead, Global Commercial Digital &
Innovation, AstraZeneca BioPharmaceuticals

With a passion for understanding the unique experiences of patients, Lynn has developed strategies to generate global patient insights that strengthen the development of patient-centric products and services at AstraZeneca. She has a special interest in innovative digital solutions that will help patients better understand their disease experience and self-manage their conditions to improve outcomes. Since 1998, Lynn has served as a successful member and leader of cross-functional teams in Medical Affairs, Marketing, and Clinical Operations, across a variety of therapeutic areas at AstraZeneca. Lynn holds a PhD in Microbiology and Medical Genetics from the University of Toronto, Canada



Helena Chung

Former Global Patient Reported Outcomes Scientist
Oncology, AstraZeneca BioPharmaceuticals Medical

Helena Chung is a global patient centricity leader living out her passion to achieve the best possible patient experience and outcome together with patients, nurses, and doctors through meaningful partnerships, for patients. She started AstraZeneca's first patient centricity program in 2014 together with patients. In partnership with patients, she led meaningful and measurable patient centered initiatives through patient engagement and patient-centered science. Together with patient advocacy groups, oncologists, nurses, and health economists, she led AZ's first EGFRm NSCLC patient experience RWE study that was presented at World Congress on Lung Cancer (WCLC) 2019 in Barcelona.

Helena received M.A. in International Relations from Yale University and B.A. in Political Science from University of California at Berkeley.

Dawn DiCandilo

Medical Execution Director CVRM, AstraZeneca
BioPharmaceuticals Medical

Annex 1: Questions from reviewers

In this section you can see what additional questions the reviewers had regarding this patient engagement initiative, as well as the answers provided by the submitting organisations. The section intends to provide more information and insight into this patient engagement initiative.

General

Q1. Did people with health conditions work with you to set this up?

Yes, we worked with several Ovarian Cancer and Asthma patients to set up the initial pilot program in these TAs, define the process and even to choose the program name and logo.

Q2. What criteria do the third-party company have to recruit people?

There is a screener in place & then a call where the patients go through a set of screening/interview questions. Many patients are recruited as a result of hearing about the program through patient advocacy groups.

Q3. How do you ensure FMV?

FMV guidelines are in place & we refer to these in order to comply with patients home country requirements if any.

Q4. How do you collect feedback on impact and experience?

Every engagement with PPPs has a form that has to be filled out prior to contacting the patients. The AZ group wishing to engage patients must articulate both the impact/value to patients and the company. Patient Engagement Directors check in with patients regularly to understand how their experiences with these AZ groups have been.

Q5. Some patients have been engaged in designing the program. Have they been engaged throughout or just at the beginning of the program?

Yes, patients have been engaged throughout the last 4 years in refining the program as we receive constant feedback on what works and what doesn't. For example, we are currently refreshing our entire recruitment process in response to feedback from patients.

Q6. It is noted that patients are engaged on a contractual basis for a year. Is the contract written in patient-friendly language? Some templates, for example, are available on the PFMD website.

Contracts are written in patient friendly way and we have not faced any issues in signing the contracts and when asked they are happy with the contract. They are also given the opportunity to discuss the contract at the first AZ training or initiation call.

Annex 1: Questions from reviewers

Q7. Is feedback provided to engaged patients on their advice has been used? If so, how is this managed?

The feedback to patients is provided in multiple ways – through summary email with the discussion and actions to every PPP member after the interaction or spot checks with patients on various projects to get their feedback on how the engagement went. This is done by email or phone. When patients are trained into the PPP, they are encouraged to provide continuous feedback and they often do so without prompting- most feedback we get is positive and about how much patients enjoy working on PPP engagements.

Q8. Are there broader program metrics collected to evaluate satisfaction and areas of improvement of the patient engagement activities? If so, please describe.

There is no specific collection of metrics but there are regular check ins to see if the program is working well. The main request from PPP members is to have more projects. They really enjoy participation & see it as a way to contribute to benefit the experience of other patients.

Criteria 1



Q9. What training/preparation (other than on your company vision/purpose of program) do you provide to ensure people feel confident contributing?

Patients go through the training/initiation slide deck with AZ Patient Engagement Director where they are free to ask questions. We also offer support or information at any time, they just need to contact us.

Criteria 3



Q10. What are the different ways people can engage? Just via Webex?

Patients can be engaged in various ways - virtual or F2F meetings, virtual or in person speaker presentation, virtual or F2F panel discussions, online surveys & material review or co-presenting at conferences about our work together.

Q11. Great to see lots of countries and conditions – have you been able to ensure contributions from people with diverse backgrounds and experiences?

Broad representation of countries globally. A requirement to speak English can restrict diversity to an extent, but the aim is to drive & utilize PPP in local countries where the need to speak English is less important.



Consulting a patient and carer group on the delivery of Exoskeleton assisted walking to aid cardiovascular fitness in patients with gait problems due to Multiple Sclerosis

Organization: NIHR Sheffield Biomedical
Research

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Consulting a patient and carer group on the delivery of Exoskeleton assisted walking to aid cardiovascular fitness in patients with gait problems due to Multiple Sclerosis

Organization:
NIHR Sheffield Biomedical
Research



Basic Information

Project Description

A study was designed with the principle aim of exploring whether using an exoskeleton will enable persons with MS to exercise at a moderate intensity and whether they would find this acceptable and safe to do on a regular basis

Multiple sclerosis (MS) is a chronic, recurrent, inflammatory disease of the central nervous system. Reduced mobility due to gait dysfunction is a key problem in the majority of persons with MS (pwMS). This is a major factor resulting in physical inactivity. Deconditioning due to lack of physical activity is in pwMS particularly those who are unable to walk. This can lead to several comorbid conditions such as obesity and diabetes. There are increasing studies suggesting that physical exercise can play an important role in managing symptoms and preventing complications and comorbidities in pwMS. However, exercising can be very challenging for pwMS who have moderate/severe problems with mobility. In this project, we aim to investigate the use of a powered exoskeleton as an exercise tool for people with moderate to severe difficulty walking due to MS. Powered exoskeletons are wearable robots that offer opportunity to persons with lower limb weakness to stand and walk. The exoskeleton provides active assisted training with potentially much less intervention from therapists.

In healthy people, walking, when performed with sufficient intensity and duration, is often cited as an easy and effective means of exercise. However, it is not clear whether pwMS can walk with a powered exoskeleton at speeds and intensities sufficient to positively affect health and fitness outcomes. Thus, in this study, the principle aim is to explore whether using an exoskeleton will enable pwMS to exercise at a moderate intensity and whether pwMS find this acceptable and safe to do on a regular basis. It will also be explored whether training with an exoskeleton improves walking. We will also compare whether walking with an exoskeleton is better than conventional fitness training in terms of fitness outcomes, walking and psychological factors.

If proved successful in people with MS, it is hoped that use of the exoskeleton can be explored for people with other long term neurological conditions such as stroke and Parkinson's disease. Further positive results could lead to commissioning of the exoskeleton at physical fitness centres for people with mobility issues where they can perform assisted walking as an exercise for cardiovascular fitness.

The NIHR Sheffield Biomedical Research Centre host a regular Multiple Sclerosis Research Advisory Group. The patient and carer members of this group were sent draft documents relating to the proposed study in advance of meeting with the Principal Investigator and other members of the research team. The background, aims and proposed methodology were clearly explained in the face-to-face meeting and discussed with the group. We also demonstrated the Exo-skeleton to the group. All feedback from individual members of the research advisory group was minuted, and sent via email follow up for any additions. Patient input from an early stage on the feasibility, wearability and usability of exoskeleton whilst exercising helped influence project design.

Members of the research advisory group suggested including practice sessions for participants before starting the trial intervention. The group also identified the fear of falling whilst using the exoskeleton as an unaddressed priority. We modified the protocol to include two extra sessions before the start of interventions. The protocol now includes two researchers including a qualified physiotherapist to be present with the participants while walking with exoskeleton. One person will walk behind the participant with a manual wheel chair for safety. The solutions also included an external gyro sensor with alarm to alert the trainers if participant falls, a tracking ceiling harness and practice controlled falls with healthy volunteers in early tests to provide reassurance. The researchers took these suggestions on board and have since been back to update the group on the product development and expected timelines.

Start Date:

16/04/2019

End Date:


16/04/2021

Geographic focus

- Global
- Continental/Regional
 - Europe
 - North America
 - South America
 - Asia
 - Africa
 - Antarctica
 - Oceania
- National: United Kingdom

Purpose of the initiative

 **Involving patients** in the lifecycle of medicine development

 **Providing guidance** to others in their PE efforts

Initiative Focus Area

- Research
- Access
- Care Delivery
- Education
- Advocacy
- Gap analysis
- Organization/ System development
- Policy

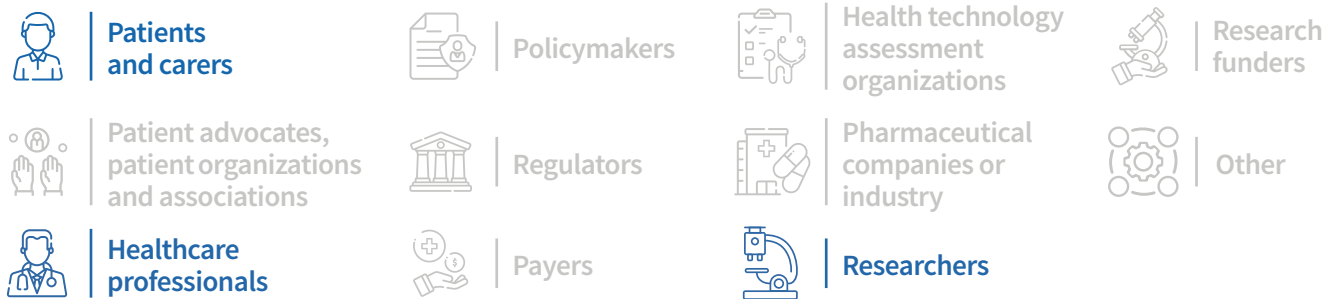
Which phase(s) in the patient care journey does the initiative align with?

- Prevention
- Newly Diagnosed
- Clinical Trial
- Other
- Onset of symptoms
- Treatment
- Long Term Management

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



Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the
PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

The Sheffield Multiple Sclerosis Research Advisory Group (SMSRAG) brings together those affected by Multiple Sclerosis. The main focus of the group is to advise researchers across the region; offering feedback on research proposals and ensuring that patient and carer views and needs are always considered. Clearly laid out information is provided both prior to, and following meetings. The research advisory group meets regularly throughout the year to help researchers shape their research, review documents and influence project design. The advisory group chair acted as a liaison to help organise the activity and make mutually agreed arrangements. Draft documents and a short feedback form were sent to members in advance of the arranged meeting so they were aware of the topic to be discussed.

What is your stated “shared purpose”?

To involve patients and carers in the development of a study to assess the delivery of an Exoskeleton-assisted walking to aid. In particular, advice from representative patients is sought on the feasibility of the study design to help develop an intervention that fits with the requirements of the population group. Incorporating this feedback will give the study the best chance of success in recruitment, retention and delivery on research objectives.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

The overarching purpose of the research advisory group is agreed by members upon joining and by researchers in advance of engaging with the group. The specifics of the activity were agreed by email and in person at the start of the meeting via the chair of the advisory group.

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

Yes. The shared purpose was revisited by a follow up email shortly after the meeting, as well as at a face to face meeting 9 months later.

At what time points?

It will continue to be reviewed through further updates with the research advisory group as the research progresses. The group meet every 3 months to discuss a variety of research projects which are taking place in Sheffield and the wider region. As key stages of the research develop the advisory group chair will disseminate relevant information in relation to the study progress, and as time passes, the research findings.



2. Respect and accessibility

How have you addressed respect and accessibility in this project?

This work was completed ensuring that the needs of people affected by Multiple Sclerosis were considered at every stage. The provision of information and time given for feedback was scheduled in accordance with a co-agreed timeline and outlined both verbally and in the written communication which followed. The research advisory group actively advertise for membership via online platforms; all opportunities for involvement are in clear and simple language on these platforms and other promotional material. Involvement was sought both from people with Multiple Sclerosis and those affected in other ways (family members, carers, partners, friends) through the mixed research advisory group. Using email as a way of conducting some of the follow on activity enabled more accessible and timely follow-up. Travel budget and refreshments for the research advisory group members was provided by the NIHR Sheffield BRC.

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

The chair of the research advisory group ensures that the location, timing and format of meetings and email exchanges are acceptable to all stakeholders.

3. Representativeness of stakeholders



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

In order to develop a feasible project design, a diverse representation of patients was desirable; the inclusion criteria of the study is fairly wide therefore the varied mix of people in the research group was ideal. It was important to have feedback from a cross section of patients/carers, those who are in work/retired along with various lifestyle differences. The research group has a good mixture in terms of these, along with male:female ratio, years since diagnosis and experience of research participation from both patients with Multiple Sclerosis and respective family members. The active membership of the SMSRAG is typically ~10 people who regularly attend. A full group of 14 attended the meeting for this project.

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

Some patients from the research group volunteered their activity levels and age during the discussion. The mix of representation was very varied, and the group are well accustomed to considering the feasibility aspects from the perspective of others in their disease area.



4. Roles and responsibilities

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

The shared purpose and clear goals for the project were outlined at the start of the session ensuring each stakeholder was aware of their responsibilities. The research advisory group chair was defined as the go-to person in organising the project and the point of contact. The chair also followed up with all stakeholders and provided feedback from the study team.

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

The role of the research advisory group members and the responsibilities of the researchers and members are outlined in the terms of reference, which is circulated upon joining the group. The types of questions which would be posed by the researchers were communicated to the group along with the study document drafts ahead of the arranged face-to-face meeting. The deadline for returning written feedback was agreed upon at the meeting.

At what frequency have you checked this in?

This was confirmed in the face to face meeting and at subsequent follow-up emails.



5. Capacity and capability for engagement

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

The research advisory group members had prior experience of reviewing and commenting on research proposals, study protocols and patient facing documents. Members can also access free training courses through the Sheffield Clinical Research and Innovation Office should they wish. Group members were given access to study documents prior to the face-to-face meeting, thus facilitating more informed discussion. Prior to the documents being sent out, they were overlooked by the group chair to ensure clarity for lay readers. The format of the session was identical to all previous sessions, ensuring all were members expectations were met, and were prepared to take part. Time was included to spent explaining the research and answering questions to ensure a full understanding of the subject. This was followed by a demonstration of the exoskeleton was greatly received by the group and really helped the understanding of the subject. A further Q&A session followed.

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

This was confirmed in the face-to-face meeting via discussion and follow-on email contact.

6. Transparency in communication and documentation



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

The research advisory group received all documentation in relation to the study 2 weeks before the face-to-face meeting; this included a drafted summary of the study protocol. The slides used in the investigators presentation were circulated to the group via email following the face-to-face meeting along with a copy of the 9 question feedback form which was handed out in person. Minutes taken at the meeting were circulated soon after, which included various questions and ideas highlighted by the advisory group. The study team then took this information into account when rewriting the study protocol and projects plans.

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

Timescales were agreed in advance with the research advisory group members, and again at the face-to-face meeting to confirm that these were still reasonable and workable for them. The research advisory group members have shown prompt and positive email responsiveness; a great indication that the communication is well implemented.

7. Continuity and sustainability



What did you do to achieve this criterion?

The NIHR Sheffield BRC supply a well organised patient research support network; the research advisory group is sustained by them and continued involvement is both encouraged and supported by their PPIE strategy. Face-to-face meetings are followed up by the chair of the advisory group, and updates sent sporadically should they arise; the latest update being that the study team are completing the documents to send for ethical review, having taken the groups suggestions on board. In line with key developments in the course of the research (accepted by ethics, study opening to recruitment, study findings) a plan is in place to update the group via both email and at the corresponding face-to-face meetings.

How did you gather feedback on what you have done?

Feedback is gathered via email follow-up, as well as in person at the quarterly face-to-face meetings. Updates are sent to the group as the study team inform us of any aspect of the project progress which may be of interest to the group; e.g where suggestions haven been implemented and how the project was shaped as a result of the research advisory groups involvement.

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

During the initial setup of the research advisory group, it was agreed unanimously that continued involvement was of major interest.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

A number of useful comments were put forward on the usage of the exoskeleton as an aid to exercise for those patients with MS. All comments were captured in minutes by the administrator who is also member of the research advisory group. Follow up questionnaires were also completed after the meeting and returned via email. Members of the research advisory group identified patient's fear of falling in the exoskeleton as an unaddressed priority; to provide reassurance, changes in relation to these concerns were incorporated into the study protocol as a result of the patient engagement.

Positive impact for specific medicines development phases

Operational impact - The study team hope to experience a smoother process through ethical approval as a result of incorporating patient suggestions into study design - Once the trial is open to recruitment, it is hoped that the patient input into the study and product design will lead to maximum recruitment and retention of participants.

Direct or indirect positive impact for patients

Empowerment for patients/public who are involved - Increased awareness of relevant clinical programmes - Patient voice embedded in decision making.

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

The project led to better understanding of the patient perspective in the acceptability of study procedures and expectations.

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?

Yes

No

Lessons learned

The Principal Investigator and study team found the discussion at the face-to-face meeting hugely beneficial. It opened their eyes to a number of aspects that were not previously highlighted. Having an experienced administrative assistant experienced to minute the meeting was a great positive; this ensured any questions recorded and all comments captured whilst enabling the study team to be fully engaged with the advisory group at all times. The hands on demonstration of the exoskeleton was greatly appreciated, and really helped the advisory group grasp how the project will work and what sort of issues would possibly be encountered when using the equipment. The comprehension of the actual product to be used as well as the study design was key; the importance of building relationships with patients to facilitate frank and open discussion was strongly reinforced here.

About NIHR Sheffield Biomedical Research

The National Institute for Health Research (NIHR) Sheffield Biomedical Research Centre (BRC) is a research partnership between the University of Sheffield and Sheffield Teaching Hospitals NHS Foundation Trust, dedicated to improving the treatment and care of people living with chronic neurological disorders. We bring together the Faculties of Science, Medicine and Engineering for the common theme of Translational Neuroscience; our three main areas of research are Neurodegenerative disease, Neuroinflammation and Cerebrovascular disease.

About the team

The Sheffield Multiple Sclerosis Research Advisory Group (SMSRAG) was set up in 2018 to enhance patient and public involvement in MS research. The group brings together those affected by multiple sclerosis to use the unique knowledge of living with, or affected by the disease, to join the fight against it; helping shape future research. The SMSRAG have a shared interest in enhancing patient care, improving understanding of the disease, and helping to develop new treatments and ways to manage symptoms.



Annex 1: Questions from reviewers

In this section you can see what additional questions the reviewers had regarding this patient engagement initiative, as well as the answers provided by the submitting organisations. The section intends to provide more information and insight into this patient engagement initiative.

General

Q1. What were the thoughts of the MS Patient Advisory Group on the idea of using an exoskeleton for exercise?

It was clarified that the purpose of the study was not to use the exoskeleton as part of daily life but as an aid to exercise in controlled conditions. The advisory group were positive about the idea of using an exoskeleton for exercise. Patients expressed their existing limitations when exercising, along with their ideas of requirements for exercise aids. The fear of falling was the main concern voiced, however, the group were reassured once the study team informed them of the safety features already built into the exoskeleton. The study team understood and identified with their apprehensions, adding further safety measures to the protocol as a result.

Q2. How did patient needs and preferences factor into developing the shared purpose? What processes were used to ensure the purpose was truly shared?

The advisory group were given information about the project, and the overarching shared purpose was agreed by the members and researchers via email ahead of the face-to-face meeting. The specifics of the activity were then recapped at the meeting itself. Throughout the meeting, care was taken to ensure that the group felt that they could speak freely on both positive and negative aspects of their current exercise experience and what form of intervention they felt may prove most beneficial. They fed back that they felt their input had a very meaningful and powerful impact since they could see how their feedback had been incorporated into the final study design.

Q3. Were patients compensated for their involvement?

Patients are not compensated for their involvement, however, travel budget and refreshments are provided for the research advisory group by the NIHR Sheffield BRC.

Q4. When did the advisory group meet? Did it suit the schedules of patients involved?

The advisory group meet quarterly. The usual meeting time, place and frequency were mutually agreed at the implementation of the advisory group after discussion. At the end of each meeting the next date is scheduled in person, and followed up by email, giving people time to ensure they can attend; alternative dates can always be found if a number of members cannot attend.

The meeting in this instance took place on 16th April 2019. Dr Nair followed this up with a presentation at another meeting in February 2020, in which he gave the advisory group an update on the study progress.

Q5. Did patients have the opportunity to provide input into the terms of reference?

During the set up of SMSRAG, the members input into and agreed the terms of reference. Feedback was provided on the draft ToR before it was mutually agreed upon and finalised. It was important that it outlined the responsibilities of both the members and researchers, as well as the overall purpose of the group itself. These terms of reference are circulated to new members upon joining the group, and we plan to review these at yearly intervals to ensure they are still relevant.



KPI framework to measure progress on the Novartis Commitment to Patients and Caregivers

Organization: Novartis

The PFMD Book of Good Practices

3rd edition | 2020





KPI framework to measure progress on the Novartis Commitment to Patients and Caregivers

Organization:
Novartis



Basic Information

Project Description

In 2017 Novartis refreshed the 2015 Patient Declaration. 40+ patient organizations (POs), representing > 200 mio patients provided insights and expectations for a new “Declaration”. Measurement, transparency and reporting progress were critical for the community. In 2018 the Commitment to Patients and Caregivers was launched. To respond to Patient Organization’s (PO) feedback, a KPI framework measuring progress was developed. Throughout the KPI development process, POs were involved to guide on prioritizing the KPIs. Three KPIs important to the patient community and measurable were reported on Novartis.com in 2019 together with 17 data points on patient engagement, access and transparency. The Patient Organizations contributing to the Commitment design received a personal update from the CEO of Novartis. In Feb, 2020 the report expanded to 6 KPIs and 16 data points. Work continues in line with IMI PARADIGM.

Start Date:

01/05/2017

End Date:

Ongoing

Geographic focus

- Global
- Continental/Regional
 - Europe
 - Asia
 - Oceania
 - National
 - North America
 - Africa
 - South America
 - Antarctica

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

- Research
- Education
- Organization/
System development
- Access
- Advocacy
- Policy
- Care Delivery
- Gap analysis

Which phase(s) in the patient care journey does the initiative align with?

- Prevention
- Onset of symptoms
- Newly Diagnosed
- Treatment
- Clinical Trial
- Long Term Management
- Other: Metrics for measurement of organizational progress in Patient Engagement (PE) along the entire life cycle of medicine

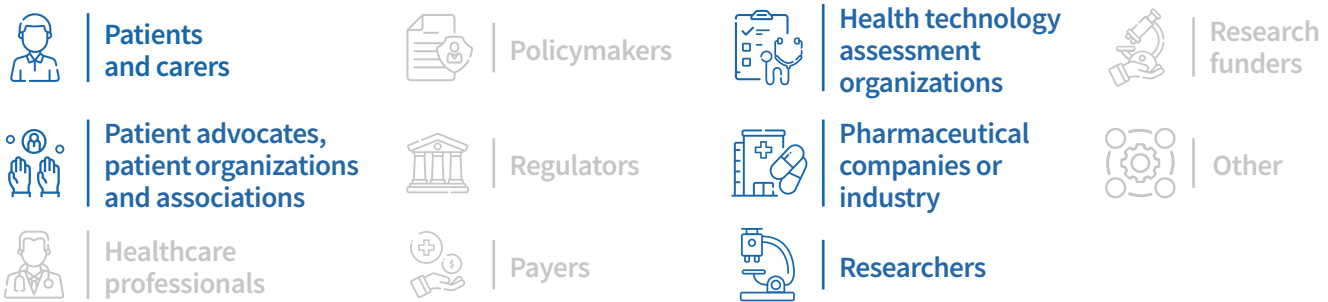
Which phases of research, medicines development, lifecycle or disease management does your PE project cover?



Other:

Patient Engagement along the lifecycle

Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

The list of potential KPIs was developed based on the input of POs. The draft was shared and fine-tuned with POs at multiple stages. The final framework as well as the results of the framework are being shared in a continuous feedback process and work shared with IMI PARADIGM.

What is your stated “shared purpose”?

Develop a meaningful KPI framework for PE, relevant for the patient community and reportable for the organization to drive patient engagement organizationally and demonstrate the value of PE.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

The final list of KPIs and results of the KPIs have been shared with participants and the public. During the review meetings and in routine engagements with the patient community, real time feedback was given to the community on feasibility and systems of measurement.

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

At each of the feedback meetings and in routine meetings, the intent was presented with Q&A before the actual input. At publication of the output direct feedback from participants was collected.

At what time points?

Formal feedback was collected during the KPI development process, at publication of KPI results in year 1 and 2 and continuously when results are presented to the community in meetings worldwide.

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

Broad access to this project was given through a multichannel approach across geographies with a mix of meetings, voting, online meetings and a continuous feedback loop.

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

Acknowledgement of a respectful process is documented in meeting reports where input to KPIs was given and assumed proven by the feedback received via mail to the personal address to the CEO of Novartis and absence of negative feedback on the published KPIs.

3. Representativeness of stakeholders



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

The meetings where KPIs were developed and feedback given range across all therapeutic areas and geographies of the company. The published factsheet is accessible for anyone anywhere world wide.

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

The contributing 40+ POs had global, regional and local reach and represent 200 mio patients world wide across multiple disease areas and geographies, the resulting report is visible globally and continuously discussed and open for feedback globally.

4. Roles and responsibilities



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

All meetings where the KPI framework is discussed have co-created agendas, with defined roles and feedback loops on agenda, clarity in the meetings and reports documenting outcome.

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

NA see above.

At what frequency have you checked this in?

Routinely checked as part of our interactions with the patient community.

5. Capacity and capability for engagement



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

Documentation on the planned metrics, the data sources, available measurement systems, complexity of measurement were shared upfront as pre-reads and introduced in the meetings in the format of spreadsheets and explanatory slides on the process and progress.

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

Patient advocacy leaders who are the relationship managers checked this and we leveraged the IMI PARADIGM Work Package on metrics for input and feedback.

6. Transparency in communication and documentation



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

For input to the KPI framework development (as stated) timely communication was part of the input process of routine meetings. In the output process (the communication on the KPIs) the publication date was shared upfront and contributing POs received a personal copy of the factsheet from the CEO.

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

Google analytics and Instagram tracking, 34,000 .com hits in launch month, 2000 IG views with constant audience on all posts, at any time an analytics report on the KPI factsheet views can be conducted, internal tracking is conducted by a count of the views and likes of related Yammer posts.

7. Continuity and sustainability



What did you do to achieve this criterion?

Sustainability is inherent in the Commitment to Patient and Caregivers with the commitment to report progress. On an annual basis (now 2 consecutive years) a KPI factsheet is published on novartis.com until the next progress report is given at the Anniversary of the Commitment – this is an established annual process. In addition, select KPIs are regularly reported to the Board of the company.

How did you gather feedback on what you have done?

Statistics on the views of the KPI report, input from meetings where the feedback on the build out of the KPIs framework as well as the results are minuted.

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

See above, based on input from building of the Commitment for the need of progress report, input onto individual KPIs and feedback on KPI results, feedback from PARADIGM KPI workgroup and desire to integrate the work into the metrics work package.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

The impact is best measured by comparing results on KPI framework and KPIs themselves:

On the framework – expanded from NO PE reporting in 2018 to 1st progress report in 2019 with 3 KPIs and 17 fact points to 2nd progress report in 2020 with 6 KPIs and 16 fact points.

On the KPIs themselves: simplified summaries of clinical trials to participants from 28 trials to 50 trials (4652 patients to 8.016 patients); Access to Managed access from 5789 to 10 503 patients; Patient reported outcomes in integrated in 55 of 92 trials starting in 2019, reach with European Patient Innovation summit from 270 patient advocates to 400; 10+ manuscripts on insights obtained published in both reporting years.

Positive impact for specific medicines development phases

The fact sheet ranges across the lifecycle, access and transparency:

<https://www.novartis.com/sites/www.novartis.com/files/commitment-anniversary-factsheet.pdf>

Direct or indirect positive impact for patients

The KPI factsheet drives organizational change and awareness, which leads to increased Patient engagement, access and transparency which impact patients with better engagement thus better addressing patient needs and better access.

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

All of the above plus expanding access to medicines and the importance of transparency and reporting.

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?

Yes

No

Lessons learned

Building a KPI framework for an organization is complex for the following reasons:

- Achieving alignment on priority KPIs in the Patient Community ranging across a wide range of disease areas and geographies requires a rigorous process
- Internal alignment on priority KPIs requires a wide range of internal stakeholders and approval steps for both the KPI framework as well as the annual report of the data
- Measurement of the impact of patient engagement, particularly in development, is complex and requires several years to obtain results - Need to initially measure activity rather than impact, given that data on activity is easier and faster available
- Data capture can be a challenge – proposed KPIs are not always captured in existing systems and manual capture is a significant effort and may lead to inaccuracies
- Positive impact derived specifically from Patient engagement in a clinical trial may be confounded with other factors due to the variability across trial designs and disease areas, as well as improvements in operating approach of global drug development
- Data sourcing and capture across multiple involve departments along the life cycle with a variety of systems require significant effort and commitment from involved departments
- Measuring KPIs and reporting increases awareness and attention to patient engagement and drives willingness to report and “do more”. The statement “what gets measured gets done” proves right for patient engagement and the mindset shift towards more patient focus and patient engagement
- An organizational KPI framework on Patient engagement is not only a tool to report progress on the engagement, it is also a facilitator of change management by enhancing organizational awareness
- The process to collate the KPI must be documented and auditable

About Novartis

Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 109,000 people of more than 140 nationalities work at Novartis around the world.

Find out more at <https://www.novartis.com>.

About the team



Michaela Dinboeck

Global Head Patient Engagement CoE

Michaela leads the Novartis Center of Excellence for Patient Engagement (PE) and is responsible for building the conditions to enable systematic and consistent PE across all disease areas and along the medicine lifecycle, including common standards and KPIs, capabilities and sustainable insights from the patient community.

Team includes:



Laura McKeaveney

Global Head Patient Advocacy



Geoff Cook

Executive Director, Patient Engagement Hematology



Nigel Cook

Head, Decision support & Insights, Pharma Patient Access



David Parrish

Head of Patient Engagement and Advocacy, Novartis Institute for BioMedical Research NIBR



Veronica Foote

Executive Director, Patient Engagement Solid Tumors



Amber Spierer

Executive Director, US Patient Engagement



Cathryne Clary

Global Head, Patient Affairs and Policy (left Novartis 2018)



Jill Gregson

Head, Corporate Responsibility Reporting, Corporate Reporting and ESG Office

in building the initial KPI framework, many more contributors for obtaining feedback from the Patient Community and reporting the KPI data.

Social Media Information:

Novartis is on Twitter. Sign up to follow @Novartis at <https://twitter.com/novartisnews>

For Novartis multimedia content, please visit <https://www.novartis.com/news/media-library>

For questions about the site or required registration, please contact media.relations@novartis.com



Clinical Trials - Something for You? Information about participating in Clinical Trials

Organization: Novartis

The PFMD Book of Good Practices

3rd edition | 2020





Clinical Trials - Something for You?

Information about participating in Clinical Trials

Organization:
Novartis



Basic Information

Project Description

Patient organizations communicated need for easy-to-understand information about clinical trials, what it entails to participate in a clinical trial, and where to find information on on-going trials.

Project Objectives

To improve patients' knowledge and access to clinical trials in Norway by:

- making it easier for patients to find information on clinical trials
- supporting informed choices so that patients and investigators will be able to discuss and conclude together, ie shared decision making
- delivering on Novartis' commitment to patients and caregivers.

Methodology

A patient leaflet on clinical trials in Norwegian, English and Urdu: Clinical trials – Something for You? Available also as e-leaflets in Norwegian and English. Link to the English version of the Norwegian e-leaflet: https://issuu.com/inbusiness/docs/pasienthefte_om_kliniske_studier_eng_mai_2019_fina

The leaflet is a result of a cooperation between Novartis, 10 PAGs and the Pharma Trade Association in Norway (LMI).

Start Date:

17/10/2017

End Date:

December 2019

Geographic focus

- Global
- Continental/Regional
 - Europe
 - North America
 - South America
 - Asia
 - Africa
 - Antarctica
 - Oceania
- National: Norway

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

- Research
- Access
- Care Delivery
- Education
- Advocacy
- Gap analysis
- Organization/
System development
- Policy

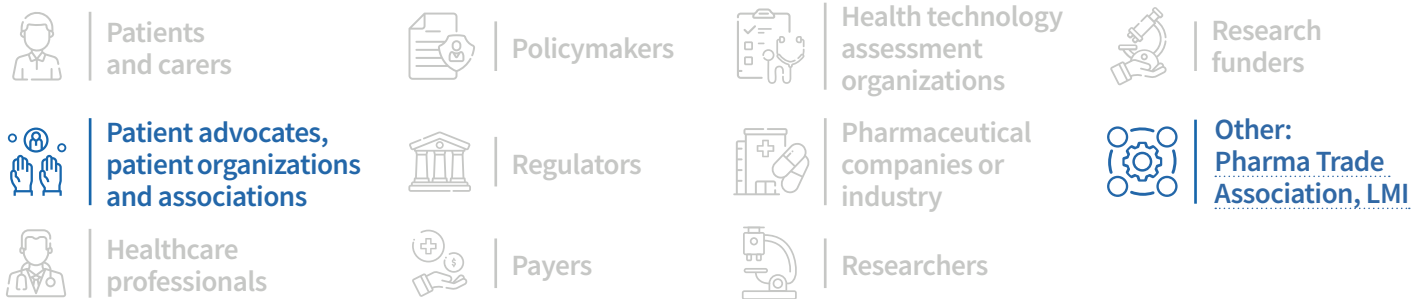
Which phase(s) in the patient care journey does the initiative align with?

- Prevention
- Newly Diagnosed
- Clinical Trial
- Onset of symptoms
- Treatment
- Long Term Management
- Other

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



Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

Consulting patient organizations PAGs to evaluate the format and content, define what was missing and what could be changed.

What is your stated “shared purpose”?

Providing easy-to-understand information about clinical trials for patients, what it entails to participate in a clinical trial, and where to find information on on-going trials.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

During regular meetings with the patient organizations, by email asking for feedback and discussions on phone.

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

Yes as described above as well regulated in cooperation agreement.

At what time points?

At the two annual events Novartis is organizing with patient organizations, ie April and October 2018 and 2019.

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

By listening and delivering on defined needs regarding content, wording, languages, format and font, etc. By publishing the leaflet with all logos.

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

Feedback from patient organizations has been implemented. Examples: Choosing bigger format and bigger letters in respect for those with impaired sight. Making an e-leaflet – easy to use. Patient organizations as well as the Pharma Trade Organization, LMI, have shared the leaflet with members and used it in adequate contexts.

3. Representativeness of stakeholders



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

We engaged patient organization representatives broadly across therapeutic areas as well as the local Pharma Trade Association.

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

The stakeholders represented multiple therapeutic areas to ensure representative views, independent of the disease states.

4. Roles and responsibilities



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

Roles and responsibilities were clearly defined in the cooperation agreement as well as discussed at regular meetings, communication by email and phone.

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

As above.

At what frequency have you checked this in?

At meetings with patient organizations in April and October 2018, experience sharing at meeting April and October 2019.

By regular e-mails and phone calls. There was also a follow communication on webpages and via social media etc.

5. Capacity and capability for engagement



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

Providing regular educational meetings focusing on clinical trials by Novartis Medical Affairs colleagues and relevant external stakeholders. Providing the content for review based on defined needs from patient organizations.

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

By involving, listening and delivering on insights. Work Package on metrics for input and feedback.

6. Transparency in communication and documentation



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

Ongoing communication and reviews throughout the process.

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

The Novartis cross-functional team met regularly and had ongoing exchange with the patient organizations and the Pharma Trade Association. The involved organizations logos are published on the patient leaflet.

7. Continuity and sustainability



What did you do to achieve this criterion?

Listening to needs defined by patient organizations for easy to read and reliable information about clinical trials, asking them about preference for format and content to secure relevance.

How did you gather feedback on what you have done?

By involving patient organizations from the start to the end, listening to needs defined, discussing content, presenting draft documents, asking for feedback to ensure capturing insights and need along the way, adapting materials and re-sending for review and finalization.

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

By continuous dialogue through regular meetings, e-mail communication and phone. Continuity and sustainability has been shown by the leaflet being actively used at educational events, political meetings, at a Nordic Pharma Trade event etc. The leaflet has also been showcased in patient magazines, webpages and in social media. Some clinics have asked for leaflets for their outpatient clinics.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

Outcome: Delivering on need defined by PAGs: Easy to understand patient leaflet on clinical trials - **Clinical trials – Something for you?** Available in Norwegian, English and URDU. Also available as e-leaflets in English and Norwegian. Impact: Actively used by PAGs, Pharma Trade Association and Novartis.

Positive impact for specific medicines development phases

Contributing to the understanding and participation in clinical trials and conducting clinical trials responsibly among all involved stakeholders from a long-term perspective.

Direct or indirect positive impact for patients

Increased knowledge about clinical trials contributes to a better dialogue between patients and doctors and thereby support shared decision-making, and makes it easier to participate in a clinical trial.

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

Improved knowledge among patients supports clinical trial execution and potentially faster access to new treatments.

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?

Yes

No

Lessons learned

- Listening, respecting and understanding the patient need is key for supporting the patient voice.
- Partnering with patient organizations early and establishing common ground for fruitful collaboration.
- Identifying insights to collaborate for mutual benefit. For dissemination, a shorter executive summary could be valuable to reach an even wider audience.

About Novartis

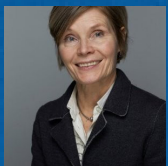
Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 109,000 people of more than 140 nationalities work at Novartis around the world.

Find out more at <https://www.novartis.com>.

Clinical Trials - Something for You? Information about participating in Clinical Trials:

The patient leaflet Clinical trials – something for you? is the result of a project with input from 11 patient organizations across several disease areas and endorsed by The Pharmaceutical Industry Association of Norway.

About the team



Åsta Gjersvik

Project coordinator, has worked in Novartis Norway in several roles and is presently Patient Relations Head.



Øyvind Fensgård

PhD in molecular medicine, joined Novartis Norway in 2013 as Clinical Research Medical Advisor.



Caroline Skar Mjønes

Patient Safety Manager

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PATIENT FOCUSED
MEDICINES DEVELOPMENT



NOVARTIS



Global Oncology Patient Insight Panel (GOPIP)

Organization: Novartis

The PFMD Book of Good Practices

3rd edition | 2020





Global Oncology Patient Insight Panel (GOPIP)

Organization:
Novartis



Basic Information

Project Description

Evidence-based guidance on how to best facilitate patient engagement in the drug development process is lacking. Patient advocates, with deep knowledge of the special needs of patients within their respective disease areas and experience in patient centered research and programming, were identified by the patient relations team of the organization. Participating advocates were generally contracted for consulting support across the development lifecycle and agreed to provide feedback within a 3-4 week timeframe to requests for support. Called Global Oncology Patient Insight Panel (GOPIP), the approach enables fast-moving development teams to quickly engage advocates for advice while enabling projects to remain on timeline. GOPIP members engaged with teams within the organization on an ad-hoc basis across the drug development process, including review of protocols, PRO selection, and informed consent, and plain-language-based summaries, among many other patient-centered projects.

Start Date:

31/05/2018

End Date:

Ongoing

Geographic focus

Global

Continental/Regional

Europe

Asia

Oceania

National:

North America

Africa

South America

Antarctica

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

Research

Education

Organization/
System development

Access

Advocacy

Policy

Care Delivery

Gap analysis

Which phase(s) in the patient care journey does the initiative align with?

Prevention

Onset of symptoms

Other

Newly Diagnosed

Treatment

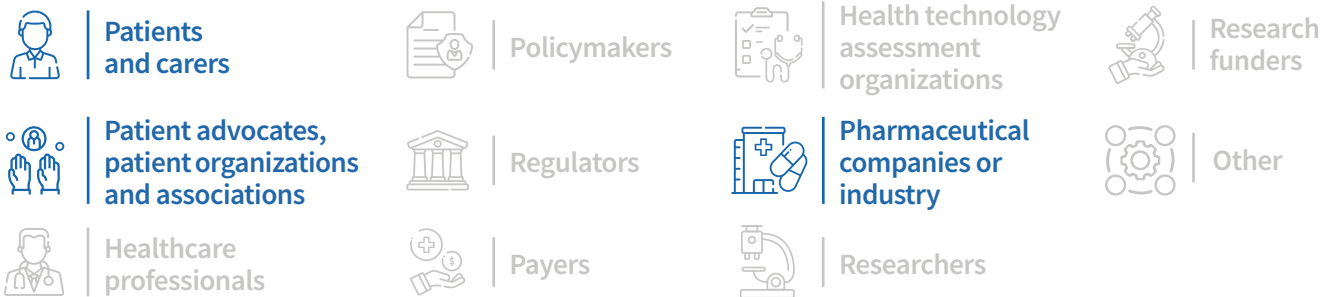
Clinical Trial

Long Term Management

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



Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

We met with each stakeholder to present and discuss the aims of the project.

What is your stated “shared purpose”?

The purpose is to gather valuable patient insights on all stages of the research and development processes.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

Yes, the purpose has been confirmed with each GOPIP consultant and with internal team stakeholders.

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

Yes, the shared purpose and its understanding among stakeholders was reviewed.

At what time points?

The shared purpose and its understanding is reviewed with the GOPIPs at the inception of engagement, at the beginning of each project, and annually upon contract renewal. Shared purpose is reviewed at the start of each project with internal teams.

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

The Global Patient Relations team worked with members of the team from diverse regions and countries to identify knowledgeable advocates with whom to engage. Communications and contracting were discussed personally with each patient/advocate involved. A mutually agreed upon time frame, usually two weeks, is accounted for in each project. Participants are compensated using guidelines of local fair market value.

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

Initial meetings to explain and acknowledge the tenets of the program, including mutual respect and guidelines of engagement, is established before contracting and in regular touchpoints with the GOPIPs. Each project is moderated by a Patient Relations team member for consistency in respect and engagement. At the completion of each project, the stakeholders are asked to complete a feedback form to evaluate their experiences.

3. Representativeness of stakeholders



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

The patient/advocates are identified through a broad network of Patient Relations leads around the world. The global team ensures that GOPIP members include a broad mix of representatives from disease areas, gender, age, ethnicity, social circumstances. Since most project work is completed offline, the GOPIP works according to their own time requirements.

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

We monitor outcomes of the engagement through reporting measures of each project (e.g. using a spreadsheet). Patient Relations and the Project Owner maintain regular communications through the process to ensure stated objectives are being met. At the end of each project, the project owners are surveyed to check about their satisfaction with the engagement.

4. Roles and responsibilities



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

The roles and responsibilities are communicated at the start of each GOPIP contracting period, as well as with the advocates at the start of each project. The contract sets forth the expectations of the engagement. Internal stakeholders are also met with at the start of each project and for regular check points to communicate roles and responsibilities.

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

We check understanding while meeting with participants (as described in previous question).

At what frequency have you checked this in?

Frequency of checks depend on the project. Some are short term, or finite in nature. A longer term and complex project includes at least one check every 2 weeks and as needed.

5. Capacity and capability for engagement



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

GOPIPs are chosen according to their level of expertise, such as in patients in a particular disease area or

knowledge of the clinical trial process. The process includes using virtual and electronic methods to support capacity and confidentiality.

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

The Patient Relations lead checks in and communicates with stakeholders at the start of, and throughout the project to ensure that they have what they need to contribute effectively and meaningfully. Surveys at end of project evaluate these perspectives as well.

6. Transparency in communication and documentation



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

Materials and documentation are provided at project start with stated expectations at each project start. GOPIPs have direct and regular communication with the Patient Relations lead during the project. At least two weeks are agreed upon for project feedback and timing for feedback is mutually agreed upon.

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

Validation is completed throughout the project process and at the end of project via evaluation. Patient Relations is responsible for setting up regular calls, updates, and communications.

7. Continuity and sustainability



What did you do to achieve this criterion?

The GOPIP agreements are designed for continuity as the terms of agreement are for one year. At the end of the year, a review of the projects and outcomes, as well as future strategy, are considered regarding contract renewal, additional advisors, or scaling back of a panel.

How did you gather feedback on what you have done?

Feedback is gathered through satisfaction surveys of the GOPIP advisor and of the project owner after each project. Quarterly and annual review of project status and outcomes provide regular evaluation of value.

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

The planning to secure continuity and sustainability were discussed with stakeholders at the start of contracting with GOPIPs and at project start with Project Owners.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

GOPIP engagements included 49 advocates from 19 countries, across 14 disease areas, in the program's first 18 months. 54 projects were completed. Multiple engagements on a clinical development program level for three clinical trials have been accomplished. Seven plain language summaries of published data results were reported publicly with consultant inputs.

Positive impact for specific medicines development phases

Increased understanding of unmet medical needs, patient-driven solutions, and improved study design.

Direct or indirect positive impact for patients

Increased involvement and impact on all stages of development, improved patient-directed materials for clinical trials, better and more accurate patient-reported outcomes instruments.

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

Patient insights and involvement in decision-making process, as well as development of patient materials, initiatives, and related initiatives.

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?


Yes


No

Lessons learned

This project was proven to be successful both for internal teams and consultants. Satisfaction scores were high among advocates and internal teams. Once the GOPIPs were established, the speed and efficiency aspects were highly valued among development teams. We aim to incorporate these insights and method at an earlier stage in development, as well as to effectively evaluate outcomes.



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About Novartis

Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. Novartis products reach nearly 800 million people globally and we are finding innovative ways to expand access to our latest treatments. About 109,000 people of more than 140 nationalities work at Novartis around the world.

Find out more at <https://www.novartis.com>.

About the team

The Novartis Global Oncology patient advocacy team, **Dawn Aubel**, **Geoff Cook**, **Susan Hayes** and **Alexey Salamakha** bring a diverse set of experiences and backgrounds to their daily work to improve outcomes for patients. With backgrounds in healthcare delivery, patient advocacy, communications and bioethics as well as decades combined experience in the pharma industry, the team focuses on cultivating mutually beneficial working relationships with advocacy coalitions and patient leaders. Through these relationships, the team seeks to identify critical patient insights that help improve all aspects of the Novartis mission to reimagine medicine for people living with cancer.

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Consumer Involvement in Research Program

Organization: Cancer Council NSW

The PFMD Book of Good Practices

3rd edition | 2020





Consumer Involvement in Research Program

Organization:
Cancer Council NSW



Basic Information

Project Description

Cancer Council NSW values the views of the community we serve and represent. Cancer Council NSW is one of the largest non-government funders of cancer research in Australia and we are committed to involving consumers – cancer patients, survivors, carers, family and friends of someone diagnosed with cancer, and members of the general public – in all research activities funded or conducted by the organisation. We expect all researchers who apply for our competitive research funding to involve consumers in their research, and all funding applications we receive are reviewed by a panel of trained consumers.

Cancer Council NSW is committed to the Consumer Involvement in Research program in the following ways:

1. Facilitating and delivering training courses: (i) the annual Consumers in Research and (ii) the biennial Consumer Review Panel training
2. Ensuring all grants administered by Cancer Council NSW have undergone review by consumers
3. Requiring all recipients of Cancer Council NSW research funding, and all internal research activities, to involve consumers.

Cancer Council NSW, in collaboration with advocacy organisation Cancer Voices, delivers training courses for consumers so there is a pool of trained consumers available for researchers to involve in their work. Each year, a half-day Consumers in Research workshop is run to equip consumers with the appropriate tools to work with researchers. A small set of online modules must be completed before attending the workshop.

The Consumers in Research training (online and face-to-face workshop) covers cancer biology, cancer incidence and mortality in Australia, cancer research fundamentals, grant and ethics applications, and how to work as a consumer advisor to researchers. Participants in the course also hear from researchers and consumers about their experiences working together, including benefits and challenges and how to overcome any barriers.

The core role of graduates of the Consumers in Research course is to work with a researcher or research team as a consumer advisor to provide the perspective of a 'lived experience.' Consumer advisors are able to work with researchers from any cancer research field, including discovery research, drug development research, clinical trials, and public health.

Cancer Voices is a volunteer organisation that has the purpose of representing and acting on the needs of people affected by cancer. Members of Cancer Voices founded the Consumer Involvement in Research program, which has been in operation since 2005. Pivotal to the Consumer Involvement in Research program, Cancer Voices maintains a database of available consumer representatives, which graduates of our Consumers in Research training course are invited to join. The database is used by Cancer Voices to match researchers' requests for consumer representation with appropriate consumers. This is a free, online service available to researchers nationally. Consumers may be requested to join a research team as an advisor, panel or committee member.

Cancer Council NSW offers a further training course for consumers. In alternate years, a half-day Consumer Review Panel training course is run to prepare consumers to participate on the Cancer Council NSW Consumer Review Panel to review the community-relevant portion of grant applications.

Research applications are assessed by the Consumer Review Panel against five criteria developed in consultation with consumers. For our annually-offered research Project Grants, Cancer Council NSW considers community relevance of equal importance as scientific merit, and as such, funding decisions are made based on a rank that is equally derived from consumer and scientific peer review.

Cancer Council NSW expects applicants of our competitive research funding to involve at least one consumer in the proposed research. As of 2019, this will be an eligibility requirement when applying for our funding. We are also committed to involving consumers in the work of researchers who are employed directly by Cancer Council NSW.

We believe consumer involvement in research improves research conduct and outcomes in the following ways:

- Increasing research relevance so researchers consider and plan for their research to have end-user applications and benefits
- Providing transparency to the community and researchers on how research is funded and conducted
- Adding the end-user's perspective to research to ensure research is conducted with consideration of end-users, for example, consumers may advise on the real-world feasibility of a treatment, or assist researchers recruit patients to a study using appropriate communication styles.
- Improving dissemination of research outcomes throughout communities to increase public knowledge and awareness of research
- Contributing to a positive feedback loop whereby there is increased confidence among the community in research, leading to increased support for research funding
- Informing research funding decisions to ensure applicants can demonstrate: (i) their research is of importance to the community, (ii) the probability and timeline for achieving outcomes that benefit end-users, and (iii) the potential for research findings to be equitable in the community.

Start Date:

2005

End Date:

Ongoing

Geographic focus

- Global
- Continental/Regional
 - Europe
 - North America
 - South America
 - Asia
 - Africa
 - Antarctica
 - Oceania
- National: Australia -NSW

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

- Research
- Access
- Care Delivery
- Education
- Advocacy
- Gap analysis
- Organization/
System development
- Policy

Which phase(s) in the patient care journey does the initiative align with?

- Prevention
- Newly Diagnosed
- Clinical Trial
- Onset of symptoms
- Treatment
- Long Term Management
- Other: Our initiative is focused on any or all phases of the patient care journey that aligns with the research journey, from planning and development through conduct of research, to dissemination of findings

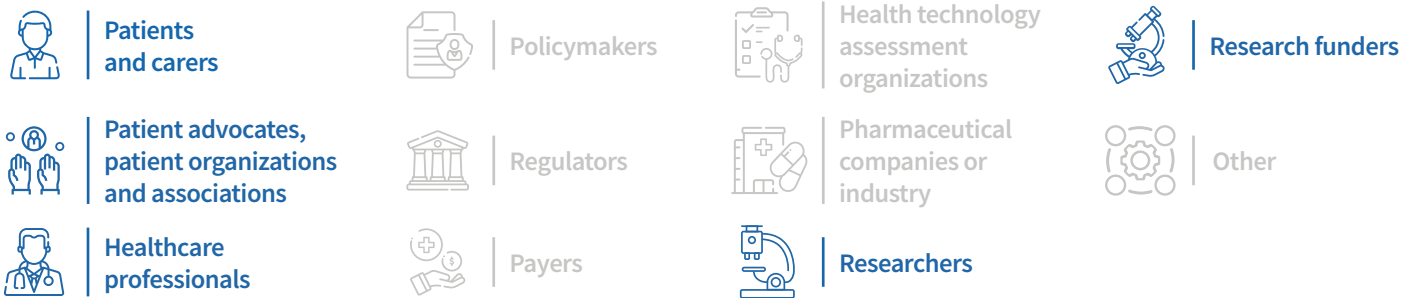
Which phases of research, medicines development, lifecycle or disease management does your PE project cover?



Other:

Our initiative is focused on any or all phases of the patient care journey that aligns with the research journey, from planning and development through conduct of research to dissemination of findings.

Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

The training courses in the Consumer Involvement in Research program have been in operation since 2005 and are at a mature stage; however, adjustments are made based on feedback each year to continue to optimise the courses. Consumers are actively involved in planning and delivering the training sessions to ensure the courses remain current and continue to meet the needs of attendees. This collaboration also ensures there is a shared understanding of the purpose of the program.

In planning the training sessions, consultations are undertaken with Cancer Voices' executive team and other experienced consumers. Several components of the training courses are presented by consumers who have been previous attendees of the training and have gained considerable expertise as a consumer in research or Consumer Review Panel member.

Researchers are also involved in the delivery of the Consumers in Research training course. Researchers who are funded by Cancer Council NSW and, therefore, already have consumer involvement in their work due to our funding eligibility requirements are invited to present sessions during the training.

The training courses we deliver are evaluated at the end of each training session via feedback surveys, and graduates of the Consumers in Research training are contacted approximately 6 months after completion to improve our understanding of the utility of the course and the activities they have gone on to be involved in as a consumer. This follow-up contact is a second opportunity for attendees to provide feedback for course improvement.

The wider research community receives information about consumer involvement in research in the following ways:

- Information sessions on the value of involving consumers in research for researchers are delivered by Cancer Council NSW
- Consumer Review Guidelines, which detail the process and mechanism of the consumer review of grant applications Cancer Council NSW receives, are available on our website and are written for applicants and consumer reviewers to ensure both stakeholders share a common understanding of the review expectations.

Researchers working in Cancer Council NSW's Research Division are also expected to involve consumers in their work and have access to an internal document, Statement on Community and Consumer Involvement in Research. This document sets out our vision that "consumers are actively and meaningfully engaged in all research activities managed by the Cancer Research Division, to ensure research is performed in partnership with the community and for the community."

What is your stated “shared purpose”?

Our research funding comes from donations from the general public. Consequently, we are committed to funding research that is both of significant scientific merit and of value to the community we serve and represent.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

No answer

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

No answer

At what time points?

No answer

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

In facilitating the Consumer Involvement in Research Program, the following aspects of communication and organisation foster respectful engagement and ensure accessibility:

- Organising the training for consumers with consumers ensures the training facilitator at Cancer Council NSW and training presenters are engaging with consumer attendees in an appropriate manner.
- Information on the training courses published on Cancer Council NSW’s website, in electronically distributed mail, and in other promotional material has been reviewed by a team of communicators and website user experience specialists such that the information is clearly presented, engaging, and relatable.
- To supplement the training information on Cancer Council NSW’s website, a video interview with a training graduate and three case studies of experienced consumers are available. These resources are relatable to prospective attendees of the training and reinforce the program’s value.
- Prior to and during the courses, we emphasise to attendees at the training that scientific, research, or a professional background is not required to be an effective consumer in research.
- Training materials are offered in several formats: a series of free online modules, face-to-face workshops that incorporate seminar-style and discussion-based sessions, and a free training manual is provided to all attendees. The training manual includes all information (and more) covered during the online and face-to-face components of the course.
- The training courses are free to attend. Cancer Council NSW provides travel support for NSW-based attendees travelling from outside metropolitan Sydney to ensure people living in regional areas are able to participate in our program.
- The training courses are held at a central location in metropolitan Sydney within short walking distance from public transport.

- The training courses are half-day courses, usually held on a Thursday and Friday so that part of the travel for regional consumers can be on a weekend.
- The training facilitator and collaborator from Cancer Voices NSW individually approach Aboriginal and Torres Strait Islander networks to engage Indigenous consumers.
- Cancer Council NSW runs cultural awareness training for all staff members so that we are able to create culturally appropriate and safe environments for Aboriginal and Torres Strait Islander community members.

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

No answer

3. Representativeness of stakeholders



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

We recognise the majority of training attendees are referred to our course by people they know, so we contact people in a position to recommend community members attend the training. In promoting the training, we reach out to our network of funded researchers, previous attendees of the training, other cancer and consumer organisations, funding bodies, administrative officers at research institutes, and like-minded consumer groups such as consumer advocates. Recently we have promoted our training on social media to diversify the age range with younger consumers and consumers without a cancer experience.

To be engaged with a consumer, researchers are able to submit a request to Cancer Voices detailing their consumer requirements. We reflect on previous researchers' requests and target our promotion to population groups that are in demand (e.g., consumers with a rare cancer experience).

The membership of Cancer Council NSW's Consumer Review Panel changes annually. In selecting trained consumers to participate on Cancer Council NSW's Consumer Review Panel to assess funding applications for community relevance, we aim for gender balance, and diversity in geographical location, age and cancer experience among members. This can sometimes be challenging, as the majority of trained Consumer Review Panel members are female, >50 years old, and from metropolitan Sydney. This is by no means a reflection of selective recruitment to the program, but likely to be a reflection of the demographic interested in participating in the program. To counteract this bias, trained male consumer panelists are invited to participate on the Panel more frequently than females for the objective of gender balance on a panel.

Consumer Review Panel members are offered travel reimbursements to attend a face-to-face panel meeting and are provided with review materials in electronic and hard copy formats. We train a surplus of consumers to ensure a certain quota of reviewers are available to participate on a panel.

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

No answer

4. Roles and responsibilities



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

The role of each stakeholder in facilitating the training courses is long-established and has remained unchanged since program implementation in 2005.

During the Consumers in Research training, the role of consumer advisors in relation to a researcher is clearly defined in both the online and face-to-face components of the course, as well as in the handbook that accompanies the training.

The roles and responsibilities of members of the Consumer Review Panel are outlined in a Terms of Reference document (updated annually) and described during the training and orientation sessions for Panel members.

The roles of researchers in the program are outlined in application guidelines, during ad hoc information sessions delivered by Cancer Council NSW and, for Cancer Council NSW's internal research team, outlined in the Statement on Community and Consumer Involvement in Research.

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

No answer

At what frequency have you checked this in?

No answer

5. Capacity and capability for engagement



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

Together with a cross-functional governance team comprised of patient engagement, R&D, patient centred The training courses, and subsequent consumer-researcher matching service, were designed and are delivered with the intent to empower and mobilise skilled consumers who are equipped with the tools required to commence a role as a consumer advisor to a researcher.

The training courses were co-developed by the consumer organisation, Cancer Voices, and an academic with prior experience in consumer training. The criteria used by the Consumer Review Panel to assess the community relevance of grant applications were developed as part of a PhD project, in collaboration with Cancer Voices, and after consultation with community members. Working alongside consumer groups ensures we are providing relevant and accessible information to consumer stakeholders.

Researchers have access to information about consumer involvement in research on Cancer Council NSW's website, including links to other helpful resources. Cancer Council NSW also delivers free information sessions to researchers on consumer involvement in research, usually by invitation.

All stakeholders are welcome (and encouraged) to contact the Consumer Involvement in Research coordinator

at Cancer Council NSW with any enquiries or feedback regarding their consumer in research activities.

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

No answer

6. Transparency in communication and documentation



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

According to the Standard Operating Procedure developed by Cancer Council NSW for facilitating the training courses, the following communication is made:

- Simple, clear information is available on Cancer Council NSW's website about the training and who it is suited to, including a video interview with a previous participant and a step-by-step guide for how to become a consumer advisor or Consumer Review Panel member.
- Following online registration for the face-to-face courses, registrants are sent an auto-generated email confirming receipt of their expression of interest and further details about the courses.
- Registrants are contacted approximately 6 weeks prior to the training to confirm their interest in attending the course and provide details for the training day(s).
- Registrants are provided with an Agenda for the training day(s) in the week prior to the training.
- In 2019 Cancer Council NSW will work toward solidifying and enriching our engagement with consumers by delivering biannual newsletters to previous participants of the courses to keep them updated on consumer-related activities, events of interest to consumers, and Cancer Council NSW research highlights.

All information presented during the training is provided in hardcopy and includes any slides shown for attendees to take notes.

The online Consumers in Research course is freely available for anyone to access.

For the Consumer Review Panel, communication is made in the following ways:

- Consumer Review Guidelines are provided to each panel member (and all applicants) that clearly describe the process of the review and how to assess each criterion of the application. These materials are made available in hard and soft copy formats for panel members.
- Each application is provided to reviewers in hard and soft copy formats.
- An orientation teleconference is held prior to the meeting to reacquaint panel members with the process, their roles and responsibilities, and provide an opportunity for panel members to ask questions.
- At the end of the Consumer Review Panel meeting (that is held face-to-face for efficiency and efficacy) panel members are invited to provide feedback on the process.
- The Consumer Review Panel process is run with tight time constraints (due to external timelines), so email is the most common mode of communication during this time

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

No answer

7. Continuity and sustainability



What did you do to achieve this criterion?

The following strategies have been employed for sustainability of the program at Cancer Council NSW:

- Standard Operating Procedures are in place to maintain consistency for the training courses we deliver and Consumer Review Panel grant review process.
- All material for the training courses is produced for longevity, such that the same materials can be re-used with minor updates for cost and time efficiency, as well as continuity in standards.
- While our training material is the intellectual property of Cancer Council NSW, we welcome observers to our courses so that other organisations may model their in-house training on our established program.
- Each training session is evaluated and lessons learned incorporated into the Standard Operating Procedure and future courses.

How did you gather feedback on what you have done?

No answer

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

No answer

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

The Consumer Involvement in Research program was evaluated by Cancer Voices 10 years after the program was implemented. In their evaluation, Cancer Voices separately surveyed consumers and researchers who have participated in the program. Key results from the survey included:

- 95% of researchers regarded the contribution made by consumers to their research project as highly valuable
- 60% of researchers noted that consumers played a significant role in shaping research direction and helped researchers gain a deeper understanding of the consumer perspective
- 80% of researchers agreed that consumers provided insight into issues that were important to the community
- 80% of consumers felt they were able to offer advice to ensure the research project would benefit consumers.
- 88% of consumers felt that the Consumers in Research training course prepared them for their role in research.

Positive impact for specific medicines development phases

No answer

Direct or indirect positive impact for patients

No answer

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

No answer

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?

Yes

No

Lessons learned

We have learned the following:

- Consumers find the most valuable part of the Consumers in Research training is hearing from researchers and experienced consumers
- Consumers find the most valuable part of the Consumer Review Panel training is practicing reviewing applications
- Researchers are increasingly receptive to consumer involvement in research, particularly following initial engagement with a consumer
- Consumer involvement is most effective when the consumer is engaged early in a project
- Consumers and researchers often do not speak the same “language”, and a middle ground must be found for communication.

We face the following challenges, which we are working to overcome:

- Recruiting diverse consumers to the program (age, gender, ethnicity, cancer experience), in particular members from the Aboriginal and Torres Strait Islander communities. We continue our efforts to form relationships with members of the Indigenous Australian community.
- Ensuring consumers are engaged by a researcher throughout the duration of a Cancer Council NSW grant. We have reinforced continued consumer engagement in the Annual Progress Reports researchers are required to complete.
- Responding to consumers’ requests for refresher training or networking events. Cancer Council NSW is a charity with limited funds for functions and events. We have so far facilitated one refresher/networking event for consumers.
- Delivering the training course in another area of Sydney. While we provide travel support for consumers

travelling from regional areas in the state, and our current training location is accessible by public transport, some consumers are limited in their capacity to travel due to poor health or requirements of caring for someone affected by cancer.

The following aspects of the Consumer Involvement in Research program continue to be successful:

- Delivery of the Consumers in Research course in blended mode: the online modules were implemented in 2017 and complement the face-to-face workshop.
- Presentations by researchers and experienced consumers at the Consumers in Research workshop
- Long morning tea and lunch breaks during the training sessions to allow consumers to network
- Giving consumers an opportunity to practice their skills in mock grant review panels during the Consumer Review Panel training
- Providing information sessions to researchers on the value of involving consumers in research
- Maintaining a strong relationship with Cancer Voices.



 @CCNSWResearch

 Cancer Council NSW

About Cancer Council NSW

We are Australia's leading cancer charity, working across every area of every cancer. Every day, we support families affected by cancer when they need it most, speak out on behalf of the community on cancer issues, empower people to reduce their cancer risk, and find new ways to better detect and treat cancer.

Our purpose: uniting the community, providing support, investing in research and saving lives.

We want to reduce the impact of cancer by:

- Reducing cancer risk
- Increasing cancer survival
- Improving the quality of life for people affected by cancer
- Improving cancer outcomes for priority populations

Research is one of the Cancer Council NSW's most important and significant investments, and we are the largest non-government funder of cancer research in Australia.

Cancer Council NSW funds research of the highest scientific merit and of value to the community that it serves and represents.

About the team

The **Research Grants Management Team** provides high-quality administrative support to the research we fund and the research we conduct. The team supports key governance committees, facilitates the assessment and funding of research applications and the development of competitive grant proposals. Our communications specialist works with Cancer Council's fundraising and media teams to showcase the impact of our researchers.

Annex 1: Questions from reviewers

In this section you can see what additional questions the reviewers had regarding this patient engagement initiative, as well as the answers provided by the submitting organisations. The section intends to provide more information and insight into this patient engagement initiative.

General

Q1. Is the involvement that is required for funding sustained through the project and how do you check that the researchers did what they said they were going to do in terms of involvement?

Our funded researchers are expected to involve consumers throughout the research cycle, from project inception and conducting the research, through to communicating research outcomes. Researchers who apply for our funding must complete a Consumer Review Form and outline how trained and/or experienced consumers have been involved during the development of the research proposal, also describing plans for ongoing consumer involvement over the course of the research. We require annual reports from our funded researchers throughout the duration of their project, where they must provide evidence of how they have been involving consumers.

Q2. Did you involve consumers in the development/oversight of your programme?

Cancer Council NSW's consumer training courses were first delivered in 2005 and were developed in collaboration with our consumer stakeholders at Cancer Voices NSW. Development of the courses arose from the pioneering work of Cancer Voices' Consumer Involvement in Research program.

Adjustments are made to the program based on consumer feedback each year to continue to optimise the courses. Consumers are actively involved in planning and delivering the training sessions to ensure the courses remain current and continue to meet the needs of attendees. The training courses we deliver are evaluated at the end of each training session via feedback surveys, and graduates of the Consumers in Research training are contacted approximately 6 months after completion to improve our understanding of the utility of the course and the activities they have gone on to be involved in as a consumer. This follow-up contact is a second opportunity for attendees to provide feedback for course improvement.

Q3. Is the compensation provided to consumers involved in the program?

We do not provide compensation, however, we do cover out of pocket expenses for consumers attending the training. The training courses are free to attend and Cancer Council NSW provides travel support for NSW-based attendees travelling from outside metropolitan Sydney to ensure people living in regional areas can participate in the program. The training courses are also held at a central location in metropolitan Sydney within short walking distance from public transport.



Q4. Are people able to contribute to this if they are unable to leave the house? Are your training offer and involvement opportunities also online?

We offer an online Consumers in Research course, which can be freely accessed by anyone at any time. These modules cover a broad range of consumer content and are designed to inform and prepare community members to be involved in health and medical research. The Consumers in Research workshop is delivered in an interactive format, which provides an opportunity for participants to interact and connect with other consumers, consumer stakeholders and researchers. We are currently looking into delivering the workshops virtually this year, and in utilising the web conferencing technologies available, we hope to maintain the same level of interactivity with our consumers whilst attending the training remotely and accessing the same training resources online.

In relation to consumer involvement in research activities post training, researchers and consumers can decide upon the mode of interaction which works best for them, and may conduct the involvement virtually if the consumer is unable to meet in person (e.g. via emails/zoom).



Roadmap for a future-proof healthcare system

Organization: Pfizer

The PFMD Book of Good Practices

3rd edition | 2020





Roadmap for a future-proof healthcare system

Organization:
Pfizer



Basic Information

Project Description

Pfizer and 11 external patient experts developed a “roadmap for a future-proof healthcare system” with 10 theses and actions for a sustainable health care system – a basis for public and political discussion. The experts are members of the Pfizer-Patient-Dialog, a national platform for PAG and HCPs to discuss healthcare policy, transferring patient voice into political discussion. No theoretical ideas but practical action to improve HC system in Germany and promote international cooperation on health issues. This may be of special interest also in the Corona-crisis, even the roadmap was developed before the crisis.

Start Date:

20/08/2019

End Date:

(28/04/2020) With ongoing political debate

Geographic focus

- Global
- Continental/Regional
 - Europe
 - Asia
 - Oceania
 - North America
 - Africa
 - South America
 - Antarctica
- National: Germany

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

- Research
- Education
- Organization/
System development
- Access
- Advocacy
- Policy
- Care Delivery
- Gap analysis

Which phase(s) in the patient care journey does the initiative align with?

- Prevention
- Onset of symptoms
- Other: Health care management – micro level (doctor-patient-relationship/communication) and makro level (system approach)
- Newly Diagnosed
- Treatment
- Clinical Trial
- Long Term Management

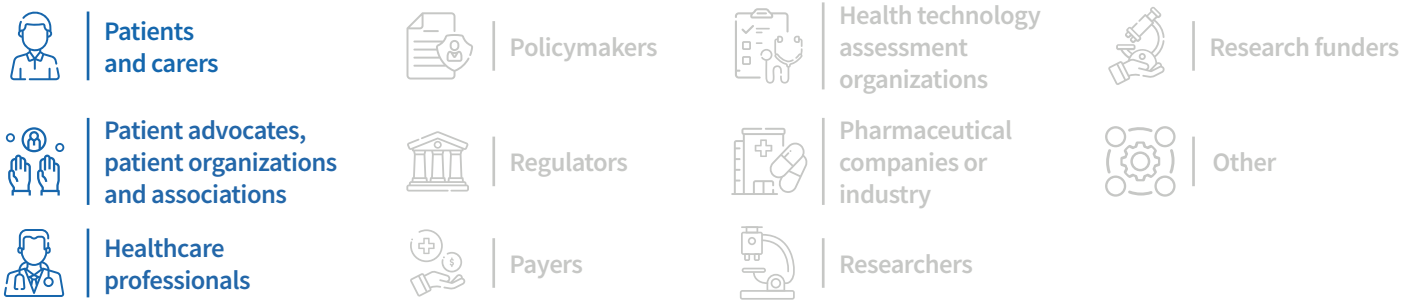
Which phases of research, medicines development, lifecycle or disease management does your PE project cover?



Other:

Holistic approach of Healthcare System

Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

The concept for the roadmap originated from patients, physicians and nurses attending a regularly occurring Pfizer-Patient-Dialogue in Germany. Discussions continued among a smaller group confirmed the appropriateness and progress at regularly updates.

What is your stated “shared purpose”?

Our purpose is to improve healthcare and HC policy by patient involvement/patient engagement considering their practical recommendations - on micro (individual) and macro (system) levels of healthcare.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

All stakeholders provided their input to create this „Roadmap for a future-proof healthcare system” in order to create a holistic view and patient-oriented solutions.

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

We have organized a process to review all roadmap elements with regard to the shared purpose. All stakeholders were asked to focus in their interviews to that purpose. We checked the shared purpose individually and finally with the entire group.

At what time points?

All the time: start, interview-phase and final alignment of the roadmap.

2. Respect and accessibility



How have you addressed respect and accessibility in this project?

The roadmap was shaped from the start with guidance from patients and in an iterative manner through its development. It is a co-creation project respecting the authors’ preferences. We received feedback that all expectations about patient engagement were verified.

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

Pfizer expressed its respect in statements in the Roadmap. In addition all other stakeholder contributing to the Roadmap were fully engaged via deep dive interviews and individually consultation. Our co-creation process not

only covers the concept- and development-phase, but also includes the political follow-up discussion. We have started together and keep working on together.

3. Representativeness of stakeholders



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

Patient advocates and healthcare professionals involved in the project reflect a broad range of therapeutic areas (e.g. Oncology, Rare Diseases, Pain etc.) as well as above-therapeutic-area perspectives (e.g. elderly, nurses, physicians). They interact on a regular basis with a diverse group of patients within Germany and strived to integrate those diverse experiences into the project.

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

Pfizer's dialogue with patients laid the foundation for trusting, constructive cooperation to improve provision for and with patients in Germany. Experts from various fields are involved and share their knowledge and experience. In their contributions to this roadmap, they explain what changes and improvements are necessary and possible in the health care system. These are not theoretical ideas but concrete recommendations. The roadmap content is holistic because the authors reflect a broad range of stakeholders.

4. Roles and responsibilities



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

Clear alignment between all stakeholders: Pfizer, Patients, Physicians and Nurses from start to end.

The point of contact for the patients involved was a skilled and dedicated Patient Relations Manager and team who has developed and nurtured relationships with patients and patient organizations involved.

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

Clear alignment in process and contracts.

At what frequency have you checked this in?

At the beginning, during development of roadmap and at the end via finalizing text – and transferring into ongoing political discussion.

5. Capacity and capability for engagement



What did you do to support building the required 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, physicians and nurses from the start as well as Pfizer colleagues to ensure the alignment of capabilities with the goals of the project.

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

We formally aligned all relevant aspects in contracts and worked together with support of a journalist/moderator.

6. Transparency in communication and documentation



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

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

1. Concept: Co-Development of Concept, Topics and Content together with Patient Organisations
2. Interviews: Patients' Theses to improve healthcare system
3. Review I: Check by patients and incorporation of their feedback
4. Review II: Check by medical and legal

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

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

7. Continuity and sustainability



What did you do to achieve this criterion?

We worked on the roadmap to start a national and international discussion about future health care system – improving with patient experts. We are currently working on further political discussions based on the Roadmap.

How did you gather feedback on what you have done?

All stakeholders involved provided input to co-create the roadmap – and jointly we are pushing for a political follow up. We are approaching media and politicians to gather further feedback and follow up programs.



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

The roadmap is the starting point for political change.

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

We continue the project at the Pfizer-Patient-Dialog in September 2020. In the meantime, we are currently approaching media and politicians to ensure ongoing political debate including patients' voice. We have scheduled virtual discussions starting end of May.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

The roadmap is providing recommendations and practical actions by patient experts to improve healthcare system in Germany and to promote international collaboration on health issues.

Positive impact for specific medicines development phases

We jointly push for a political discussion to address many issues to improve healthcare system via patient-driven solutions (e.g. digitalization, cross-boarder collaboration, doctor-patient-communication etc.).

Direct or indirect positive impact for patients

Increased influence in shaping the political discussion with a patient-focus (before and after CORONA crisis) – e.g. with impact on digitalization, coordination of services, health literacy, medical appointments, patient participation and doctor-patient-communication in order to improve quality of life for patients, easier access to treatment options and increased influence of patients in healthcare decision making.

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

Better understanding of patient needs, patient focus and patient-led improvements in Healthcare system. We encourage patient engagement in cross-functional and international working groups to share best-practices of patient-centered healthcare systems.

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?

Yes

No

Lessons learned

We have learned that patients, physicians and nurses provide best-practices in improving healthcare system. This may be of special interest also in the Corona-crisis, even the roadmap was developed before the crisis. We are starting a debate in Germany for more patient-focus in healthcare system with a joint approach of all stakeholders. If we keep the momentum of focusing what really matters (in CORONA-crisis), we can change the „business as usual-style” in a real „patient-focused healthcare policy” which will be of benefit for us and our children.


Key Learnings:

- **A “co-creation” 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.
- **Partnership along the journey:** The roadmap reflects the position of patients, physicians and nurses – however, Pfizer enabled these advocates to raise their voice.
- **Keep patient focus:** Our Roadmap reflects that patient needs may differ from public needs. Risk groups have specific needs which should be considered in political debates and we can observe that in Corona-Crisis - Patient Engagement is even more important in political discussion than before.



 @Pfizer

 Pfizer

 Christine Claussen

About Pfizer

Pfizer Inc.: Breakthroughs that change patients' lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 170 years, we have worked to make a difference for all who rely on us.

About the team



Christina Claussen

Christina Claussen is Director Patient Relations & Alliance Management for Pfizer Germany. She holds a master in politics/economics/administration sciences (University of Konstanz) and worked for a German sick fund and the Federal German Pharmacists Association, before she joined Pfizer in 2002. She is leading the “Pfizer Patient Dialog” and the Patient Relations Group initiating projects and alliances in co-creation with patient organizations across all businesses and functions of the company.

Project team

Anja Schmidt - Manager Patient Relations

Ulrike Voigtländer - Manager Patient Relations

Dr. Oliver Burgard - Journalist



Leukaemia Patient Preference Study

Organization: Evidera, Takeda,
Leukemia & Lymphoma Society

The PFMD Book of Good Practices

3rd edition | 2020





Leukaemia Patient Preference Study

Organization:
Evidera, Takeda, Leukemia & Lymphoma Society



Basic Information

Project Description

Background: Philadelphia Chromosome Positive (Ph+) Acute Lymphoblastic Leukemia (ALL), a rare subtype of ALL, is a cancer of the blood and bone marrow and is more common in adults than children. Ph+ ALL progresses rapidly without treatment and traditional chemotherapy alone is not effective. Historically the prognosis for people with Ph+ALL was poor, due a lack of effective treatment. In the 2000s, a new type of targeted treatment was developed, tyrosine kinase inhibitors (TKI), improving the prognosis for people with Ph+ ALL. Since then several TKIs have been approved. TKI therapies in combination with chemotherapy have improved Ph+ALL patient outcomes, with more patients reaching remission and overall survival increasing. The various TKIs differ in terms of their efficacy and risks, and patients may have different preferences when making treatment decisions. Little is known about patients' preferences when choosing TKIs for treatment of Ph+ ALL.

Objectives: The aim of this study was to understand what matters to patients with Ph+ ALL, in the United States, when choosing a first-line treatment. What is important to patients might vary and this can affect their preferences for different treatments. Specifically, the goal of the study was fourfold: to identify the attributes of treatment that matter to patients; to quantify patients' preferences for these attributes; to use the preference data to generate insights into patients' choices; and to understand why patients have these preferences.

These data can be used to support patients getting access to the best treatments for them, through several routes. This includes a broader patient perspective than typically considered in regulatory approval and

reimbursement decisions; ensuring that physicians are aware of patient preferences when prescribing treatments; and empowering patients take an active role in their treatment decision.

The Leukemia & Lymphoma Society is a Patient Advocacy Organization for people with a range of blood cancers, including Ph+ALL. One of the key objectives of the collaboration with The Leukemia & Lymphoma Society was to ensure that research involving patients and patient engagement is brought back to patients in meaningful ways.

Method: This study used a mixed-methods approach, combining qualitative and quantitative research, including:

- A survey-based method to elicit patient preferences using a Discrete Choice Experiment (DCE). This method involves patients choosing between a series of hypothetical pairs of treatment. The analysis of patients' choices allows insight into how patients trade-off the attributes of treatments. Before the survey was fielded, cognitive pre-testing interviews were undertaken to ensure patients understood the survey and that the attributes and levels were relevant to patients.
- Qualitative interviews. The qualitative research involved interviews with patients to understand their experience of Ph+ALL, their treatment expectations and concerns, and the reasons for their treatment preferences.

Stakeholders:

The following stakeholders were involved in the study:

1. Takeda (Pharmaceutical company)- Co-Investigator and study sponsor
2. Evidera (Scientific Health Research Consultancy)- Co-Investigator
3. The Leukemia & Lymphoma Society (Patient Advocacy Organization)- Collaborator
4. Oncologists (Healthcare professionals)- Advisor
5. Patient Advocate- Advisor
6. Patients in pre-testing interviews of survey- Reviewers

The Leukemia & Lymphoma Society has been involved throughout the lifecycle of this study, reviewing all study deliverables, providing input on the survey design, recruiting for the project, supporting interpretation of the findings, and co-authoring study outputs.

Designing a DCE in oncology can be complex and input from stakeholders such as patients, patient advocacy organizations and physicians can greatly improve the relevance and understandability of the survey to patients and the value of the results. This case study will predominantly focus on the collaboration with the patient advocacy organization, The Leukemia & Lymphoma Society.

Start Date:

13/11/2018

End Date:

Ongoing

Geographic focus

- Global
- Continental/Regional
 - Europe
 - North America
 - South America
 - Asia
 - Africa
 - Antarctica
 - Oceania
- National: United States

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

- Research
- Access
- Care Delivery
- Education
- Advocacy
- Gap analysis
- Organization/
System development
- Policy

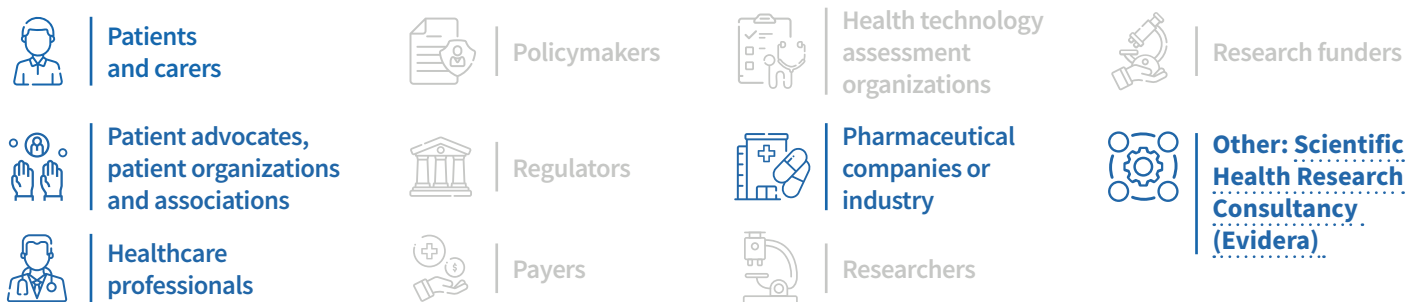
Which phase(s) in the patient care journey does the initiative align with?

- Prevention
- Newly Diagnosed
- Clinical Trial
- Other
- Onset of symptoms
- Treatment
- Long Term Management

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



Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



What did you do to achieve this criterion?

A collaborators workshop was organized at the initiation of the study, attended by Evidera, four representatives of The Leukemia & Lymphoma Society and Takeda. The workshop focused on the objective and scope of the research, including: the target population, the data that was to be collected from the patients, and the outputs that were to be generated from the data. The workshop also addressed the design of the study and the roles of collaborators. The workshop helped ensure alignment on the research objective, methods, output and roles.

Following the workshop, a scope of work – description of the study and collaborator roles – was drafted and shared for review. Comments from collaborators were incorporated into a final version of the scope of work which was included in a Collaboration Agreement ('Agreement'). Evidera's legal team worked with The Leukemia & Lymphoma Society during the contractual process.

What is your stated “shared purpose”?

Our primary purpose was to understand what matters to patients when choosing a first-line treatment for Ph+ ALL, and how this varies between treatments.

Further, it was our shared purpose that:

1. Patients should be a key stakeholder in the conduct of the study to address this goal, ensuring that it was designed and conducted in a manner that reflected patients' needs
2. The study be impactful, supporting the development and availability of treatments that align with patients' needs and preferences.

How have you confirmed with all stakeholders that your purpose is understood, that contributions have influenced the original plans and that disagreements have been addressed?

The Agreement was signed once all parties were comfortable with the content.

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

It has not been necessary to review the shared purpose, as there has been strong ongoing commitment and clarity among all team members. The original scope of work is available for review, but the scope of work and shared purpose has remained the same. Throughout this project we have had regular calls to ensure the project is progressing as anticipated. This includes a review of study materials and interim deliverables with all collaborating stakeholders.

At what time points?

NA



2. Respect and accessibility

How have you addressed respect and accessibility in this project?

The stakeholders representing the patient voice were engaged in different ways to reflect their respective expertise in the research:

- Patient Advocacy Organization:
 - Accessibility of project materials: The Leukemia & Lymphoma Society had some previous experience with the methods being employed on this study. This expectation was confirmed prior to collaborating on the study; Evidera also provided further information on the study methodology in the initial workshop. It was thus possible to share standard research documents – preliminary design slide deck, protocol, statistical analysis plan – with The Leukemia & Lymphoma Society for input.
 - Stakeholder expectations: Roles and responsibilities were clearly defined in the Agreement (see Section 1), which was developed collaboratively. Meetings with The Leukemia & Lymphoma Society were arranged in advance and at times that worked for The Leukemia & Lymphoma Society.
 - Budget and payment considerations: Remuneration for program management, technology system costs and patient outreach were pre-defined in the Agreement signed by Evidera and The Leukemia & Lymphoma Society, along with an agreed payment schedule.
- Patient advisor:
 - Accessibility of project materials: The patient advisor was referred by The Leukemia & Lymphoma Society, was not a technical expert and was not familiar with the research method being employed. However, the patient representative was advising on patient facing materials, so no modifications were needed and there was no need to enhance accessibility. Any questions the patient had were clarified by the project manager during the interview.
 - Stakeholder expectations: The patient representative was a constituent of The Leukemia & Lymphoma Society. The advisor was informed how much of their time would be required before participating and the interview was arranged at a time chosen by the advisor.
- Cognitive pre-testing patients:
 - Accessibility of project materials: These participants were asked to engage with materials designed to be patient facing, so no modifications for understandability, from a non-technical perspective, were required.
 - Stakeholder expectations: A consent form that was approved by Takeda and Ethical & Independent Review Services (E&I) was signed by participants. This outlined the purpose of the study, what procedures would be involved, what data would be collected and who to contact with questions.
 - Budget and payment considerations: Remuneration for time participating was aligned with fair market values and was defined and agreed to in the consent form.

As the Evidera team members are based in the UK and are English speakers, there was no need for major cultural adaptations when working with The Leukemia & Lymphoma Society, who are based in the US, but also English speakers. However, flexibility was required to organize meetings across varied time zones.

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

The very thorough Agreement signed by The Leukemia & Lymphoma Society was an acknowledgment of their level of commitment to and involvement in the study. In this study, The Leukemia & Lymphoma Society representatives and the patient advocate all had access to computers and were flexible to adapt to the communication systems which allowed optimal involvement.

The Evidera project manager represented Evidera as the contact person for The Leukemia & Lymphoma Society representatives and for the patient advocate. Additionally, in all correspondence with The Leukemia & Lymphoma Society, Evidera would welcome any questions about the study and their participation.

3. Representativeness of stakeholders



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

Ensuring meaningful patient input into the study faced two challenges:

- The complex nature of the research method meant it was difficult to get meaningful patient input on the study design.
- The small number of patients with Ph+ALL meant it was difficult to achieve fully representative samples of sufficient size within the study timelines.

Patient Advocacy Organization: The Leukemia & Lymphoma Society' role in the study was central to addressing these two challenges. First, relatively uniquely among Patient Advocacy Organizations, The Leukemia & Lymphoma Society had experience with and some expertise in the method being employed in the study. Second, their knowledge of all blood cancers, including the Ph+ALL patient population, meant that they were able to represent the patient voice. Third, their constituency facilitated reach to study participants.

The Leukemia & Lymphoma Society collaborators represented different areas of expertise and levels of access to patients. For example, one of the staff members is the senior vice president of patient access and outcomes, making her input very valuable in understanding the potential burden on patients.

Additionally, The Leukemia & Lymphoma Society will be involved in ensuring that this research will be shared with patients in a meaningful and patient friendly manner.

Clinical and Medical leads at Takeda: The clinical and medical leads at Takeda reviewed study materials for this project. These individuals are aware of current treatment gaps and have insight into the unmet need from a physician and patient perspective.

Practicing oncologists: While it is to be expected that oncologists might not have a complete understanding of their patients' preferences, their experience of working with many patients (they were treating an average of 1-2 Ph+ ALL patients a month) meant that they were able to represent a diversity of patient experiences in a population that is hard to reach.

Patient Advisors: The patient advocate and the 5 patients who advised on the survey design were not familiar

with the study methodology. To ensure their input was valuable they were given discrete tasks and were asked to provide feedback on patient facing materials, which was used to integrated into the DCE online survey. It was not necessary to offer training on the study methodology as the patient facing materials were designed to require no knowledge of the methodology. The patient facing materials included an introduction to the study, a description of treatment features and an explanation of what the survey would involve. The patient advisors were a mix of genders, age and had varied disease history.

Patient participants: Since Ph+ ALL is extremely rare no formal quotas were enforced during patient recruitment. However, in order to target patients from diverse backgrounds a variety of recruitment routes were utilized: 101 patients were recruited from The Leukemia & Lymphoma Society' constituency; and 100 patients were recruited by a specialist recruitment organization. The recruitment specialist utilized a variety of recruitment methods: referrals from healthcare practitioners across diverse areas of the United States (n=67), online patient panels (n=30) and social media (n=3). Since this is an online survey the sample may not be representative of those without computer access.

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

Sociodemographic and clinical information was collected on study participants to allow an assessment of the representativeness of the final sample. Despite no formal quotas the sample had a mix of males and females and patients from different ethnic and racial backgrounds.

4. Roles and responsibilities



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

Roles and responsibilities of study collaborators were agreed upon and summarized in the Collaboration Agreement (see Section 1 for more detail).

The patient advisor from The Leukemia & Lymphoma Society who reviewed the study design ahead of protocol finalization was introduced to the study by The Leukemia & Lymphoma Society initially. The Evidera project manager contacted the patient advisor ahead of their interview to share materials for the interview and to provide further information on the study and what their role would be. The advisor was able to ask questions at this point as well as during their interview. The patients who provided feedback in the cognitive pre-testing interviews were classed as study participants and were provided with an information sheet containing information about the study and their role, ahead of providing online consent.

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

Roles and responsibilities were agreed upon and summarized in the Agreement (see response to Section 1 for more detail). The Evidera project manager was available to answer questions from The Leukemia & Lymphoma Society throughout the study, as needed. However, no adjustments were needed to the roles and responsibilities outlined in the agreement.

Patient advisors were provided with information on their role ahead of their participation. Evidera verbally confirmed that the patients understood their role ahead of participation and answered any questions from patients advising on the survey design. Patients understood that there were no right or wrong answers and that their input was to improve the understandability of materials from the patient perspective.

At what frequency have you checked this in?

Collaborators roles and responsibilities were agreed upon at the start of the study and written into the Agreement. It was not necessary to review these during the study, as all collaborators fulfilled their roles as expected. Regular check-ins with collaborators took place at each stage of the project, as agreed upon at the start of the project.

Patients advisors provided input on discrete tasks were undertaken over short and pre-defined timescales. No review of roles and responsibilities was necessary.

5. Capacity and capability for engagement



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

Most collaborators, from The Leukemia & Lymphoma Society, were familiar with the methods being employed in the study. The collaborator workshop held at the start of the study was used to communicate the study design and scope, agree upon roles and ensure collaborators had the capacity and capability to undertake these roles. Beyond that workshop, no meaningful capability/ capacity building was required to ensure collaborator engagement. More minor clarifications or support were facilitated by the project manager, for instance through facilitating access to relevance expertise at Evidera.

Evidera have both Patient Preference and Patient Engagement working groups, who provide training and internal workshops to ensure staff have the capacity to deliver on the type of study being undertaken on this project. This ensures quality by providing access to more specialist expertise, template materials and Standard Operating Procedures (SOPs).

Patient advisors were engaged in discrete and time-limited tasks, meaning it would be inappropriate to engage in extensive capacity building as knowledge of the study methodology was not required for the tasks that they input on. Their capacity to engage was ensured by (1) tailoring their role to their capabilities, such as advising on the patient-friendliness of the study designs and materials, and (2) tailoring materials shared with the advisors to ensure they were understandable.

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

See responses in Section 2 where we expand on accessibility to study materials. All patient advisors' role and materials were designed to align with their capabilities. Additionally, the collaborator workshop held at the start of the study was used to communicate the study design and scope, agree roles, ensure collaborators had the capacity and capability to undertake these roles.

All representatives had access to computers, email and internet in order to actively participate in meetings and email discussion. Continuous support from the Project Manager was available as required.

6. Transparency in communication and documentation



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

Overall, the Collaboration Agreement provided transparency on how collaborators would provide input into the study, with specific reference to the points during the study at which their input was required and through what mechanism. This primarily involved: reviewing key study deliverables; attending collaborator meetings at strategically important parts of the study; and ad hoc email and telephone meetings at other times as required. The Evidera project manager gave collaborators advance warning of when key deliverables and meetings were approaching, agreeing a specific timeline for collaborator input.

The Evidera project manager was responsible for sharing information with collaborators and keeping them updated on the project including components not requiring their direct input, such as informing them when IRB approval was gained.

Collaborator inputs were recorded in either meeting minutes, which were reviewed and approved by participants, or in written comments on study documents. Written responses to comments on study documents were shared with collaborators so that it was transparent how their input had been used.

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

N/A

7. Continuity and sustainability



What did you do to achieve this criterion?

Project delivery: As agreed in the project initiation workshop and the Collaborator Agreement, the Evidera project manager was responsible for ensuring the smooth input of The Leukemia & Lymphoma Society into the study (see response to section 1) and ensuring collaborators were updated on key study developments, such as achievement of IRB approval.

Ongoing relationships: The Takeda Patient Advocacy team leads patient organization engagement, serving as a single point of contact, to establish trusted and credible relationships based on shared priorities. Takeda Patient Advocacy leads patient organization engagement to champion the patient voice and experience through Takeda research, value demonstration efforts, and post-approval programs and services.

Takeda supports The Leukemia & Lymphoma Society's patient support, education, and policy efforts.

Continuous learning: Evidera has both Patient Preference and Patient Engagement working groups, responsible for promoting good practice in these components of our studies. Learnings from our studies are shared with these working groups, who in turn disseminate relevant best practices to the organization.

Dissemination of study results: Patient-friendly dissemination will be generated that will allow the Patient Advocacy Organization to inform their patient community about their investment in research and a summary

of results that could be of their interest. This could range from a brief announcement on their social media up to a short report written in plain language and with illustrations that are relevant for patients. For any of these strategies a last Patient Engagement task would be defined where Patient Advocacy Organization constituents, Patient Advisors and Patient Advocates can inform the team on the design and script of these materials.

How did you gather feedback on what you have done?

Evidera's standard procedures include a process for collecting Patient Advocacy Organization feedback on the experience of collaborating on studies. This includes a survey that will inform us about collaborators' experience and will highlight points for improvement. The survey comprises of a brief set of closed-ended and open-ended questions that allow us to score the delivery of the Patient Engagement task and to receive qualitative feedback. Topics covered include: the experience of collaboration, the impact on the project they felt their involvement had, and feedback on how both could be improved.

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

Project delivery: The project initiation workshop and the Collaborator Agreement covered processes for communication between collaborators (see section 1).

Ongoing relationships: The Takeda Patient Advocacy team maintain regular weekly or monthly communication with their counterpart at The Leukemia & Lymphoma Society to ensure any issues are raised and addressed in a timely manner.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

Patient preference studies increase awareness of patients' needs and preferences to decision makers (sponsors, regulators or prescribers). They do so by quantifying the trade-offs patients are willing to make between treatment characteristics. This generates a precise picture of patient preferences and facilitates insight into why a patient would choose a treatment with certain attributes over another, which directly addresses decision makers' concerns. Incorporating these data into the phases of medicines development ensures that they are more aligned with patients' needs.

Collaborating with The Leukemia & Lymphoma Society and other stakeholders helped to generate meaningful and robust study results. First, this helped to identify the treatment attributes that mattered the most to patients, it improved the attributes' definitions and descriptions to capture patients' concerns. Second, this ensured that the survey was clear and understandable to patients, ensuring they could provide meaningful responses to the survey questions. Third, The Leukemia & Lymphoma Society input supported the interpretation of study results, ensuring clarity on the implications of the results for decision makers.

Direct or indirect positive impact for patients

The dissemination of the patient preference data will ultimately lead to the availability of first-line treatments that best meet Ph+ALL patients' needs. This occurs via several routes, including:

- Ensuring regulators, reimbursement agencies and clinicians are aware of patients' needs, their willingness to tolerate treatment risks and their preferences for treatments attributes.
- Allowing patients to understand each other's preferences, empowering them to share their own preferences with their physicians.

The collaboration with The Leukemia & Lymphoma Society will ensure that that the results of the study reach patients in a manner than they can access.

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

The Leukemia & Lymphoma Society gained additional knowledge into this valuable patient preference methodology, which can be transferable into future collaborations. This study has helped them to demonstrate to their patients that they are engaged in research that is meaningful to their quality of life and outcomes. Furthermore, this study has provided information that The Leukemia & Lymphoma Society can use to help guide the education and support they provide Ph+ALL patients.

From a clinical perspective the results of this study support clinicians' understanding of attributes most relevant for decision-making and should support customized care and shared decision making.

For Takeda the results from this study can identify patient-centered endpoints that can be used in clinical development of treatments for people with Ph+ALL. The results also generate complementary evidence on patients' preferences alongside clinical trials for regulatory needs. Additionally, this study supports Takeda's Advocacy Team who can use the study results to develop educational materials for patients and patient advocacy organizations on aspects of Ph+ALL and treatment, from the patient perspective.

Is the initiative generalisable or replicable in other phases?

Yes

No

Is the initiative generalisable or replicable across other therapeutic areas?

Yes

No

Lessons learned

Patient Engagement is not currently commonplace in preference research. Patient Engagement should be considered more regularly by those conducting patient preference studies to improve the quality of the research and improve patient centricity.

What worked well?

- Introductory workshop- Having a thorough introductory workshop with The Leukemia & Lymphoma Society and Takeda was extremely useful. We were able to discuss the project goals and align on our shared purpose. Some collaborators had experience with this study methodology, but for those with less experience this workshop was fundamental in providing information on the study design, to ensure enough knowledge to contribute to the study effectively.
- Formal Collaboration Agreement- Outlined clear collaborator responsibilities and ensured that all parties were aware of and comfortable with their respective roles. This also defined remuneration and a payment schedule increasing transparency. Each key stakeholder brought a different perspective and knowledge, which enhanced the study during collaborative discussions.
- Project co-ordination- The Evidera project manager was responsible for co-ordination between all stakeholders, project timelines and keeping stakeholders updated on study progress.

- Designated points of contact- Evidera, Takeda and The Leukemia & Lymphoma Society had designated points of contact throughout the study, which meant that questions could be answered quickly.
- Patient Advocacy Organization involvement- The Leukemia & Lymphoma Society constituency is highly engaged in initiatives that promote the patient voice; advertising the opportunity for study participation to their constituency provided connection to patients. The Leukemia & Lymphoma Society was also able to identify a patient advisor interested in participating in the study. In the case of relatively rare or rare disease, recruitment via patient advocacy organizations represents not only a centralized alternative but also a recruitment platform that brings confidence to the study candidates.

Challenges and future projects:

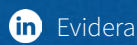
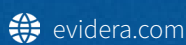
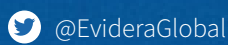
- Knowledge of methodology- More work is required to develop and test patient friendly materials explaining patient preference research methods, the issues raised by their design, the outputs they generate and how these are interpreted. Material to train non-expert patients in the field of patient preferences would allow the engagement of a more representative group since early stages of these type of studies.
- Awareness of study relevance and expectations- In this case, The Leukemia & Lymphoma Society constituents reported previous experience and interest in the patient preference studies. However not all Patient Advocacy Organizations are aware of the relevance of these studies and the degree of engagement may vary. Having a Patient Advocacy Organization-friendly way of presenting the opportunity may help to gain their interest and therefore encourage their engagement and may even trigger their input as co-investigators.
- Timeframe for contracting- Establishing our Collaboration Agreement formally took a long time, roughly 4 to 5 months. All parties involved agreed to continue working together prior to execution of the agreement. However, it would be useful in future to initiate this process at an earlier stage, possibly on proposal acceptance, and consider standardized agreements.
- Patient Engagement feedback- Currently, we are collecting feedback only from the Patient Advocacy Organization on their involvement in the study. However, it would be interesting to expand this feedback option to include study participants in future projects. Knowing how relevant they consider their input, or their participation, would inform us about potential gaps in the information materials or introduction to the study that explain the relevance of their involvement.



About Evidera

Evidera, a PPD business, is a leading provider of evidence-based solutions to demonstrate the real-world effectiveness, safety, and value of biopharmaceutical and biotechnology products. We provide integrated and tailored scientific expertise and global operational capabilities.

Evidera's Patient-Centered Research team specialises in capturing patient insights, such as patient reported outcomes and preferences, patient engagement, and understanding patients' uptake of and adherence to treatment. Evidera's Patient Preference Team is an industry leader in the design, implementation, and use of instruments to elicit patient preferences to inform healthcare decisions.



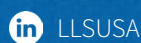
About Takeda

The Takeda Oncology mission remains constant - we endeavour to deliver novel medicines to patients with cancer worldwide through our commitment to science, breakthrough innovation and passion for improving the lives of patients. Delivering on the unique needs of each person with cancer has been at the core of our oncology business since our inception, and it remains at the very heart of our commitment, research and outreach. The strength and bravery of these individuals inspire and compel us every day. Alongside our boundary-pushing global R&D unit, Takeda Oncology's proven commercialization capabilities allow us to quickly respond to the diverse and ever-changing needs of patients, healthcare providers and health systems around the world. We are a force of more than 2,000 dedicated oncology professionals, driven by a patient-focused entrepreneurial spirit, and commitment to agility. We are working every day to achieve our aspiration to cure cancer.



About Leukemia & Lymphoma Society

As the global leader in the fight against blood cancer, The Leukemia & Lymphoma Society (LLS) is working tirelessly to find cures and provide more support for blood cancer patients and families than any organization in the world. Since 1949, LLS has invested nearly \$1.3 billion in cutting edge blood cancer research worldwide, leading to breakthroughs in treatment—including revolutionary CAR T-cell immunotherapies. LLS is the leading source of free information, education and support for blood cancer patients, caregivers, survivors, families and healthcare professionals. And through a nationwide grassroots network of more than 50,000 active online volunteers, LLS advocates for policies to protect cancer patients and break down barriers to care.



About the study team

This patient preference study was a collaboration between Evidera, Takeda and Leukemia & Lymphoma Society.



Kevin Marsh, PhD is an Executive Director at Evidera. Kevin completed his PhD at the University of Bath. He is an expert in the use of preference information and decision analysis to inform health decisions, including pipeline optimization, authorization, reimbursement, and prescription decisions. He is currently co-chair of the ISPOR Health Preference SIG and has chaired ISPOR taskforces and working groups.



Caitlin Thomas, MSc, is a Research Associate at Evidera, within the Patient Preference Team and is a member of Evidera's Patient Engagement Interest Group. Caitlin completed her MSc in Health Economics and Decision Science at University College London.



Ajibade O. Ashaye, MD, MBA, MPH, MSc is a Director of Global Evidence & Outcomes – Oncology at Takeda where he leverages his multidisciplinary training in Medicine, Health Care Management & Policy, Epidemiology, Health Economics, and Business administration. He leads global evidence, outcomes, and patient-centered strategies for haematology-oncology assets in Lymphomas and Leukemias from early development through commercialization.



Mehul R. Dalal, PhD is a Senior Director and Head of Hematology (Oncology) in Global Evidence and Outcomes at Takeda. In his role, he manages and oversees health/patient centered outcomes and real-world evidence strategies and studies across lymphoma, leukemia and myeloma malignancies. He has a background in health economics and health policy and services research across different therapy areas.



Scott Campbell, is a Director of Patient Advocacy & Engagement at Takeda Oncology. As the leader of Takeda's multiple myeloma and leukemia advocacy efforts, Scott drives efforts to integrate the patient perspective from clinical development through commercialization. As part of his work at Takeda, Scott manages relationships with patient organizations and collaborates internally with R&D and Commercial teams to gather patient insights to inform Takeda decisions.



Maria Sae-Hau, PhD, MS, is Senior Director of Access Initiatives & Evaluation at The Leukemia & Lymphoma Society. She manages the design, implementation, and evaluation of national patient access initiatives and multi-institutional research studies focused on blood cancer patients and caregivers. She is a Clinical Health Psychologist with a background in community-based health intervention research.



Meredith Barnhart, PhD, LCSW-R, OSW-C served as Director of the Information Resource Center at The Leukemia & Lymphoma Society at the time of this project. In this role, she oversaw a team of 14 master's level oncology professionals who provide comprehensive, personalized, and up-to-date information on blood cancer treatment options including clinical trials, educational and emotional support, and financial resources. She had previously served as practicing pediatric oncology social worker.



Elisa S. Weiss, PhD is Senior Vice President of Education, Services & Health Research at The Leukemia & Lymphoma Society. In this role, she oversees the development, implementation and assessment of patient and professional education initiatives, as well as LLS's Information Resource Center, Clinical Trial Support Center, all field outreach and support, and several psychosocial and health services research projects that aim to foster equity in access to care.



R&D Key Performance Indicators and Patient Engagement Planning -A collection of cases

Organization: Takeda

The PFMD
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3rd edition | 2020





R&D Key Performance Indicators and Patient Engagement Planning - A collection of cases

Organization:
Takeda

Basic Information

Takeda is committed to partnering with patients in the process of drug development. To help bring about this shift in mindset across R&D, we have implemented a Key Performance Indicator (KPI) evolution which began in 2018. The KPIs are effectively creating a “push and pull” whereby global program teams (GPTs) must carry out activities but the teams are thinking through implementation of activities where the patient perspective will be valuable in informing the development of their asset.



In the cases featured below, we describe how the Patient Engagement mindset is embedded within the R&D culture through the KPI implementation as a proactive roadmap designed to optimize and clarify why to engage with patients and when best to do so in the asset lifecycle of a potential medicine. Additionally, 3 specific examples are provided to highlight patient engagement approaches that may stem from this proactive planning, such as via a longitudinal patient advisory panel, a patient advisory board in clinical research, and also in early drug discovery - all designed to advise Takeda R&D on how best to partner with patients throughout the medicines development continuum.

Use of R&D Key Performance Indicators: Beginning in 2018, all R&D employees were required to complete three Patient-themed activities (which included examples such as attending a panel discussion given by patients sharing their experience, attending an education day offered by a patient organization, volunteering at a hospital, reading a book or watching a video about a disease or condition that offered the patients' perspective). These activities helped bring employees closer to patients and their experiences particularly since employees were able to choose which would be most meaningful to them. Feedback from employees emphasized how valuable and inspiring these experiences were as well as how motivating. Building on that in 2019, the R&D Patient Engagement KPI focused on the more narrowly defined activity, patient engagement. This KPI stipulated that 100% of Global Program Teams (GPTs) were to identify and track one or more specific patient engagement activity (two-way dialogue with patients to more deeply understand the patient experience, in order to meet a shared purpose and mutual benefit, resulting in measurable outcomes) in their 2019 goals, and that 30% of GPTs would establish a Patient Engagement Plan in 2019, moving to 100% in 2020.

Patient Engagement Plan (PEP): The PEP is a roadmap to optimize patient engagement opportunities throughout the asset lifecycle. It ensures that GPTs and commercial functions, along with patient facing functions (i.e., Patient Engagement, Patient Advocacy, Patient Recruitment & Retention), are aligned on how and when the company will seek patient input. Key components of the PEP process include:

- setting expectations with GPT leadership;
- reviewing source materials (e.g., previously completed engagements with patients, relationships with patient groups, asset strategy, Target Product Profile, team goals);
- preparing for and completing a three-hour cross-functional brainstorming workshop; and
- developing the Patient Engagement Plan roadmap output.

The R&D Patient Engagement Office (a centralized center of excellence) supports the GPT with the implementation of specific patient engagement activities so that lessons learned, and best practices can be shared, global policies and internal guidance can be applied in a consistent manner and GPTs can be supported through the process.

Lessons learned

Advancing a culture of patient-centric R&D is a goal that has obtained leadership approval and support. In practice, achieving this goal requires a multi-pronged approach including communications, training, cross-functional alignment, and resources for patient engagement.

The 2019 R&D KPI requiring 30% of GPTs to complete a PEP provided the opportunity to systematically and thoughtfully embed patient engagement into GPT plans in alignment with team development priorities and timelines.

At the GPT level, employees care about how their work impacts patients and ensuring the medicines they develop meet patient needs. Many do not work in patient facing functions or have experience with patient engagement and thus the Patient Engagement Plan process provides knowledge and training on the value of patient engagement, and best practices for patient engagement, in an interactive and collaborative way. The involvement of all three patient facing functions in these plans educates teams on the role of Patient Engagement, Patient Advocacy, and Patient Recruitment & Retention, and reinforces how the roles are distinct but complementary – all supporting GPTs to undertake quality engagements with patients and patient communities.

For additional information please check [Annex R&D Key Performance Indicators and Patient Engagement Planning Supplementary Information](#) .

Projects description

Patient Engagement Patient Advisory Council (PEPAC)

Takeda recognizes that patients possess a deep understanding of their disease, its symptoms, and treatments and can offer a relevant and often nuanced perspective to the drug discovery and development process. In line with the Takeda's commitment to ensuring therapies are developed with patient needs and priorities as the focal point, the Head of the R&D Patient Engagement Office formed a standing body to advise R&D on patient perspectives on drug development representing multiple disease areas, countries, regions, backgrounds and experiences. In addition to developing the council, the team built an efficient and consistent process for bringing patient perspectives into R&D through a standing-body that has engaged with Takeda on four topic specific sessions over eight months – insights have been shared with internal teams to create real change informed by the patient perspective.

Understanding the celiac patient experience to inform research and development

Celiac disease is common and affects over 1% of the population. Takeda's celiac global study team is interested in gaining a deeper understanding of the experience of celiac patients' post-diagnosis and the extent that symptoms persist despite best efforts at a gluten-free diet. As celiac disease is a relatively new area for clinical development, there were no existing optimized trial design models and deeper insights as to the patient experience are needed.

The celiac study team worked with the R&D Patient Engagement Office and external vendor to implement several patient engagement modalities, starting with a landscape assessment to understand the global and local patient organizations advancing research and advocacy in this disease area, leading to a patient engagement advisory board occurring soon after a Key Opinion Leader (KOL) advisory board to allow the study team to compare and contrast the payer and patient perspectives on unmet needs, and continuing onto contracting with several longitudinal advisors who now serve as ad-hoc members of the study team to provide important patient insights throughout the medicine development lifecycle.

Patient engagement in early discovery to inform research direction

Neurogenic Bowel Dysfunction has been an area of research interest for Takeda. This early research team in the Gastroenterology unit was interested in expanding their understanding of the neurogenic bowel dysfunction burden for patients and their care partners by engaging in active, direct dialogue with these advisors during two multi-stakeholder advisory boards. In addition to using social media, engaging with key opinion leaders, and gathering insights from published literature, the research team followed their strong hunch that exploring with patients and their families directly could uncover important insights that might not otherwise surface. This team wanted to explore a potential disconnect between HCPs (Healthcare Practitioners) and patient/caregiver perceptions of neurogenic bowel dysfunction burden and unmet needs for individuals living with Parkinson's Disease and Spinal Cord Injury.

Two advisory boards, one focused on Parkinson's Disease and one focused on Spinal Cord Injury, were designed to develop in-depth understanding of patient psychosocial, practical and clinical challenges, unmet needs, and touchpoints from

In the description below, more context is shared about how Takeda has conducted the patient advisory board to gain direct patient and care partner insights to understand which symptoms matter most to patients, how they manage their disease, and their views on possible treatments. The team also sought feedback on a potential clinical trial design.

symptoms through diagnosis and treatment.

For the research team, the expected outcome of the meetings was to offer recommendations and implications for drug discovery and development based on learnings. At a higher level, this engagement provided an opportunity for Takeda to understand how patient engagement in early research can contribute to internal decision-making and research prioritization.

Project timeline:

- Start Date: March 2019
- End Date: 31st December 2020

Geographic focus:

- Europe, North America, Asia

Purpose of initiative:



Involving patients in the lifecycle of medicine development

Initiative focus area:

- Research

In which phases of research:

- Clinical Trials

Project timeline:

- Start Date: September 2017
- End Date: Ongoing

Geographic focus:

- Global

Purpose of initiative:



Involving patients in the lifecycle of medicine development

Initiative focus area:

- Research

In which phases of research:

- Onset of symptoms
- Newly Diagnosed

Project timeline:

- Start Date: August 2018
- End Date: January 2019

Geographic focus:

- National: United States

Purpose of initiative:



Involving patients in the lifecycle of medicine development

Initiative focus area:

- Research

In which phases of research:

- Onset of symptoms
- Treatment

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

- Research and discovery phase
- Pre-clinical phase
- Clinical study phases I, II, III
- Post launch activities

Which stakeholders does this project involve?:

- Patients and carers
- Patient advocates, patient organizations and associations
- Pharmaceutical companies or industry

- Treatment
- Clinical Trial
- Long Term Management

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

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- Clinical study phases I, II, III
- Post launch activities

Which stakeholders does this project involve?:

- Patients and carers
- Patient advocates, patient organizations and associations
- Pharmaceutical companies or industry

- Long Term Management

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

- Research and discovery phase

Which stakeholders does this project involve?:

- Patients and carers
- Patient advocates, patient organizations and associations
- Healthcare professionals
- Pharmaceutical companies or industry

The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)



1. Shared purpose

Patient Engagement Patient Advisory Council (PEPAC)

The stated shared purpose is to collaborate on an ongoing basis with patients and care partners to help Takeda move from developing medicines for patients to developing medicines with patients. To ensure the shared purpose was established and agreed upon, a launch call was hosted in August 2019. During the kickoff, Takeda set out to confirm each PEPAC advisor's reason for participating and what they hope to get out of the engagement as well as to present the vision and goals for the long-term engagement for comment and confirmation. At the launch of PEPAC, advisors were asked about their level of interest in a range of topics so that focus could be given to topics of mutual interest. Following each topic/session with PEPAC, all advisors are asked to share feedback and ensure the initiative continues to live up to the agreed upon shared purpose.

Understanding the celiac patient experience to inform research and development

The goals of the patient and care partner advisory board was to deepen understanding of the patient experience of celiac disease and patients were eager to help Takeda understand unmet needs and design trials to help in developing a potential new medicine. Specifically, the study team desired to:

- Better understand the patient experience and relationship with gluten
- Identify unmet needs in celiac disease management
- Seek participants' feedback on a potential trial design The vendor partner communicated the meeting purpose to external stakeholders at several points during planning and execution.
- Prior to being invited to join the advisory board, all external stakeholders participated in a telephone call with the Takeda's

Patient engagement in early discovery to inform research direction

The shared purpose for the advisory board meetings was to:

- Share the Takeda's approach to research in Neurogenic Bowel Dysfunction for advisors to understand and advise company with an interest in address patients' unmet needs
- Build a greater understanding of how people living with Spinal Cord Injuries and Parkinson's Disease experience and manage bowel function
- Align on unmet needs for improving bowel management

This shared purpose aligns with the research team's objectives for the project and was communicated with external stakeholders at several points during planning and execution.

- Prior to being invited to join the advisory board, all external stakeholders participated



patient engagement vendor partner. These conversations allowed the vendor to introduce the company and its research interests in Celiac disease and provide information on the advisory board and its objectives. Prospective advisors were able to ask the vendor clarifying questions, share their personal story, and consider whether they would like to participate in an advisory board. During the advisory board, patient and care partner participants shared very personal experiences and emotions – ones that they may not even be comfortable sharing with their physicians. The introductory calls were an important part of the engagement approach, supporting the creation of a trusting, psychologically safe environment for patient and care partner advisors to share their perspectives.

- Information shared during introductory phone conversations contributed to the advisory board agenda and discussion guide and helped Takeda understand the unique needs of participants (e.g. strict dietary restrictions and access to emergency medical care if needed).
- The shared purpose was again communicated with participants within the advisory board invitation.
- The advisors' "welcome letter" upon hotel

in a telephone call with the Takeda's patient engagement vendor partner.

These conversations allowed the vendor to share information about the company and its research interests in Neurogenic Bowel Dysfunction and provide information on the advisory board and objectives. Participants were able to ask the vendor clarifying questions, as well as share their perspectives on the purposes of the engagement from their personal lived experience, or the perspective of advocating for and working with the patient community. Information shared during these conversations contributed to the advisory board agenda and discussion guide and helped all to understand unique needs people participating in the advisory board may have.

- The shared purpose was articulated in the communication with participants within the advisory board invitation.
- When participants arrived at their hotel accommodation for the advisory board, they were provided a "welcome letter" at check-in that in addition to logistical information reiterated the objectives of the meeting.
- The evening prior to the advisory board meeting an informal welcome reception/dinner was held. This served as an opportunity for the research team and external advisors to meet in

check-in included logistical information, reiterating the objectives of the meeting.

- The evening prior to the advisory board meeting, an informal welcome reception/dinner was held. This served as an opportunity for the study team and external advisors to meet in a less formal setting, establish rapport, and create a foundation of psychological safety for the next day's meeting.
- At the start of the advisory board, the shared purpose was again reiterated. During introductions, each participant shared their name and their experience with the condition and diagnosis.

For all R&D Patient Engagement Activities, the aim is to achieve the agreed upon objectives for the celiac study team and to ensure that external participants feel heard and valued. This expectation was set at the beginning of the advisory board during the welcoming remarks. At the close of the meeting, internal stakeholders were asked to share an "a-ha" moment and patient/care partner advisors were polled on whether this objective has been met, both in-person and in evaluation forms.

Several months later after the advisory board, the company hosted a virtual share back meeting, which convened the patient and care partner participants voluntarily to articulate what the clinical study

a less formal environment and to share what they hoped to get out of the engagement. This approach is an important part of creating a trusting environment for the patient and care partner advisors that feels psychologically safe for them to share very personal experiences and emotions that they might not even be comfortable sharing with their physicians or patient communities.

- At the start of the advisory board, the shared purpose was again reiterated. During introductions, each participant shared their name, experience with the condition, and one thing they hoped to get out of the day.

In addition to these objectives, all patient engagements at the company have the objective that the research team walks away with impactful insights, and that external participants feel heard and valued. This expectation was set at the beginning of each advisory board during the welcoming remarks. At the end of each meeting, internal stakeholders were asked to share an "a-ha" moment and external participants were asked to share "what is the single most important thing you would like the researchers to know about the daily experience of people living with bowel challenges?". In meeting evaluation forms, participants were asked whether they felt their voices were heard during the meeting. 100% of participants stated that they felt they were heard.

team had learned from the advisors directly and how those insights are shaping research decisions, as well as plans to further engage with the celiac disease community, patient organizations, and healthcare practitioners to amplify the learnings through educational materials.

2. Respect and accessibility



Patient Engagement Patient Advisory Council (PEPAC)

Respect and accessibility were considered and implemented in the following ways:

- All topic session questions and materials are accompanied by a concise document that provides background and context in simple, jargon free language
- All participants are compensated for their time based on the fair market value (FMV) hourly rate according to local country guidelines and based on their level of education and involvement in the community

Understanding the celiac patient experience to inform research and development

The R&D Patient Engagement Office engaged a third-party vendor to support the execution of the advisory board. The vendor took great care to plan and manage the advisory board to help advisors through the contracting process and with meeting logistics (e.g., travel, accommodations).

Additionally, specific attention was given to dietary considerations for people living with celiac, with the vendor working closely with the caterer to ensure meals provided were free of gluten contamination.

In addition to working with participants on their

Patient engagement in early discovery to inform research direction

The external stakeholders at the advisory board meetings included people living with spinal cord injury and people living with Parkinson's disease. As such, it was important that meeting logistics considered their unique needs. The partner vendor worked with each participant to understand their unique needs related to travel, accommodation, meeting room accessibility, and dietary preferences.

For individuals living with spinal cord injury:

- Accommodations included securing handicap accessible hotel rooms, directing a local

- Each session utilizes two platforms for engagement:
- Secure online communication tool used to allow PEPAC members to review documents on their own time across time zones and the platform allows members to visit and revisit to provide feedback at a pace that works for their comfort-level
- Live teleconference call(s) scheduled at mutually agreed upon times to allow for members to speak to their insights and feedback, often 2 or more calls are conducted to be mindful of advisors needs, time zone and restrictions
- As stated in the previous section – after each engagement members are invited to provide feedback on the engagement process to ensure we continue to fulfill our shared purposed in a manner that is respectful and accessible

logistical needs prior to participation, the vendor partner shared the schedule of events, including travel and accommodation information.

All participants were provided with the direct contact information (e.g., cell phone) of the vendor partner who was onsite at the meeting to help troubleshoot any challenges that arose. All participants were paid honoraria for their time and incidental costs were covered. The vendor partner booked all air fare, accommodations, and car service to reduce up-front out-of-pocket expenses for participants.

The R&D Patient Engagement Office and vendor partner worked with the study team to ensure presentations during the meeting were written in appropriate, easy to understand language. Preparation calls were held with internal stakeholders to provide guidance on how to respectfully engage patient and care partner stakeholders including use of language and terminology and listening to and respecting diverse viewpoints.

A welcome dinner prior to the advisory board helped to foster a connection and psychological safety between company celiac program team, R&D Patient Engagement Office and patient/care partner advisors prior to the more formal advisory board meeting

participant to the availability of handicap accessible parking, ensuring airlines were notified of needs, securing wheelchair accessible vans for local transportation, and ensuring the meeting venue was accessible and that they had any needed assistance.

- During meeting room setup, it was realized that the table height in the room may not be an appropriate height to accommodate a wheelchair. Table height was adjusted accordingly.
- While there is a preference at the company to hold patient engagement advisory boards in person, as opposed to virtual participation, exceptions were made for a participant with spinal cord injury that no longer travels long distances. Takeda's IT department and the vendor partner worked with this participant to ensure she was able to participate in the meeting by video conference.

For individuals living with Parkinson's Disease:

- Accommodations made included securing local transportation for the advisory board meeting, working with airlines to ensure comfort and accessibility during air travel, and extending invitations to care partners where the participant felt this additional support would be of value.

In addition to working with participants on their



		<p>logistical needs prior to participation, the vendor partner shared logistics notes outlining schedule of events and including travel and accommodation information. All participants were provided with the direct contact information (e.g., cell phone) of the vendor partner who was onsite at the meeting to help troubleshoot any challenges that arose.</p> <p>All participants were paid honoraria for their time and incidental costs were covered. The vendor partner booked all airfare, accommodations, and car service to reduce up-front out-of-pocket expenses for participants.</p> <p>The R&D Patient Engagement Office and vendor partner worked with the research team to ensure their presentations during the meeting were written in appropriate, easy to understand language. Preparation calls were held with the research team to provide internal participants with guidance on how to respectfully engage external stakeholders including use of language and terminology and listening to and respecting diverse viewpoints.</p> <p>The welcome dinner prior to both advisory boards helped to foster a connection and psychological safety between the Takeda's global research team, the patient engagement office, and patient/ care partner advisors prior to the more formal advisory board meeting.</p>
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3. Representativeness of stakeholders

Patient Engagement Patient Advisory Council (PEPAC)

To initiate the recruitment and confirmation of individuals for this PEPAC engagement the PEO agreed upon the criteria for diversity and representativeness listed at the end of this section. Once criteria were agreed upon the PEO worked through Patient Advocacy Organizations for introductions to individuals for an initial phone screen and once a full list of individuals was populated the PEO selected ten to twelve members to invite and contract for the long-term engagement. Please also refer to the [Annex 2 Supplementary information PEPAC](#) information. Including diversity and representativeness on the council allowed for robust discussions where the Company benefited from the full experience of patients and care partners in the real world.

Diversity in perspectives

- Age
- Gender
- Race/ethnicity
- Socioeconomic

Understanding the celiac patient experience to inform research and development

In order to support project objectives, the team decided to recruit a diverse group of patients living with celiac and several care partners to participate in the advisory board. Participants were found through networks managed by the vendor partner, including patient organizations. An initial screening and full assessment were conducted upon receipt of a completed opt-in form from a potential participant. This assessment gathered information on demographics, disease status, burden of disease, treatment experience, and clinical trial experience. Upon completion of assessment, a bio was developed and shared with the celiac study team highlighting these specific areas. The comprehensive group of interested participants was evaluated to select a diverse advisory board, considering gender, geography, age, ethnicity, and educational level and profession (as a proxy for socioeconomic status). Internal stakeholders attending the advisory board included the R&D Patient Engagement Office and celiac study team members from functions including global clinical science, global outcomes research, and global

Patient engagement in early discovery to inform research direction

External participants were made up of people living with the condition, care partners and advocates. For the spinal cord injury advisory board, the multi-stakeholder group of participants also included medical professionals who work closely with people living with spinal cord injury. Internal stakeholders included early research team members, the R&D Patient Engagement Office, and the vendor partner who facilitated the meetings and provided on-site logistical support to all stakeholders.

People living with the condition represented their personal lived experiences and shared insights from their peers. Advocates and professionals provided a broader perspective based on their work advocating for and working with the patient populations. Advocates at both the Parkinson's Disease and Spinal Cord Injury advisory boards shared data they had collected from their communities related to the burden of bowel dysfunction.

An effort was made to secure a diverse group of participants by working with advocacy organizations, including:



- Education level
- Health literacy
- Rural/urban/suburban
- Professional experience

Representation of perspectives

- Geography (EU; Japan; US)
- Therapeutic area (Gastroenterology; Neuroscience; Oncology; Rare diseases)
- Experience (Care-partner; individual lay patient; patient advocate and patient opinion leader)

clinical operations. The patient engagement vendor attended the meeting to support participants and facilitate a two-way dialogue between company, patients, and care partners.

- Time since diagnosis/injury
- Disease progression/injury type
- Gender, ethnicity/race, age, educational level and profession (as a proxy for socioeconomic status), and geographic location

During project debrief, the R&D Patient Engagement Office identified recommendations to increase the diversity of future engagements including working with patient groups and community leaders that have deep ties to minority populations and considering the use of virtual meetings to expand accessibility to those with more severe disabilities or for whom travel from a rural location is burdensome.



4. Roles and responsibilities

Patient Engagement Patient Advisory Council (PEPAC)

During screening calls, through contracting negotiations, and during the launch call (referred to in section 2.) proposed roles and responsibilities were shared with PEPAC to allow for comment and agreement. Following the launch call a survey that summarized the preferences was distributed for anonymous feedback to obtain final agreement for roles and responsibilities and ways of working together in a longitudinal manner. Please refer to the [Annex 2 Supplementary information PEPAC](#) for additional detail on roles and responsibilities.

Understanding the celiac patient experience to inform research and development

In the kick-off meeting with internal stakeholders, roles and responsibilities were defined. Standard meeting documents – agendas, minutes and action items – helped to define the roles and responsibilities for the activity’s progression. The R&D Patient Engagement Office and vendor worked closely with the celiac team to develop agendas, discussion guides, and presentation materials to ensure alignment and to gain their medical and scientific input. Before patient engagement activities, the R&D Patient Engagement Office met with the study team to share guidance on interacting with patients and care partners. The objective of the session was to ensure internal stakeholders understood their roles at the advisory board and were prepared to communicate in a respectful way. Among the topics covered were active listening, respectful behavior, and how to build an open and honest dialogue. From the outset of the project, the celiac team and R&D Patient Engagement Office enlisted a third-party vendor partner to provide a consistent point of contact with all stakeholders and to manage the project. The vendor worked closely with external advisors to prepare them for meeting participation,

Patient engagement in early discovery to inform research direction

From the outset of the project, the research team and R&D Patient Engagement Office engaged a third-party vendor partner to provide a consistent point of contact with all stakeholders and to manage the project. The vendor worked closely with the advisory board participants to prepare them for meeting participation, including walking them through the contracting process and emphasizing that their role was to share their personal experience or perspective on how bowel dysfunction issues impact people living with either Parkinson’s Disease or Spinal Cord Injury. The role of the external advisors was communicated to both the research team, and to advisors throughout the project including during the welcome dinner at the beginning of each advisory board.

The R&D Patient Engagement Office and vendor worked closely with the research team to develop the advisory board agenda, discussion guide, and presentation materials to ensure alignment and to gain their medical and scientific input. Prior to the advisory board meeting, a preparation meeting was held with all internal stakeholders to walk through

emphasizing that their role was to share their personal experience or perspective, living with or caring for an individual with celiac. The role of the external advisors was communicated to both the study team and to advisors throughout the project, including through:

- Correspondence with the patient engagement vendor partner,
- Written contracts, with the vendor supporting participants in answering questions about the contract,
- A welcome reception prior to the advisory board, and
- Reiterating roles at the outset of the meeting.

During the meeting, the vendor's professional moderator facilitated a two-way dialogue, ensuring that all external participants had the opportunity to share their perspectives, and that the internal stakeholders had the opportunity to listen and ask questions. The use of interactive exercises allowed participants to provide their perspectives in a variety of formats

the flow of the advisory board and ensure all internal stakeholders were clear on their role in the meeting. The vendor partner facilitated the advisory board meeting, ensuring that all external participants had the opportunity to share their perspectives, and that the internal stakeholders had the opportunity to ask questions.



5. Capacity and capability for engagement

Patient Engagement Patient Advisory Council (PEPAC)

Since the aim of the PEPAC engagement is to gain the patient and care partner perspective on Company materials, programs and resources, Takeda's R&D PE group, together with specialized external consulting, spend considerable time and effort ensuring that internal colleagues are trained and prepared in best practices of communicating with advisors. Materials and questions are created, reviewed and approved to ensure that they include accessible and appropriate language and terms for the advisors as well as making sure there is a small team with the capacity to address any questions or concerns in a timely manner.

Understanding the celiac patient experience to inform research and development

As part of the advisor selection process, the partnering vendor interviewed prospective meeting participants. The purpose is to ascertain whether the individual has the appropriate medical history to participate, and to gauge the individual's comfort level and ability to contribute to an interactive, group discussion. Once participation was agreed upon, the vendor partner worked closely with each confirmed advisor to ensure that any special needs were understood and accommodated. As with other R&D patient advisory boards convened by the company, a detailed discussion guide was prepared, outlining the meeting objectives, the methodology, the agenda, and the content to be facilitated by the moderator. The discussion guide was an internal reference for the company stakeholders. At the meeting, slides were used to facilitate discussion. The R&D Patient Engagement Office worked closely with the celiac team to ensure that the meeting slides were appropriate for patients and care partners, and that health literacy principles were followed. During the advisory board meeting, the moderator paused to ensure all stakeholders had the opportunity to ask questions about terms or subjects before engaging in deeper discussion.

Patient engagement in early discovery to inform research direction

By identifying patient participants through advocacy group partners, the company was able to ensure that participants had a level of comfort with sharing their personal experiences with bowel dysfunction. All patients who participated in the meeting have also acted as advocates themselves, including serving as Ambassadors or peer mentors, or having participated in an advocacy group training program.

Prior to the advisory board meeting, the vendor partner undertook a series of informational interviews with community members from both the Parkinson's and spinal cord injury communities to ensure the terms used in the meeting would resonate with stakeholders. A clear example of the value of this approach was the use of the term "neurogenic bowel dysfunction". Prior to the project, the research team was using the term "neuropathic constipation" to describe the condition. Feedback from the spinal cord injury community was that this term didn't resonate with them, but that "neurogenic bowel dysfunction" was commonly understood.

During the advisory board meeting, facilitators paused to ensure all stakeholders had the

		<p>opportunity to ask questions about terms or subjects before diving into discussion. To level set and create common understanding, for both meetings, a portion of the agenda up front was spent defining what bowel dysfunction looks like and means to patients and people advocating for patients.</p>
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6. Transparency in communication and documentation



Patient Engagement Patient Advisory Council (PEPAC)

PEPAC is a longitudinal engagement where members are consulted approximately every six to eight weeks on topics of interest to R&D organization. In advance of each engagement the members receive the documents for review as well as the background and context information; members are offered approximately one to two weeks' time (depending upon the complexity of the session) to comment on materials and questions offered in the virtual on-line tool as well as to consider and respond to others

Understanding the celiac patient experience to inform research and development

The use of an external vendor provided external participants with a single point of contact for pre-meeting communications. These communications included:

- A preparatory phone call
- Formal meeting invitation
- Phone calls and emails to arrange logistical details

Patient engagement in early discovery to inform research direction

The use of an external vendor provided patient and advocate participants with a single point of contact for pre-meeting communications. These communications included:

- A preparatory phone call
- Formal meeting invitation
- Phone calls and emails to arrange logistical details



comments; soon after the close of the online session a live call is hosted to review the session feedback summary and gain deeper insights on the patient and care partner perspectives. The on-line call serves the purpose of allowing a two-way dialogue with stakeholders as well as fostering community among the PEPAC members. Following the close of the online engagement the team is provided the session transcript to see the back and forth dialogue and the high-level summary to properly prepare for member engagement on the live call. Then following the live call, the team receives a full summary including additional insights and points of agreement from the live call.

- Pre-meeting logistics note
- Welcome letter on arrival at the hotel
- Post-meeting compensation and documentation

Regular project check-ins were established among internal stakeholders, including:

- Weekly meetings between the R&D Patient Engagement Office and vendor
- Bi-weekly meetings between the R&D Patient Engagement Office, celiac study team and vendor
- Meeting documentation, including agendas and minutes to capture decisions and action items

The vendor facilitated the using slides developed from the discussion guide. Flip-charting allowed participants to refer to points made. Feedback from external stakeholders was collected via an evaluation form at the close of the meeting to assess the quality of the engagement. The evaluation collected feedback on clarity of meeting objectives and content, and quality of discussion, meeting facilitation and organization.

A comprehensive report was developed after the advisory board and reviewed by internal stakeholders. Several months after the advisory board, the company hosted a virtual share back meeting with the advisory board participants.

- Preparatory calls and correspondence with participants providing data/survey results during the meeting

- Pre-meeting logistics note
- Welcome letter on arrival at the hotel

Regular project check-ins were established among internal stakeholders, including:

- Weekly meetings between the R&D Patient Engagement Office and vendor
- Bi-weekly meetings between the R&D Patient Engagement Office, research team and vendor

During the advisory board meeting, participants were provided with a briefing book which included:

- Logistical information
- Agenda and flow of the day
- Venue maps
- Short bios of participants

The vendor facilitated the advisory board using a discussion guide. On post-meeting evaluation forms, participants noted what worked well, including:

- Interaction between all participants
- Openness in communication and conversation; group size was appropriate to discussion
- Use of discussion and visuals, including flip charting – being able to see written notes helped



This allowed the company to share directly with advisors what the study team had learned and how those insights are shaping research decisions. The company also shared their plans to further engage with the celiac disease patient and care partner community, patient organizations, and healthcare practitioners to amplify the learnings through educational materials.

- refer discussion back to the meeting objectives
- Willingness of internal stakeholders to actively listen and ask questions

7. Continuity and sustainability



Patient Engagement Patient Advisory Council (PEPAC)

Contracting and engaging with the PEPAC members in a longitudinal manner allows for continuity and sustainability in the engagement as well as the opportunity to build trust and continue to confirm the engagements fulfillment of the shared purpose agreed to at the outset of the engagement. PEPAC advisors continually show a strong interest in helping Company understand their perspective in order that Company can best their needs and interests.

Understanding the celiac patient experience to inform research and development

The R&D Patient Engagement Office views engaging in a two-way dialogue as essential to truly understanding the patient perspective and embedding it in the company's clinical development program and asset value proposition. Given the important learnings gained from the first advisory board convened, the celiac team decided to continue to extend and expand their engagements with the patient community. One way in which the learnings and impact of the advisory board was extended was through the establishment of a patient and care partner longitudinal advisory panel. Longitudinal

Patient engagement in early discovery to inform research direction

A comprehensive report was created following the advisory board meetings. These reports were presented to the research team and R&D Patient Engagement Office for discussion and alignment on key impactful learnings. To share the learnings beyond the research team, the R&D Patient Engagement Office developed a video that features the research team leads discussing the project, its value, and what they learned. This video is widely available to all R&D colleagues.

advisors enable the study team to obtain insights from diagnosed patients and their care partners on a variety of topics such as the patient journey, symptom management/treatment, unmet needs, meaningful treatment outcomes, disease impact on quality of life, challenges/motivators to clinical trial participation, Patient Reported Outcomes questionnaires, and route of administration. Longitudinal advisors are engaged in virtual, online and face-to-face discussions, as appropriate to the advisor's background and comfort level, and the objective for the engagement.

The progression of patient engagement activities allowed the team to confirm previous learnings about the patient perspective and demonstrate its commitment to improve patient outcomes.

Results, outcomes and impact

Patient Engagement Patient Advisory Council (PEPAC)

Direct or indirect positive impact for patients

Check [here](#) engagements conducted thus far.

Overall, PEPAC engagement provides patients and care partners the opportunity to engage in a two-way dialogue with one another and Takeda R&D team members. This dialogue deepens their understanding of the clinical development process; the role of industry in medicines developments as well as creates a space for patients and care partners to provide fruitful and insightful feedback to the company.

Direct or indirect positive impact for non-patient project stakeholders

PEPAC demonstrates the impact that non-traditional stakeholders can have on medicines development. While industry typically engages health care providers and other professionals to inform medicines development PEPAC brings a new voice into the process to ensure the medicines developed offer meaningful outcomes for patients.

Understanding the celiac patient experience to inform research and development

Outcomes and impact of this initiative

The advisory board meeting achieved the objectives defined by the global celiac study team in that:

- Patient feedback on an element of the study design led to an opportunity to work with regulators to make the protocol less burdensome to patients.
- Study teams learned how patients and care partners describe and view their conditions, including terms used to describe gluten exposure. Education for healthcare practitioners on the burden of disease surfaced as a key unmet need. Understanding the language that resonates with the patient community supports development of educational materials for healthcare practitioners.

Positive impact for the specific medicines development phases

The feedback from patients and care partners at the advisory board has helped inform the study team's direction and decisions for its clinical studies. Specifically, insights:

Patient engagement in early discovery to inform research direction

Outcomes and impact of this initiative

In addition to research completed through social media and with medical professionals, the clear significant unmet needs clarified by advisory board participants led to the progression of the research program for one of the conditions, as opposed to another.

Positive impact for the specific medicines development phases

The project allowed researchers to learn directly from patient community representatives, resulting in informed business decisions about the direction of a research program.

Direct and indirect positive impact for patients

The findings are allowing the research team to prioritize its research agenda to focus on indications where there is the highest treatment unmet need for patients and where modes of action have the highest promise to improve the patient experience.

Direct and indirect positive impact for non-patient project stakeholders

The research team deepened its understanding of

- Generated strong internal support to move forward with the development program
- Informed elements of trial design
- Provided patients and care partners with a fuller understanding of how clinical studies are conducted
- Established respectful relationships with the patient community as continuing partners as the study team progresses along the drug development process

Direct or indirect positive impact for patients

- Advising company on the existing unmet need for new treatments for celiac disease generated strong internal support to move forward with the celiac program with urgency to bring potential new medicines to people living with celiac disease
- Patient advisors who are meeting each other for the first time in this advisory board formed a tight bond with each other and serve as resources and emotional support for each other after the event
- More awareness of how clinical trials are conducted and confidence in possibly participating to help advance the research or option for treatment while on trial

Direct or indirect positive impact for non-patient

the impact of bowel dysfunction on people living with spinal cord injury and Parkinson's disease. At a high-level, the team learned that neurogenic bowel issues greatly impact quality of life and is not adequately address with current treatment in one disease area and not so much in the other

This information is impacting the direction of this research program and its priorities. To hear directly from the research team on the impact the project had on them, view this video [here](#).

project stakeholders

Takeda's study teams recognize that patients possess a deep understanding of their disease, its symptoms and treatments, and can offer a relevant and often nuanced perspective to the drug development process, which has resulted in the company more fully moving towards developing medicines with patients. Study team members left the advisory board with:

- A more informed understanding of the disease elevated opportunities for company to make study design more patient-friendly
- A new understanding of the impact of celiac disease on care partners
- Insights allowed the study team to build off what they had heard from health care practitioners, and to take back to health care practitioner advisors what they were hearing from patients.

Hear directly from the celiac team on the value of engaging patients [here](#).

Learned lessons

Patient Engagement Patient Advisory Council (PEPAC)

A few challenges faced in the beginning phase of the initiative were confirming the right individuals to contribute to a fruitful and dynamic dialogue as well as selecting platforms to allow for a two-way dialogue across many time zones. Maintaining openness to adjusting our approach to meet the needs of the PEPAC membership has allowed us to implement slight adjustments such as how often members want to be reminded of open sessions and what types of materials the members desire to participate in an informed manner. We had hoped for an in-person meeting, however logistical challenges and then COVID-19 concerns have led us to believe we will continue virtually using the format we have put in place. Finally, regarding facilitation/management of the live calls, PEPAC interacts with a new internal team each time, we learned we needed to prepare the Takeda teams for interaction, as well as tighter facilitation of live calls to ensure all parties have a chance to contribute.

Understanding the celiac patient experience to inform research and development

The R&D Patient Engagement Office (a centralized center of excellence) supports Takeda study teams with the implementation of specific patient engagement activities so that lessons learned and best practices can be shared, global policies and internal guidance can be applied in a consistent manner, and study teams can be supported through the process. From this engagement, the company learned:

- Understanding how patients are feeling, which symptoms are ongoing, and which are most troublesome and burdensome by directly engaging patients and care partners is a critical early step in drug development.
- Ongoing engagement with longitudinal advisors allows for the deepening of insights and for confirming insights as asset development progresses.
- While you can't capture every patient perspective in a single advisory board, with a carefully chosen stakeholders and a thoughtful approach to the discussion, you can begin to get a much better sense of the diversity of opinions

Patient engagement in early discovery to inform research direction

This project served as a pilot for understanding how patient engagement in early research can contribute to decision-making and prioritization. The quality of insights the research team gained from the engagement did contribute to decision-making and confirms that patient engagement has value, even in early research and development.

One shortcoming of the project was not sharing learnings back to external stakeholders after the advisory boards. This process of sharing back has now become standard for R&D patient engagements and is of great value to all stakeholders.

While in person engagements are often preferred, the ability for people with some health conditions to travel may be limited, especially when travelling from remote destinations. In these cases, virtual meetings should be considered to allow for more diverse participation.

Hearing directly from a patient and care partner on their perspective living with a condition can differ quite a bit from the physician perspective, published literature and what they might feel comfortable disclosing on social media, so it's important to

that may exist.

- Incorporating a mechanism of sharing back what the company has learned from the patient and care partner advisors furthers a sense of trust and collaboration, creating an environment of regarding patients as partners in the process of medicine development.

Considering a variety of patient engagement modalities is valuable for all stakeholders involved (e.g. one-day advisory boards, longitudinal advisors serving as ad-hoc members of the study team over a period of time, sharing back with the patients and care partners what the company has learned from them directly and how that feedback is being used to enhance research decisions, etc.).

gather patient perspectives more directly in a psychologically safe manner to help advance research decisions.



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About Takeda

Takeda is a patient-focused, values-based, R&D-driven global biopharmaceutical company committed to bringing Better Health and a Brighter Future to people worldwide. Our passion and pursuit of potentially life-changing treatments for patients are deeply rooted in our distinguished history in Japan since 1781.

We have presence in approximately 80 countries, with leading positions in Japan and the U.S., respectively the third and first largest pharmaceutical markets in the world. We will deliver highly innovative medicines and transformative care for more people globally.

About the R&D Patient Engagement Office

What the R&D Patient Engagement Office is focused on is the opportunity to bring the patient perspective to research and development teams at Takeda. Instead of developing drugs *for* patients, we develop drugs *with* patients. The way we do this is through a direct two-way dialogue between the patients and our researchers. What we learn helps steer the research teams towards developing medicines that really matter to patients – things like making it easier for them to participate in clinical trials, addressing symptoms they truly care about, and meeting needs that we didn't even know existed. By partnering with patients, we can create medicines that really matter.

About the team

Tricia Haut

Nan Doyle

Liz Cunniff

Jessica Scott

Janet Peterson

Vivian Larsen



Annex 1

Supplementary Information R&D Key Performance Indicators and Patient Engagement Planning

Cross functional members of the team participate in PEP

Recommended GPT Functions

Global Program Lead _____
Global Program Manager _____
Global Clinical Lead _____
Product Strategy Lead _____
Market Insights _____
Clinical Operations _____
Global Outcomes Research _____
Payer Value & Patient Access _____
Clinical Translational Sciences _____
Patient Safety _____
Global Medical Affairs _____
Global Health Economics _____
Regulatory Affairs _____
Pharmaceutical Sciences _____
ClinPharmacology/transl. _____
Patient Services _____
Team specific _____

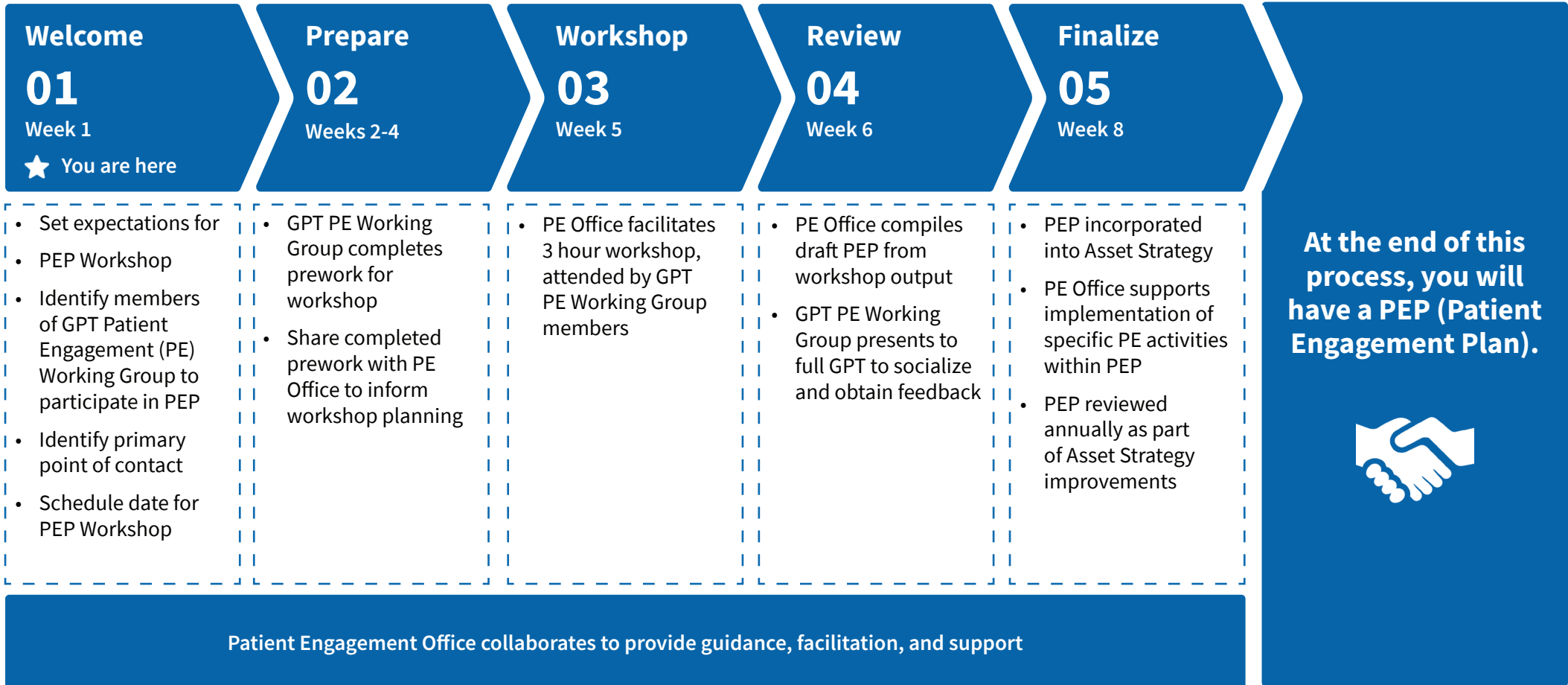
P3 Matrix Team Functions

Patient Engagement _____
Patient Advocacy _____
Patient Recruitment & Retention _____

Patient Engagement Planning

Process

OBJECTIVES



Patient Engagement Planning

Sample PEP Workshop Agenda

Patient Engagement Planning Workshop

Time	Agenda
9:00 AM	Welcome, review workshop goals and agenda Brief opening remarks from GPL and GI Patient Engagement Lead.
9:10 AM	P3: Patient Engagement, Patient Advocacy, and Patient Recruitment & Retention Introduction to the P3 functions and their role in supporting GPTs.
9:20 AM	Introductions and icebreaker Identify key challenges and upcoming priorities.
9:30 AM	Patient community characteristics Understand patient population diversity and existing relationships with community (e.g., patient groups).
9:40 AM	Previously completed or planned activities Team members speak to previously completed or planned activities to engage patient stakeholders.
10:00 AM	Identifying input opportunities Facilitated interactive brainstorm of meaningful outcomes from patient/advocate input interactions.
11:00 AM	Laying the groundwork for implementation Prioritize opportunities for patient, care partner, and advocate interactions. Identify activities needed to support priorities and map to development timeline.
11:50 AM	Meeting summary and next steps
12:00 PM	Workshop ends

Patient Engagement Planning

Sample output #1: Meeting summary

[ASSET]
[INDICATION]
**Patient Engagement Planning Workshop
Meeting Summary**

Workshop Date: February 7, 2020

Workshop Objectives:

- Identify meaningful patient input opportunities across the asset lifecycle
- Map cross-functional needs
- Align opportunities to development timeline
- Agree on next steps

Workshop Attendees:

GPT participants

- Business Ops/Project Management
- Global Medical Lead
- Research Translational Medicine
- US Medical Affairs
- Global Program Manager
- Global Regulatory Lead
- Global Access Brand Lead
- Global Commercial Lead
- Global Outcomes Lead
- Global Program Lead
- Clinical Operations Lead
- US Commercial Lead
- Product Strategy Lead
- Global Clinical Lead
- Global Safety Lead

P3 participants

- R&D Patient Engagement
- Patient Advocacy
- R&D Patient Engagement

Workshop Summary:

Together with the Patient Engagement Office (PEO), the GPT convened team members and cross-functional leads to identify where input from patient community stakeholders (e.g., patients, care partners, providers, advocates) may mitigate asset development challenges and help achieve program milestones. Attendees included members of the GPT, and representatives from GOR, the PEO, and PR&R (Patient Recruitment & Retention).

The moderated workshop was conducted in five parts: During the first session, the team explored "what keeps them up at night" as they think about patients and care partners. During the second session, the team aligned on characteristics of and considerations for the study population, including socioeconomic and geographic diversity; the third session reviewed completed and planned stakeholder engagement activities. The fourth session was a facilitated brainstorm of patient input opportunities to address the informational needs identified during the first session. The fifth and final session saw the team discuss priorities for FY2020 and beyond.

The output from the workshop is detailed in an excel PEP, an accompaniment to this meeting summary. The PEP tracks patient engagement activities from clinical development to launch, the Takeda stakeholders and the critical timepoints aligned with the asset development.

Teams are provided with a detailed summary of workshop discussion which details:

- Workshop objectives,
- Attendees,
- Key priorities and challenges facing the development program,
- Current understanding of the patient and care partner population and relationships with the patient community (e.g., patient organizations),
- Completed patient engagement activities, and
- Proposed patient engagement activities including purpose, priority and timing.

Patient Engagement Planning

Sample output #1: High Level PowerPoint outlines strategic imperatives

Strategic imperatives	01 Understand symptom burden and treatment priorities from patient and caregiver perspectives	02 Demonstrate meaningful impact of treatment on daily life to different stakeholders	03 Optimize clinical trial designs: Input regarding trial burden, options for patient support, increasing information and access to studies	04 Build relationships with patient groups in key geographies
Patient interaction Priority	<ul style="list-style-type: none">• Understand symptom burden and impact on QoL, functioning• Understand treatment priorities and expectations• Identify differences between patients, caregivers, providers	<ul style="list-style-type: none">• Determine appropriate tools (e.g., PRO) to quantify outcomes• Ensure data is meaningful to multiple stakeholders• Demonstrate impact to different stakeholders (patients, caregivers, HCPs, regulators, Payers)	<ul style="list-style-type: none">• Understand patient motivations for trial participation• Identify potential patient pain points related to protocol	<ul style="list-style-type: none">• Understand patient/caregiver populations represented by PAGs• Build foundational relationships with PAGs
Output ("To Inform What")	<ul style="list-style-type: none">• Provide foundational understanding• Define value of new product• Inform development of tools to quantify outcomes• Inform trial retention plan	<ul style="list-style-type: none">• Inform data to present at end of Ph2, conversations with regulatory agencies, payers, HCPs• Create urgency payers• PRO-based label claims• Support HTA, EMA discussions	<ul style="list-style-type: none">• Inform trial recruitment plans (Ph3)• Inform trial retention plans (Ph3)	<ul style="list-style-type: none">• Increase trial and disease awareness• Collaborate to fill unmet needs (e.g., patient education)

Patient Engagement Planning

PEP is a living document, that develops throughout asset lifecycle

Just as with the Asset Strategy, it is important to track PEP progress, as well as revise the plan as additional data is collected. The PE Office will provide reminders to GPTs to revisit their PEPs, but ultimate responsibility lies with the GPT.



Annex 2

Supplementary Information PEPAC

Patient Engagement Patient Advocacy Council (“PEPAC Council”)



Company recognizes that patients possess a deep understanding of their disease, its symptoms and treatments and can offer a relevant and often nuanced perspective to the drug discovery and development process. In line with the company’s commitment to ensuring therapies are developed with patient needs and priorities as the focal point, the Head of the R&D Patient Engagement Office organized a small cadre of patients to provide ongoing advice and counsel to ensure R&D is informed by the patient perspective throughout the process.

Form standing body to advise R&D on patient perspectives on drug development representing multiple disease areas, regions, backgrounds and experiences.

KEY OUTCOME:

Built an efficient and consistent process for bringing patient perspectives into R&D through a standing body that has engaged with Company **R&D on topic specific sessions over eight months insights have been shared with internal teams to create real change informed by the patient perspective.**

INSIGHTS:

- Patients offer important and useful perspectives that require a two way dialogue between R&D teams and the advisors to elucidate.
- R&D teams appreciate the opportunity to obtain the patient perspective on questions in alignment with program timelines and key decision points.
- R&D teams are often surprised by the learnings gathered during the PEPAC engagement and find value well beyond the initial question asked.

Project Overview

PEPAC Council Aims to Engage a Diverse Group of Patients and Care Partners in a Longitudinal Manner to Inform R&D

GOALS

Support processes that allow for the integration of patients' and care partners' points of view into discovery and development as appropriate

Identify best practices and novel approaches for integrating patients' and care partners' perspectives into R&D

Foster long term relationships with patients and care partners to allow or continued learning and growth

OPPORTUNITIES FOR ENGAGEMENT

Live discussions informed by pre work

Online collaboration sessions

Survey

Forum discussion

Document annotation

Project Overview

PEPAC Council Composition | By the Numbers

ROLE

8

Patients

- patient opinion leaders (1)
- advocates (4)
- patients (3)

2

Care partners

DIAGNOSIS

5

Rare disease

2

Cancer

2

GI

1

Cancer & GI

1

CNS

GEOGRAPHY

6

United States

- rural
- urban
- suburban

2

United Kingdom

1

Japan

1

Spain

ETHNICITY

5

United Kingdom

1

African American

1

African American/ Hispanic

1

East Asian

1

Hispanic

1

Spanish

GENDER

6

Females

4

Males

Project Overview

PEPAC Council Composition | By the Numbers

AFFILIATION

7

Patient advocacy affiliations

- MÁS QUE IDEAS Foundation
- Davis Phinney Foundation Parkinson's Ambassador
- The SA Moves Foundation
- The Ehlers Danlos Society
- Advocacy Services for Rare and Intractable Disease Stakeholders (ASrid)
- International Gaucher Alliance
- Fabry Support & Information Group

HEALTH LITERACY

7

Proficient

2

Moderate

1

Basic

EDUCATION

1

High School Degree

5

Undergraduate Degrees

4

Graduate Degrees

Project Overview

PEPAC Council Composition

Stakeholder type	First Name	Gender	Highest level of education	Patient advocacy affiliation	Geographic	Race/ ethnicity	Therapeutic area	Indication	Health literacy
Patient opinion leader		M	Graduate		US, urban	Caucasian	CNS	Parkinson's	Proficient
Patient & advocate		F	Graduate	N/A	US, urban	Hispanic	GI	Celiac disease	Basic
Patient & advocate		M	Undergraduate		Spain, urban	Spanish	Cancer	Hodgkin's Lymphoma	Proficient
Patient		F	Undergraduate, in-progress	N/A	US, suburban	Hispanic,Black	Rare disease	Narcolepsy	Moderate
Patient & advocate		M	Undergraduate		US, rural	Caucasian	Rare disease	Fabry disease	Proficient
Patient		M	Undergraduate		US, rural (originally UK)	Caucasian	Cancer/GI	Pancreatic cancer, celiac disease	Proficient
Patient & advocate		F	Undergraduate		UK, urban	Caucasian	Rare disease	Ehlers Danlos syndrome	Proficient
Care partner & advocate		F	Undergraduate		UK, suburban	Caucasian	Rare disease	Gaucher disease	Proficient
Patient		F	Graduate		US, urban	African American	GI	Celiac disease	Moderate
Care partner & advocate		F	Unknown		Japan, urban	East Asian	Rare disease	N/A	Proficient

Project Overview

PEPAC Council Engagement Sessions

Session month	Session purpose
November 2019	Clinical Trial Burden Project to offer the team a deeper understanding of your perspective on recommendations to minimize the burden of tests on patients during clinical trial participation.
January 2020	R&D Patient Engagement Update Video Review to offer the team your thoughts and feedback on how the concept of and vision for patient engagement is communicated to internal colleagues and external stakeholders.
February 2020	Informed Consent and Assent Document Review to offer the team your feedback on how well the informed consent and assent documents communicate key information to potential participants as well as any gaps or areas of improvement needed.
April 2020	Trials Document and Website Review to gain your insights on how the strengths and weakness of the trial websites with a focus on usability, navigation, and visuals.
Planning phase May 2020 <small>*PEPAC has continued throughout the remainder of 2020</small>	COVID 19 Pandemic discussion to hear perspectives of those living post virus and also other conditions.

Initial Project Concept

PEPAC Council Proposed Vision and Goals

VISION

Patient informed research and development leads to the discovery, development, and commercialization of treatments that provide patients with optimal care and outcomes

GOALS

- Support processes within Company that allow for the integration of patients' and care partners' points of view into discovery and development as appropriate
- Identify best practices and novel approaches for integrating patients' and care partners' perspectives into R&D
- Foster long term relationships with patients and care partners to allow for continued learning and growth

Initial Project Concept

PEPAC Council Proposed Criteria for Membership

Expert on patient perspective (care-partner, patient or survivor)

Represent disease area aligned with R&D priorities

Willing to share and articulate aspects of patient experience

Have availability of time and be in good enough health to meet time minimum time commitment

Initial Project Concept

PEPAC Key Roles and Responsibilities

SPONSOR

- Provide high level direction and guidance
- Inform agenda development and communicate topics appropriate for PEPAC consideration
- Coordinate participation from colleagues in other functional areas as needed
- Communicate key learnings to relevant stakeholders

ADVISORS

- Prepare for and attend meetings and/or calls
- Provide input on agenda topics
- Actively participate in meetings, bringing unique expertise
- Complete follow up activities as needed and agreed upon

3RD PARTY SPECIALIZED CONSULTING

- Create meeting schedules
- Develop meeting goals, agenda, and presentations
- Ensure appropriate meeting acilitation
- Prepare and distribute a summary of each meeting and/or call
- Gather feedback from members to ensure needs are being met
- Catalog and store working group communications and materials in a compliant fashion
- Manage ongoing engagement and collaboration through online platform

Session month	Session purpose	Outcomes/Impact
November 2019	<p>Reducing the Burden of Clinical Trials -objective to offer the team a deeper understanding of your perspective on recommendations to minimize the burden of tests on patients during clinical trial participation.</p>	<p>PEPAC insights impacted the direction of this internal initiative</p>
January 2020	<p>R&D Patient Engagement Update Video Review – objective to offer the team your thoughts and feedback on how the concept of and vision for patient engagement is communicated to internal colleagues and external stakeholders.</p>	<p>PEPAC insights to the episode video producer to change the approach to future internal videos</p>
February 2020	<p>Informed Consent and Assent Document Review – objective to offer the global program team your feedback on how well the informed consent and assent documents communicate key information to potential participants as well as any gaps or areas of improvement needed.</p>	<p>Study team is taking the PEPAC insights into account while working to finalize the ICF for the optional 10-year follow-up study</p>
April 2020	<p>Company Trials Document and Website Review- objective to gain your insights on how the strengths and weakness of the Company Trials Website with a focus on usability, navigation, and visuals.</p>	<p>The website team reports that insights have been used to directionally steer the look and functionality of the new site.</p>



PATIENT FOCUSED
MEDICINES DEVELOPMENT



Reducing and mitigating the burden of clinical trials on patients and care partners

Organization: Takeda

The PFMD Book of Good Practices

3rd edition | 2020





Reducing and mitigating the burden of clinical trials on patients and care partners

Organization:
Takeda



Basic Information

Project Description

Companies ask patients to provide tissue and blood samples and undergo imaging studies during the course of clinical trials. We recognize the significant burden these requests place on patients when they are already burdened by their disease. Takeda desired to better understand the patient perspective on the burden of clinical trial testing and sampling and to elevate these insights internally to teams working on trial protocols.

To better understand the burden of testing and sampling in clinical trials, a series of qualitative interviews were held with patients and care partners across therapeutic areas (Oncology, Gastroenterology (GI), Neuroscience(CNS), and Rare Diseases) with sponsor-agnostic trial experience, followed by an advisory board meeting.

While the advisory board was designed to gain a deeper understanding of the burden patients and care partners face during trial participation due to sampling and testing, the company gained additional insights related to the overall burden trial participation may pose, and patient and care partner recommendations on how trials can be conducted with less burden on participants.

Following the advisory board, the company shared learnings broadly within the R&D organization and developed a set of recommendations for the Takeda's consideration to consistently reduce burden across its trials.

Specific project activities include:

- Interviews to prepare for a patient and care partner advisory board,
- An internal working group to develop recommendations to reduce trial burden,

- Validation of the internal recommendations by the R&D Patient Engagement Patient Advisory Council (PEPAC) a longitudinal cross therapy area advisory council of 10 patients/care partners,
- Socialization of recommendations with more than two dozen members of R&D leadership, and
- Elevating learnings internally during a Town Hall-style event with a patient panel.

The project is currently ongoing with the company aligning on how best to implement recommendations within its R&D processes.

An overview of the sequence of project activities is found in supplementary [Annex Supplementary Information](#).

Start Date:

03/2019

End Date:

Ongoing

Geographic focus

Global

Continental/Regional

Europe

Asia

Oceania

National

North America

Africa

South America

Antarctica

Purpose of the initiative



Involving patients in the lifecycle of medicine development



Providing guidance to others in their PE efforts

Initiative Focus Area

Research

Education

Organization/
System development

Access

Advocacy

Policy

Care Delivery

Gap analysis

Which phase(s) in the patient care journey does the initiative align with?

Prevention

Onset of symptoms

Newly Diagnosed

Treatment

Clinical Trial

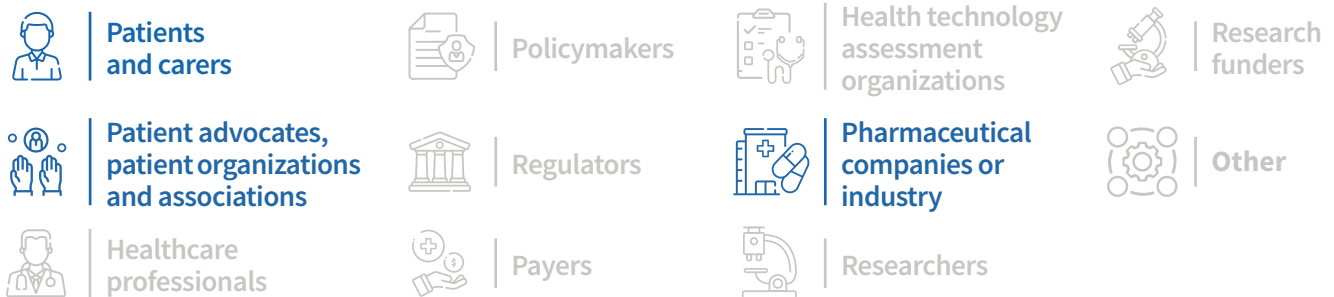
Long Term Management

Other

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



Which stakeholders does this PE project involve?



The quality of patient engagement

[Link: Descriptions of the PE Quality Criteria](#)

1. Shared purpose



Initial patient and care partner advisory board

The shared purpose for the advisory board meetings was to:

- Better understand how people participating in clinical trials view tests and procedures
- Understand thought processes in terms of deciding to participate in a trial
- Identify key considerations before finalizing a trial protocol to reduce burden

Objectives were broadly set by internal stakeholders after reviewing the learnings from patient and care partner interviews on the topic and were shared with patients at several points during planning and execution:

- During introductory phone calls with the Takeda's patient engagement vendor partner;
- Within the advisory board invitation;
- Within a "welcome letter" provided to participants during hotel check-in;
- During a welcome reception prior to the advisory board which served as an opportunity for all stakeholders to meet in a less formal environment and to share what they hoped to get out of the engagement; and
- At the start of the advisory board meeting.

Patient engagement activities at Takeda have the foundational objective for internal stakeholders to come away with impactful insights, and for external participants to feel heard and valued. This expectation is set at the beginning of each advisory board. At the end of the meeting, internal stakeholders are asked to share "a-ha" moments and external participants share key important takeaway from their perspective. In meeting evaluation forms in this (and each of Takeda's patient engagement activities), participants are asked whether they felt their voices were heard during the meeting with 100% of participants stating that they felt they were. In closing remarks, external participants encouraged company to continue to learn from patients and to find ways to make trials more patient-friendly. Inspired by this message, company convened an internal working group to develop a set of recommendations to reduce trial burden.

Next step was the R&D Patient Engagement Patient Advisory Council input

The R&D Patient Engagement Patient Advisory Council (PEPAC) members – a longitudinal group of advisors comprised of patients, care partners, and advocates – reviewed a set of internal recommendations created from advisory board learnings, validating the recommendations and providing guidance on which recommendations would be most impactful to patients and families if the company were to enact them consistently across all trials.

Prior to each convening of PEPAC, objectives for the engagement are established. Please refer to [Annex Supplementary Information](#) for additional information on PEPAC's involvement in the project.

PEPAC is a longitudinal group of advisors who regularly engage with the company to provide guidance.

After internal working group further refinement and socialization with leaders across R&D, a Town Hall-style event was held

In November, three patients and care partners who participated in the original advisory board returned to the company but this time in an auditorium setting to share their perspectives on the burden of clinical trial participation and opportunities to improve patient and care partner trial experiences. The objectives of this engagement were for company attendees to leave the event with:

- A deepened appreciation of what patients undergo to be a part of our trials
- An understanding of the actions the company can take to minimize challenges trial participants face due to testing and sampling procedures
- Inspiration to help embed a consistent approach toward decreasing the burden of clinical trials for study participants

Shared purpose was re-established with the external stakeholders during several calls and in-person meetings to prepare for the event. Objectives were documented in the event invitation and agenda and patient advisors were able to express their perspectives on the topics in order that the issues of importance to them could be heard by the wide audience of over 200 company employees.

2. Respect and accessibility



Initial patient and care partner advisory board and Town Hall-style event

Patients participating in the advisory board and Town Hall event were living with a variety of conditions including Alzheimer's, narcolepsy, celiac disease, gastroparesis, Fabry, multiple

myeloma, and non-small cell lung cancer. As such, it was important that meeting logistics considered their unique needs. The vendor partner worked closely with each participant to meet advisor needs related to travel, accommodation, meeting room accessibility, and dietary preferences.

Accommodations made to increase accessibility included:

- Tailoring travel schedules to needs related to health conditions,
- Arranging for virtual participation for a care partner of young children,
- Accommodating dietary, and
- Adapting interactive exercises to support those with cognitive challenges.

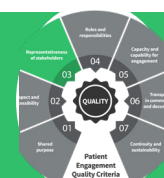
During the advisory board and welcome reception, separate meal options, free of gluten contamination, were provided for the participant living with celiac disease. Following the meeting, this patient suggested that for future engagements, all participants be provided with the same meal options – including gluten-free options – so as to not single out those living with celiac. This feedback was incorporated into planning for the Town Hall meeting.

In addition to working with participants on their logistical needs prior to participation, the vendor partner also shared the schedule of events, including travel and accommodation information. All participants were provided with the direct contact information (e.g., cell phone) of the vendor partner who was onsite at the meeting to help trouble shoot any challenges that arose.

All participants were paid honoraria for their time and incidental costs were covered. The vendor partner booked all air fare, accommodations, and car service to reduce up-front out-of-pocket expenses for participants.

The R&D Patient Engagement Office and vendor partner worked with internal stakeholders to ensure presentations during the meeting were written in appropriate, easy to understand language. Preparation calls were held with internal stakeholders to provide guidance on how to respectfully engage external stakeholders including use of language and terminology and listening to and respecting diverse viewpoints. The welcome dinner prior to the advisory board helped to foster a psychological safety and connection between company global program team members, company patient engagement team members and patient/ care partner advisors.

3. Representativeness of stakeholders



Internal stakeholders involved in the project have represented a myriad of positions, therapeutic areas, and functional focus. A snapshot of internal stakeholder representativeness can be found on [Annex Supplementary Information](#).

Initial patient and care partner advisory board

External participants represented patients and care partners with clinical trial experience in therapeutic areas of interest to company including neuroscience (Alzheimer's, narcolepsy), gastrointestinal (celiac disease, gastroparesis), rare disease (Fabry, MLD), and oncology (multiple myeloma, non-small cell lung cancer). Participants were identified through outreach facilitated by patient advocacy group partners and community leaders with reach into these disease communities.

As a company best practice, all patient engagement activities are evaluated for their diversity, equity, and inclusion. Patients and care partners participating in the advisory board came from eight different states; live in rural, suburban, and urban areas; have varying degrees of education from high school degrees to Master's degrees; and range in age from early 30's to 70's. On gender and race/ethnicity, the engagement skewed female and only included Caucasian participants. This raises the need to find additional avenues to identify and support these patients in participating in patient engagement activities. Initial interviews with patients, care partners, and advocates completed prior to the advisory board were able to capture additional male and non-Caucasian perspectives.

R&D Patient Engagement Patient Advisory Council input

PEPAC is a diverse group of patients, patient advocates, and care partners of different ages, genders, ethnicities, socioeconomic status, educational and health literacy levels, and professional experience.

PEPAC members live in the EU, Japan, and US, and have lived experience with gastroenterology, neuroscience, oncology, and rare conditions. The diversity and representativeness of perspectives represented on PEPAC allows for robust discussions where company benefits from the full experience of patients and care partners.

4. Roles and responsibilities



The R&D Patient Engagement Office involved a diverse group of internal stakeholders, with a variety of roles and responsibilities.

- The “core team”, which included the R&D Patient Engagement Office and representative of the project sponsor, were responsible for overall project management and resourcing, content creation, and coordinating stakeholder input. The core team had regular weekly meetings to align and ensure forward progress.
- Additional meetings were held on a milestone-based frequency with internal stakeholders providing input into advisory board focus and content. These stakeholders were identified by the project sponsor to participate. Following the advisory board, internal stakeholders – including those who attended the advisory board – convened to distilling learnings from patients into recommendations that the company could implement to reduce trial burden while maintaining high scientific rigor and accommodating innovation.
- R&D leadership were consulted and informed throughout the project by the core team and provided guidance on strategic direction.
- The Town Hall share back was open to all R&D employees and was livestreamed and recorded to provide access to those unable to attend the event. A tracker was developed to ensure the core team was appropriately engaging internal stakeholders throughout the project.

Refer to [Annex Supplementary Information](#) for a high-level overview of roles and responsibilities.

Initial patient and care partner advisory board

The core team worked closely with internal stakeholders attending the advisory board to develop the advisory board agenda, discussion guide, and presentation materials to ensure alignment and to gain their medical and scientific input. Prior to the advisory board meeting, a preparation meeting was held with internal attendees to walk through the flow of the advisory board and ensure all internal stakeholders were clear on their role in the meeting. The vendor partner facilitated the advisory board meeting, ensuring that all external participants had the opportunity to share their perspectives, and that the internal stakeholders had the opportunity to ask questions.

Patients and care partners advisory board participants served as advisors during the meeting, sharing their experience with clinical trials, and offering recommendations for how clinical trials can be improved and made more patient-friendly. Additionally, they shared their experiences on video, allowing their perspectives to be shared widely within the R&D organization. From the outset of the project, the R&D Patient Engagement Office engaged a third-party vendor partner to provide a consistent point of contact with all stakeholders and to manage the project. Alignment around external advisor roles and responsibilities was accomplished through:

- Correspondence with the patient engagement vendor partner,
- Written contracts, with the vendor supporting participants in answering questions about the contract,
- A welcome reception prior to the advisory board, and
- Reiterating roles at the outset of the meeting.

R&D Patient Engagement Patient Advisory Council input

As a longitudinal group of advisors, PEPAC's roles and responsibilities set in place during the establishment of the group during screening calls, through contract negotiations, and during the group's initial launch call. Following this launch call, PEPAC members provided anonymized feedback to obtain final agreement for roles and responsibilities and ways of working together in a longitudinal manner.

Town Hall-style event

During the Town Hall, patient and care partner panelists spoke directly to company employees, elevating the importance of making trials more patient-friendly. After the event, a lunch was held, allowing the opportunity for internal stakeholders to engage with the panelists in two-way dialogue. Alignment on roles and responsibilities was accomplished through:

- Correspondence with the patient engagement vendor partner,
- Written contracts,
- Preparatory calls with the core team, and
- Pre-event dinner and breakfast with the core team.

See [Annex Supplementary Information](#) for an overview of the topics addressed during the Town Hall.

5. Capacity and capability for engagement



By identifying patient participants by first holding qualitative interviews, the company was able to ensure that all participants had a level of comfort with sharing their personal experiences. The single-point of contact provided through the vendor partner ensured participants knew where to go with any questions or concerns.

Initial patient and care partner advisory board

During the advisory board meeting, facilitators paused to ensure all stakeholders had the opportunity to ask questions about terms or subjects before diving into discussion. To level set and create common understanding, for both meetings, a portion of the agenda up front was spent defining clinical trials and explaining why testing and sampling, beyond the standard of care, are performed.

R&D Patient Engagement Patient Advisory Council input

Since the aim of the PEPAC engagement is to gain the patient and care partner perspective on company materials, programs and resources, the R&D Patient Engagement Office spends considerable time and effort ensuring that internal colleagues are trained and prepared in best practices of communicating with advisors. Materials and questions are created, reviewed and approved to ensure that they include accessible and appropriate language and terms for the advisors as well as making sure there is a small team with the capacity to address any questions or concerns in a timely manner.

Town Hall-style event

Several preparatory meetings were held with the patient and care partner panelists to review the agenda and co-create prompts and questions used during the discussion. Two of the participants had previous public speaking experience. An IT meeting was held before the event to preview the venue set up and ensure comfort with the use of microphones.

6. Transparency in communication and documentation



The use of an external vendor provided external participants with a single point of contact for pre-meeting communications. These communications included:

- A preparatory phone call,
- Formal meeting invitation,
- Phone calls and emails to arrange logistical details,
- Pre-meeting logistics note, and
- Welcome letter on arrival at the hotel.

Regular project check-ins were established among internal stakeholders using meeting agendas and documenting action items and next steps.

Initial Patient and care partner advisory board

During the advisory board meeting, all participants were provided with a briefing book which included:

- Logistical information
- Agenda and flow of the day
- Venue maps
- Short bios of participants

The vendor facilitated the advisory board using a discussion guide, presentation to help ground discussion on the agenda topic, flip-charting to allow participants to refer back to points made, and interactive activities with visual outputs such as charting the level of emotional, financial, and practical burden trial participants face as a trial progresses. Feedback from external stakeholders was collected via an evaluation form at the close of the meeting.

A comprehensive report was developed after the advisory board reviewed by internal stakeholders. Additionally, video clips of patients and care partners sharing their stories filmed as short interviews during advisory board breaks were consolidated into a seven-minute video, allowing company colleagues who did not participate in the meeting to benefit from hearing from patients and care partners.

R&D Patient Engagement Patient Advisory Council input

PEPAC members are consulted approximately every six to eight weeks. In advance of each engagement the members receive the documents for review as well as the background and context information; members are offered approximately one to two weeks' time (depending upon the complexity of the session) to comment on materials and questions offered in the virtual on-line tool as well as to consider and respond to others comments; soon after the close of the online session a live call is hosted to review the session feedback summary and gain deeper insights on the patient and care partner perspectives. Following the live call, the company compiles a full summary including additional insights and points of agreement from the live call.

Town Hall-style event

The Town Hall event was livestreamed and recording of the event was archived. Following the event, and in collaboration with R&D Communications, a short summary was shared internally with link to the recording, to further disseminate and elevate the panelists' perspectives. One functional head has made viewing this recording "mandatory" for their team.

Internal socialization of the trial burden recommendations

Several presentations were created to support the core team in consulting and informing internal stakeholders throughout the project.

7. Continuity and sustainability



After the Advisory Board meetings, evaluation forms were used to gather advisors' feedback. In this feedback, advisors urged the company to continue to engage on the subject of trial burden. Patient and care partner perspectives from the advisory board were taped and shared, allowing messages to reach a larger audience.

Inspired by the learnings from the advisory board meeting, a cross-functional cross-TA group of R&D employees developed a set of recommendations for internal consideration aimed at reducing burden, while maintaining high scientific rigor and accommodating innovation. Now a part of the Takeda's Trial Burden Initiative, these recommendations have been shared with leadership for endorsement and were shared broadly with the R&D organization during the Town Hall event on November 19, 2019.

Leadership has provided their support of the project and has tasked the core team with developing a recommended framework and tools to ensure the recommendations are consistently considered during study development.

Results, outcomes and impact

Describe the outcomes and impact of this initiative and provide concrete examples

People participating in clinical trials, across therapeutic areas, face increased practical, financial, and emotional burden due to the need to undergo tests and sampling procedures beyond the standard of care.

The insights and feedback provided by patients and care partners during the advisory board made it clear that there is much to be done to reduce the burden patients and care partners face during trial participation –including, but not limited to the burden of testing and sampling

The impact of these insights has led to the development of a series of recommendations to reduce the burden faced by participants in company trials. This work has evolved from an advisory board to a broader effort within the company, the Trial Burden Initiative.

Positive impact for specific medicines development phases

The internal working group distilled learnings from the Ad Board into three main buckets of recommendation for the internal teams during clinical research and operations. Recommendations are focused on: (1) reducing the burden of sampling and biomarker plans during protocol development, (2) improving trial participant access to information, and (3) improving the overall patient experience when participating in a trial.

Direct or indirect positive impact for patients

- Patients and care partners were heard and their input has an impact in improving the future trials in a way that could potentially reduce the burden of trial participation.
 - Internal teams understand and appreciate what patients undergo to be part of the trials, so that they can act upon these challenges faced by trial participants when designing future trials.
 - Implementation project will improve future trials at the company and educate internal teams to be more considerate of patients' trial experience.
-

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

By engaging with patients and care partners, study teams learn from patients in a way that helps to design more patient-friendly studies in the future. The engagement makes each protocol not a generic protocol but one that is going to affect individual people and their families. In the words of one internal stakeholder,

“putting a face to it makes us work harder, faster, and better.” Some of the learnings internal stakeholders walked away with were:

- “As a scientist, we want to ask a lot of questions. But it’s a balance between people’s experience and getting answers to our questions.”
- “I was very impressed with the level of enthusiasm and engagement that our patient advisors showed. I learned much from them. This meeting will help me keep our study participants in the forefront of our considerations as we develop our clinical trials.”
- “I had such a great experience meeting and hearing the stories of these patients. Having them all day and being able to ask specific questions was so helpful. Thank you so much for planning and facilitating this meeting. So much food for thought.”

See supplementary [Annex Supplementary Information](#) to see feedback on the Town Hall-style event from participating employees.

Lessons learned

- By sharing learnings from the advisory board through a short video featuring patient and care partner advisors, and in bringing the learnings to a Town Hall with patient panel, the impact of the advisory board meeting was amplified.
- Engaging a diverse group of internal and external stakeholders gave additional credibility to the project, allowing it to rise above the study-team level to a project that has gained support from leadership across R&D.
- We will be working to imbed the Recommendations in a collaborative manner across R&D so that we can build on what we are already doing that aligns well with what patients need to decrease reduce the burden of clinical trials.



 @Takeda

 Takeda.com

About Takeda

Takeda is a patient-focused, values-based, R&D-driven global biopharmaceutical company committed to bringing Better Health and a Brighter Future to people worldwide. Our passion and pursuit of potentially life-changing treatments for patients are deeply rooted in our distinguished history in Japan since 1781.

We have presence in approximately 80 countries, with leading positions in Japan and the U.S., respectively the third and first largest pharmaceutical markets in the world. We will deliver highly innovative medicines and transformative care for more people globally.

About the R&D Patient Engagement Office

What the R&D Patient Engagement Office is focused on is the opportunity to bring the patient perspective to research and development teams at Takeda. Instead of developing drugs *for* patients, we develop drugs *with* patients. The way we do this is through a direct two-way dialogue between the patients and our researchers. What we learn helps steer the research teams towards developing medicines that really matter to patients – things like making it easier for them to participate in clinical trials, addressing symptoms they truly care about, and meeting needs that we didn't even know existed. By partnering with patients, we can create medicines that really matter.

About the team

Tricia Haut

Nan Doyle

Vivian Larsen

Jessica Scott

Janet Peterson

Liz Cunniff

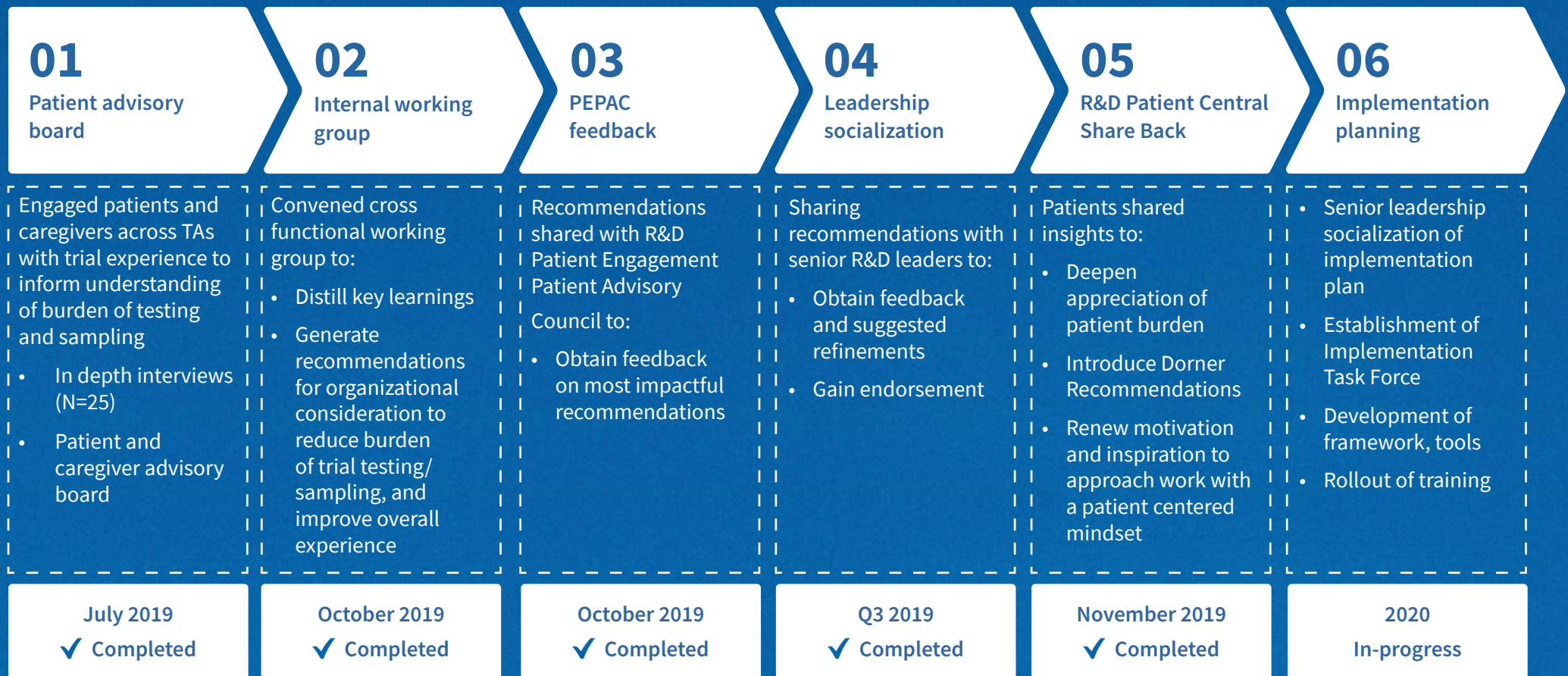


Annex 1 - Supplementary information

Project Milestones



Project initiated March 2019



R&D Patient Engagement Office desired the opportunity to further understand the care partner, patient and patient advocate perspective on recommendations

Nine of the ten PEPAC members completed the survey evaluating the extent to which each recommended action would lessen the burden placed on clinical trial participants

Due to the high ratings across the recommendations, the team engaged PEPAC to rank and prioritize the recommendations as well as seek clarification on lower rated recommendations

Project Relies on Cross Functional & Cross TA Collaboration

Patient advisors

Patients engaged to provide insight into patient burden and opportunities to improve patient experience

Patients and **caregivers** with clinical trial experience representing disease areas of interest to Takeda engaged during in depth interviews and patient advisory board:

- **Gastroenterology:** Celiac disease, gastroparesis
- **Rare disease:** Fabry, metachromatic leukodystrophy (caregiver)
- **Oncology:** Multiple myeloma, non small cell lung cancer
- **Neuroscience:** Narcolepsy, Alzheimer's (patient and caregiver)

Additional feedback from Takeda's **R&D Patient Engagement Patient Advisory Council (PEPAC)**, a diverse group of patients, caregivers and advocates, engaged by Takeda to share perspectives on topics of interest to their communities and the company

- **Gastroenterology:** Celiac disease
- **Rare disease:** Gaucher, Fabry, Ehlers Danlos syndrome
- **Oncology:** Prostate cancer, pancreatic cancer
- **Neuroscience:** Parkinson's, narcolepsy

Internal working group

Working group participated in advisory board planning and tasked with distilling learnings from patients into recommendations that can be implemented while maintaining high scientific rigor and accommodating innovation

Core team:

- VP and Head, Clinical Biomarker Innovation and Development (sponsor)
- Senior Scientific Director, QTS, Imaging
- R&D Patient Engagement

Crossfunctional, cross TA working group members:

- Early Clinical Operations, Oncology
- Associate Director, Oncology
- Clinical Scientist
- Translational Sciences
- Clinical Scientist
- Executive Medical Director
- Head, Patient Recruitment & Retention
- Head, Bioethics and Science Philanthropy
- Scientific Director
- Quantitative Clinical Pharmacology
- Translational Head

Senior leadership

Ask of leadership: Provide direction and support for recommendations from an R&D leadership perspective

R&D Leadership

- Chief Medical Officer
- VP, GPSA
- Global Head, Quantitative Translational Sciences
- President, R&D
- Global Head, Clinical Operations

Oncology

- Head, Oncology
- Head, Oncology Clinical Science
- Translational Head, Oncology

Neuroscience

- Head, NS
- Head, NS Drug Discovery
- Translational Head, NS
- Head, Clinical Science, NS
- Senior Director, Clinical Operations, NS

Gastrointestinal

- Head, GI
- Head, GI Drug Discovery
- Translational Head, GI
- Head, Clinical Science, GI
- Senior Director, Clinical Operations, GI

Rare disease

- Head, Rare Disease
- Head, Rare Disease Drug Discovery
- Translational Head, Rare Disease
- Head, Clinical Science, Rare Genetic & Neonatology
- Head, Clinical Science, Rare Hematology
- Senior Director, Clinical Operations, Rare

Agenda

Time	Agenda
10:00 - 10:55	<p>Welcome</p> <p>Engaging Patients and Care Partners</p> <p>Video - Burden of Clinical Trial Participation</p> <p>Moderated Panel Discussion with Patient Advisors</p>
10:55 - 11:30	<p>Next Steps for Decreasing the Burden of Clinical Trial Participation</p> <p>Audience Q&A with Panelists</p> <p>Closing Remarks</p>

R&D Town Hall Style Event

Attending R&D Employees Noted Messages of Thanks for Advisors

Thank you for sharing your time and perspectives!

It's such a privilege to hear your voices, and see your faces. The requirements of our science put barriers between us, particularly on specific studies we are working on. And yet at the end of the day, its you we work for and you we see if only in our portraits of numbers.

GREAT panel. Very illuminating. I will try to put your thoughts into action, THANK YOU!!

Thank you for giving more than you have already. To help us figure out how to get better at this. We obviously have a ways to go.

We hear you. We are listening.

Fantastic to gain realization of the true burden we put on you. We'll do better.

Thank you! Your words and actions will (and already have) make a huge impact on how we write, run, execute our trials. Focusing on you! Our patients.

You have touched me thank you for your openness and honesty to support how we engage with patients in the future.

Thank you for sharing your experience. We hear you!

You pave the way to make the lives of future patients better.

You are the center of our universe and we know that you should know it too.

The astronaut analogy instantly changed the way I think about study participants! Thank you!

Keep sharing your needs so we can make trials easier and better for patients

Thank you so much!!

Annex 2: 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

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 I-III)

✓ Health technology assessment

✓ Regulatory review and approval or registration phase (including submitting for market authorisation request and approval)

✓ Post-launch activities

- clinical study phase IV
- drug safety monitoring and pharmacovigilance
- pricing and reimbursement
- real-world evidence generation
- adherence
- patient education
- patient and carer support programs
- 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 organizations and associations

✓ Healthcare professionals (including clinical investigators, general practitioners, specialists, pharmacists and nurses)

✓ Policymakers

✓ Regulators

✓ Payers

✓ Health technology assessment organizations

✓ Pharmaceutical companies or industry (including medical devices and biotech companies)

✓ Researchers (academic researchers and investigators)

✓ Research funders

✓ Other (for example, contract research organizations (CRO) and hospitals)