PATIENT EXPERT CENTER

# Patient Expert Engagement with Industry

Like in all industries, listening to the voice of the customer and the end-user is critical also for pharmaceutical companies to develop products and services that match the right expectations. This implies that from the initial phases of research, these expectations are taken into account and measured as the products progress through their life cycle. It is clear that patients are not experts in pharmaceutical science, but they know probably more about what it means to live with their disease than the pharmaceutical expert. Patient organisations combine the knowledge of all their members and are represented by trained Patient Experts who can talk on behalf of their patient community. Even if the systematic and early engagement with patients may make the process more complex, at the same time it also ensures an increase in effectiveness and value generation.

The question often asked is how these patient experts can engage with industry to create meaningful collaborations. This paper tries to give an overview of the different types of patient engagement throughout the product life cycle.

This overview was developed with patient organisations who are member of the Patient Expert Center, together with representatives from five pharmaceutical companies active in Belgium. The overview was developed early 2022.

The list of patient engagement topics should serve as a general guideline of possibilities, rather than interactions that are cast in concrete. The list of engagements is not limited to the services identified below, and the services should also be adapted to the specific need of the moment.

We identified eight major categories for interaction:

1. Clinical trials
2. Disease Awareness Campaigns
3. Patient Education & Literacy
4. Real-World Data and Real-World Evidence
5. Market Access
6. Issues Management
7. Policy
8. Company culture

Within these major categories we try to identify the “Areas of Engagement”. As much as possible, we also specify the “Nature of the Engagement” – ie the more tactical interaction - and the “Level of Engagement”. This last topic identifies how deeply involved the patient experts are with the collaboration. For this, we use the model designed by the University of Maryland[1]. Although this was set up for use in clinical trials, the basic concept can also be used for other areas of engagement.

[1] University of Maryland Center of Excellence in Regulatory Science and Innovation (CERSI), Assessing Meaningful Patient Engagement in Drug Development a Definition, Framework, and Rubric (2015)

| Patient Role | Examples | Engagement Level |
|---|---|---|
| Partnership role | • Patients provide a priori and continuous consultation on outcomes of importance, study design, etc.<br>• Patients are paid investigators or consultants<br>• Patients have a governance role; patients have "a seat at the table" | High |
| Advisor role | • Patients serve as advisory committee members or provide *a priori* consultation on outcomes of importance and study design, but have no leadership role or governance authority | Moderate |
| Reactor | • Patient input is collected distally through surveys, focus groups or interviews, but patients are not consulted directly or a priori on such things as study design and outcomes of importance<br>• Patients are asked to react to what has been put before them rather than being the origin of the concepts of interest | Low |
| Study subject | • Patients are recruited or enrolled as study subjects, but are not asked for input, consultation, or reaction | None |

## 1. Clinical Trials

a. Trial Design

Even if most clinical trials are designed at international level, it is important to gain insights from patients in different countries, including Belgium. Patient Experts can be in international panels. And because Belgium is an important country for clinical research, it is also critical that patients actively participate in the design of the studies.

The most important areas of engagement are
- Identification of endpoints & patient-relevant outcomes (PROMs/PREMs)
- Identification of patient preferences
- Discussion on benefit/risk & value
- Feedback to patients involved

In order to obtain these patient insights both qualitative and quantitative data are needed. Qualitative data can be obtained through a first focus group session to scope the project and understand the full context of patients living with the disease, followed by more detailed patient questionnaires and the final discussion of the questionnaire with preferably the same group of patients to identify the commonalities and differences among patients. The process will also address current issues with the standard-of-care, expectations for innovation, prioritisation of needs and preferences, weighing of the different parameters used to evaluate outcomes, including benefits, risks and value.

It is also essential to organise feedback to the patient organisation and to the patients ultimately enrolled in a study.

b. Trial Recruitment

Patient organisations can participate in the recruitment process of a trial, by reviewing the recruitment strategy and materials but also by disseminating the recruitment itself to the members of the organisation or through their communication channels.
Typical patient engagement processes include:

- Recruitment strategy & materials – this can be done in collaboration with the investigators and will largely depend on the type of disease, its incidence and facility of identifying potential candidates. Depending on the situation, patient testimonials (videos, apps, …) can be used to highlight the value of participating in trials, but also the caveats and eligibility. Flyers and posters should be reviewed by patient experts. Some patient organisations also announce via their websites or social media when trials start recruiting.
- Lay person summary – the materials presented to the patients to explain the trial itself, what it entails and how it will be set up, with all consequences for the patient should be explained in easy-to-understand language. Patient Experts should review the final material.
- Informed Consent Forms – the enrolled patients should understand what they are signing. Most informed consent forms are very legal (complete and accurate) but lack communications value (clarity). A good informed consent form starts with a one-page summary with the key messages.
- Dissemination of results – once the study has been completed, or when it is discontinued, patients should be informed of the result of the study in an easy to understand language. Patient organisations can assist the company with the reviewing of the materials.

c. Monitoring Tools

- Patient Diary – developing a patient diary to complete during the trial is the most common tracking tool about the quality-of-life of patients.
- Digital applications – the diary can also be set up as an app, with the additional advantage that it can be linked to other smartphone metrics such as physical activity and tools such as pill-coach.

We also refer to the more elaborated format designed at European level[2], as depicted in the following graph.

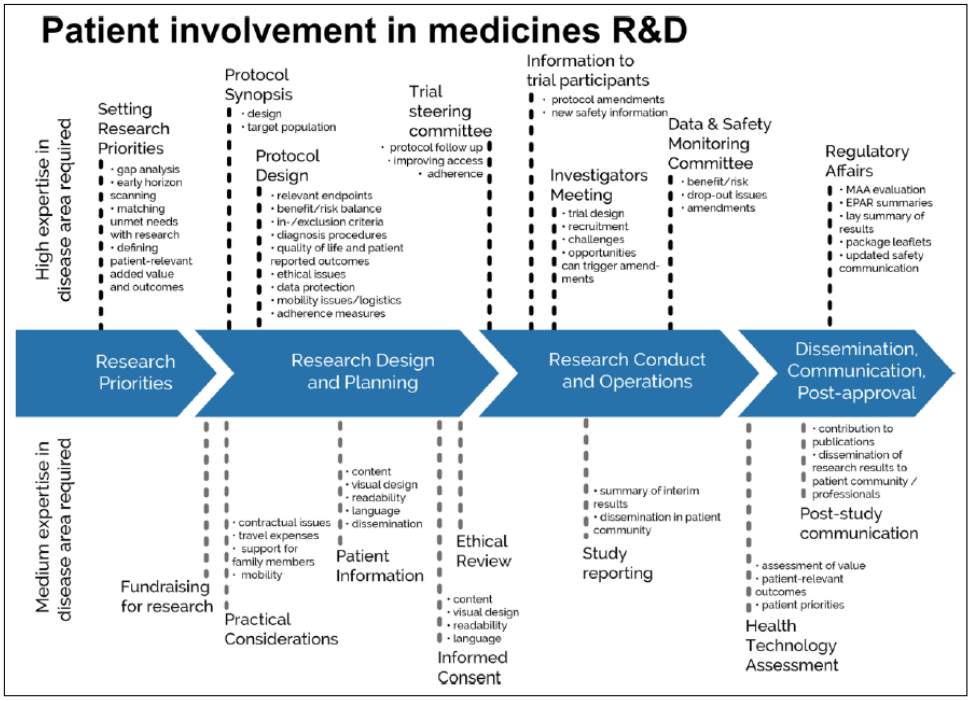


**Figure 3.** Practical roadmap for patient involvement in medicines R&D.

## 2. Disease Awareness Campaigns

Citizens are often not aware of the symptoms of a disease they might have, or insufficiently informed on the possible value of early diagnosis, or risk factors that could apply to them. Public health authorities and pharmaceutical companies will want to communicate about specific diseases when new treatment strategies or technologies become available.

Because patient organisations know their disease best, and especially which factors could motivate citizens to be more aware and conscious of what to look for, their engagement with the campaign is critical and should start as early as possible. We have identified the following actions:

a. Co-design of disease awareness campaign (objectives, strategy, tactics, ...)
    a. Core narrative and fact sheet
    b. Media communication and testimonials
    c. Advertising
    d. Information sessions among core stakeholders (GPs, hospitals, …)
    e. Street events
    f. Fundraising events
b. Review of disease awareness campaign (check objectives, tone, language, style, ...)
c. Patient Journey Description
    a. Describe the full impact of the disease and treatment on patient & family
        i. Patient focus group sessions to understand the full impact
        ii. Holistic approach vs medical only (person is not his/her disease)

[2] Geissler et al « Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap, In Therapeutic Innovation & Regulatory Science, 2017

## 3. Patient Education & Literacy

There is sufficient evidence in the medical literature that health literate and well-informed patients have better health outcomes. For pharmaceutical companies, it is also of the most importance to demonstrate that the promise of the drug in a clinical trial is also fully realised in a natural setting. The following action areas have been identified.

a. Identification of information needs
    a. Understanding patient knowledge and information needs
    b. Developing and reviewing of patient information materials and services in co-creation with stakeholders via editorial board (patients-carers / HCP's / nurses)
    c. Testing of messages, materials and technology

    Nature of the engagement
        i. Focus Group sessions
        ii. Help desk statistics
        iii. Surveys
        iv. Patient interviews

b. Patient Education and Patient Support Programmes

    This part includes educating patients on the best use of their medication, but also how to report any issues they may experience with pharmaceutical treatment, and that have not been included in the patient leaflet. It also includes education on how to use the treatment in the best possible way, emphasising the importance of compliance and adherence. This can also include the proper use of services such as digital pill alerts.

    a. Correct use of medication
    b. Compliance & Adherence
    c. Patient Safety and Pharmacovigilance
    d. Psychological support
    e. Beyond the Pill services

## 4. Real-World Data and Real-World Evidence

Increasingly, the capture of Real-World Data and Real World Evidence will become important for pharmaceutical companies to demonstrate the effectiveness of their medicines in a real life environment. In order to achieve this, programmes need to be set up to maintain/optimise the efficacy of the medicines by creating an environment in which it will be used as designed, almost replicating the conditions of a clinical trial, while at the same time tracking the actual results through the capture of data points. When the data can lead to meaningful insights, they become the required evidence that demonstrates outcomes.

a. Understanding the patient context
    a. Socioeconomic data and subpopulations (sociography, netnography, …)
    b. Patient surveys
    c. Focus Group sessions

b. Patient monitoring and data collection
    a. Patient diary
    b. Registries and claims databases
    c. Digital remote monitoring
    d. Natural history study
    e. Opinion surveys

c. Awareness on Real World Evidence & data collection
d. Health Economic analysis
    a. Cost of illness study
        i. Patient financial diary
        ii. Cost of premature mortality & morbitiy
        iii. Back-to-work analyses

The real value of a treatment is more than its clinical impact on the body. The total patient should be taken into account in his/her context. Value also includes Quality-of-Life indicators, but also the possibility for the patient to resume his/her life after treatment, or to continue to live with a quality of life that is rewarding for the individual and his/her family.

## 5. Market acces

When new treatments reach the market, it is important that the medical data are shared, but also that the patient is heard to explain why a particular treatment should be considered better than the standard-of-care. Hard medical data or clinical trial endpoints are of the essence, but patient insights are equally important. Few regulators and payers know what it means to live with a disease, and how treatments can expand the options for patients.

a. Reimbursement application
    a. Capture patient voice in value dossier (e.g. questionnaire)
    b. Invite patients to official hearing
    c. Patient voice toolkit

b. Increase patient's voice
    a. Surveys

## 6. Issues Management

During pharmacovigilance and Phase IV studies, new issues with medicines can arise, whether side effects or incompatibility with other treatments. Other issues might present themselves during production (quality or effectiveness issues, stock-outs, …). Sometimes, when newer drugs are entering the market, older ones are discontinued. In all these cases it is important that patient organisations are part of the process to evaluate how to communicate to their members, and how to help develop strategies and communications to address the situation, working towards the best possible solutions for the patient community.

a. Identification of new side effects
    a. Risk benefit analysis
    b. Patient testimonials
    c. Dissemination of information

b. Discontinuation/stock-out
    a. Fact sheets
    b. Treatment scenarios
    c. Participation in advisory board
    d. Dissemination of information
    e. …

## 7. Policy and Public Affairs

With the arrival of new technologies and new science, it is essential to also inform politicians, policy-makers and other decision-makers about the potential of new discoveries. Personalised medicine, immunotherapy, cell & gene therapy, the use of Artificial Intelligence, digital monitoring, secondary use of patient data … and other novelties require the patient perspective to explain their potential and value to the patient community.

## 8. Company Culture

Companies active in healthcare put great store in their patient-centric culture. To have a patient-centric culture, it is critical to have strong patient engagement and strong patient focus in all activities of the company. The active role of patient experts can help develop this internal culture for all management principles and employee engagement.

a. Create real patient-centricity in the organisation
    i. Patient Advisory Boards
    ii. Identify areas for improvement
        1. Analysis
        2. Surveys
        3. Benchmark
    iii. Disseminate key performance indicators
    iv. Develop signature programme

b. Visible patient engagement initiatives
    i. Patient days
    ii. Staff induction
        1. a patient can "train" newcomers in a firm on the patient aspects of the disease
    iii. Townhall meetings
    iv. …

**In conclusion**

The list of patient engagement above is just an overview of possibilities.

Companies that are interested in taking this a step further should either contact the patient organisation active in the disease they want to set up an initiative or with the Patient Expert Center. We will then come with a proposal based on a more substantial briefing on the topic for which patient engagement is sought.