PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

innovative medicines initiative

Planning Patient Engagement

Recommendations on the required capabilities for patient engagement

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Introduction

PARADIGM (Patients active in research and dialogues for an improved generation of medicines) was an IMI funded multi-stakeholder consortium to provide a framework for structured, effective, meaningful and ethical patient engagement along the lifecycle of medicines. The project focused on three decision-making points: research priority setting; clinical trial design; and early dialogues with regulators and health technology assessment (HTA) bodies. The result of the consortium / the output of the consortium is a comprehensive set of tools and practices to support the integration of the patient perspectives into medicines development beyond the focal areas of the project. Patient engagement should be a standard practice to improve medicines development and deliver results that are focused on patients' needs.

What is this tool?

This tool provides recommendations on a set of **capability requirements** for all stakeholders to implement patient engagement (PE) in the context of medicines development. These capability requirements should be defines as:

> **"The competencies (understood as knowledge, skills and behaviours) and resources that each stakeholder type should aspire to have in place in order to be able to undertake the planning, implementation and reflection of effective, ethical and sustainable patient engagement activities across the medicines lifecycle".**

The objective of the recommendations is to strengthen "system readiness" across all stakeholder groups to ease and systematise the implementation of PE by identifying the capabilities it requires. System or organisational readiness for change relates to the willingness and ability to take action[1]. Considering that patient engagement in medicines development is not a one-to-one activity but involves different stakeholder groups coming together in a collaborative partnership via their respective organisations, it was decided to focus not only on the competencies required by the individuals involved in implementing PE activities, but also on the resources required at an organisational level by all parties.

These recommendations may also be used to analyse the organisation's capabilities and consider the elements described here to further develop or adapt their own capability model.

This tool **does not** address the specific competencies of the patient participants in any specific patient engagement activity.

[1] Weiner BJ. A theory of organisational readiness for change. Implementation Sci. 2009; 4:67

Basic capability model for patient engagement

We propose a basic capability model consisting of four pillars, which should be applicable to and observed by any stakeholder organising or being involved in the specific PE activity:

1. **Competencies:** Combination of knowledge, skills and behaviours of an individual
2. **Processes:** Processes define how things can be done. They can change in accordance with internal policies, regulations, technologies and other influences.
3. **Tools and systems:** Instruments necessary to perform a specific task, from technological tools to the ability to use certain systems.
4. **Organisation:** Refers to the organisational structure (functions) of each stakeholder group and also to an organisational culture that enables ethical and meaningful engagement.

These capabilities should be designed in a way that they can be transferred between one stakeholder organisation to another to provide lateral support and knowledge exchange (transferability). Also, patient engagement provides unique opportunities to learn from other stakeholders' practices and processes and to learn from own experience (adaptability).

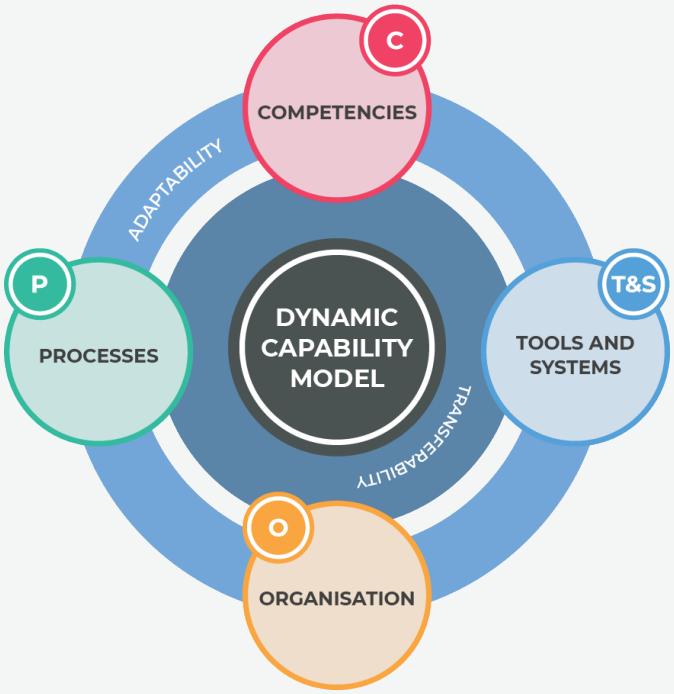

Figure 1. **The basic capability model**

[2] Patient Focused Medicines Development (PFMD) Patient Engagement Quality Guidance provides an agreed set of overarching principles that help ensuring the quality of PE in existing and future projects, and enables showcasing the results and impact of projects in a systematic way https://patientfocusedmedicine.org/the-patient-engagement-quality-guidance/ Accessed 28 Dec 2018

The following sections detail the key themes[2] identified for effective PE and detail respectively the identified capabilities required under each theme including:

1. **Shared purpose and roles and responsibilities of all stakeholders**
2. **Respect and accessibility**
3. **Representativeness of all stakeholders**
4. **Transparency in communication and documentation:**
   - 4.1. Legal agreements and confidentiality
   - 4.2. Management of competing interests
   - 4.3. Codes of conduct and rules of engagement
   - 4.4. Reach-out to and interaction with patients and patient organisations
5. **Continuity and sustainability:**
   - 5.1. Financial compensation
   - 5.2. Measuring patient engagement impact
6. **What to consider when engaging with potentially vulnerable populations**

Core set of capabilities

The core set of capabilities describes the capabilities that correspond to identified high-priority criteria that should be fulfilled when designing or implementing PE activities:

- the aims and objectives of the PE activity are agreed and understandable by all stakeholders
- the objectives should be aligned with patients' needs and
- the appropriate target population matching the activity objectives should be selected.

The capabilities related with these high-priority aspects of PE are considered core, from which capabilities specific to other relevant themes can be further elaborated.

Core set of capabilities for patient engagement

C COMPETENCIES

Knowledge

- Understanding of stakeholders' objectives, structures and conditions
- Knowledge of negotiation techniques to facilitate consensus building
- Knowledge and understanding of the concepts of diversity
- Knowledge of the patients' ecosystem
- Knowledge of the PE ecosystem and of their own stakeholder organisation

Skills

- Collaborative leadership skills
- Research and enquiry skills
- Knowledge and skills to generate/incorporate evidence-based patient input (e.g. conduct and apply qualitative research; design, test, analyse and apply patient reported outcome instruments or patient preference surveys)
- Ability to reach-out to patient and patient organisations (especially in new emerging disease areas) and to build mutually beneficial relationships before the engagement occurs

Behaviours

- Empathy
- Being open towards each stakeholders' own goals and objectives and acknowledging that they might differ
- Understanding and being sensitive to patients' accessibility needs

P PROCESSES

- Establishment of procedures to ensure consistency and traceability (e.g. agendas, reports, minutes, etc.).
- Policies on inclusion and non-discrimination.
- Guidelines of inclusive and representative patient engagement.
- Periodical multi-stakeholder meetings/ checkpoints to align on objectives and to reassess the roles and responsibilities to identify any deviation.
- Feedback-collection methods in place before (to assess needs and expectations), during (to detect and correct any deviations of the agreed upon goals and objectives) and after project completion (to get relevant feedback for future interactions).
- Flexible and adapted processes to include non-English speaking patients.

T&S TOOLS AND SYSTEMS

- Instruments to collect feedback (e.g. surveys, questionnaires, digital feedback portal).
- Structured feedback sessions (virtually or face-to-face, one-to-one interviews, focus groups, etc.) and other informal mechanisms of personal exchange.
- Databases[1] permissive of identifying the right individual for a specific activity, according to their experience and expertise.
- Tools to reach out to a large number of patients (or other stakeholders), either proprietary or via an intermediary (e.g. via a patient organisation)
- Guidance to stakeholders on the process of engagement

[1] The use of databases is subjected to data privacy regulation requirements and may not apply to all stakeholders. Some organisations, such as patient organisations, use databases for membership management purposes. Also, EMA individual experts' stakeholder database main purpose is to identify patients and consumers to participate in EMA activities.

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O ORGANISATION

Patient engagement functions and other organisational functions involved in the activity. The right expertise will be brought in at the right moment during the process of engagement. In particular, legal and compliance functions within stakeholder groups may have an important role in the definition and application of the rules of engagement and in specific aspects such as the development of reasonable legal agreements and the management of confidentiality and competing interests. Finance functions will be involved in developing and applying the financial compensation framework.

1. Shared purpose and roles and responsibilities of stakeholders in patient engagement

The set of capabilities required when planning and conducting a PE activity should ensure that:

1. The purpose of the activity is clearly defined, shared and agreed by all stakeholders involved.
2. The purpose is aligned with patients' needs and interests and includes the rationale and what is expected to be achieved with such engagement.
3. The roles and responsibilities of all stakeholders involved are defined and agreed in a clear and accessible manner in writing before the start of an interaction, and maintained throughout the project/timeframe.
    - The rules of engagement are clearly defined in terms of format and frequency, including what can be shared and how, as well as who is accountable for what and each other's expectations from the process and outputs of engagement.

Table 1. Shared purpose and roles and responsabilities of stakeholders in patient engagement

2. Respect and accessibility

RESPECT

PE should be conducted working in equal partnerships that are built on mutual trust, respect and transparency. Respect for persons is one of the ethical principles underpinning biomedical research, encompassing respect for the individual's autonomy to make their own choices and the protection of vulnerable persons whose autonomy may be impaired or diminished.[1]

Respect for the diversity and rights of the people involved, as well as understanding the drivers and facilitators of health-related stigma and their negative impact on health outcomes, will also help facilitating the engagement of certain at-risk groups.[2]

ACCESSIBILITY

At the European level laws exist regarding accessibility in some contexts and the duty to make "reasonable adjustments" for people with disabilities[3]. In addition, the European Accessibility Act[4] aims to make consumer products and services more accessible for people with disabilities across the EU. In addition, EU guidance ensures that the labelling and package leaflet are accessible and understood by those who receive it, in order to guarantee the safe and appropriate use of medicinal products.[5]

Addressing accessibility issues may be of benefit to all stakeholders and types of patients. Examples range from adapting material to patient's age and condition, finding an accessible venue for wheelchair users, to adapting the time of the meetings to patients' care needs. Access audits for events and for the accessibility of the written information provided to patients can help (see Enhancement of the EUPATI industry guidance). Some groups (e.g. sex workers living with HIV, substance users, transgender people) may experience accessibility issues that go beyond physical and practical barriers for engagement (e.g. prohibition to travel to certain countries).

Also, adapting the structure and style of communication is important and includes:

- Avoiding jargon and technical terms
- Using plain and respectful language
- Clear format and layouts

[1]Council for International Organizations of Medical Sciences (CIOMS). International Ethical Guidelines for Biomedical Research Involving Human Subjects. Geneva, 2002
[2]Stangl AL, Earnshaw VA, Logie CH, van Brakel W, Simbayi LC et al. The Health Stigma and Discrimination Framework: a global, crosscutting framework to inform research, intervention development, and policy on health-related stigmas. BMC Medicine (2019) 17:31. https://doi.org/10.1186/s12916-019-1271-3
[3]Equality Act 2010. https://www.legislation.gov.uk/ukpga/2010/15/contents
[4]Directive (EU) 2019/882 of the European Parliament and of the Council of 17 April 2019 on the accessibility requirements for products and services. European Accessibility Act.
[5]EU Guideline on the readability of the labelling and package leaflet of medicinal products for human use. Revision 1, 12 January 2009

- Considering communication contents: understanding what is appropriate (or not) to ask and expect from patients and, how to ask relevant questions
- Providing easy-to-read versions of the materials and, when appropriate, summaries of the contents with links/access to the full document. Such materials should be non-objectionable or inoffensive and age-appropriate.
- Alternative formats of presenting the information (e.g. audios when involving visually-impaired patients) are also advisable.

Table 2. Respect and accessibility

3. Representativeness of all stakeholders

BALANCE BETWEEN DIVERSITY AND EXPERTISE

Patient engagement in medicines development calls for the best achievable balance between diversity of stakeholders and the expertise and experience required. Within the patient community, also, it is important that a full range of 'patients' with different experiences and perspectives have opportunities to get involved.

INCLUSION OF UNDERREPRESENTED GROUPS AND VULNERABLE POPULATIONS

Efforts should be made to engage with underrepresented groups who are appropriate to the population and questions being asked - or vulnerable populations with specific needs. Sensitivity to the needs of vulnerable populations, and in general, people living with any type of disability or stigma, should be reflected in the processes. In some cases, including the carers' contribution is essential to provide a more holistic view of the disease and treatment burden.

Representativeness may be conditioned by the characteristics of the disease(s) as is the case of rare diseases, in which very few patients might be available for a condition with very low prevalence. Low numbers of patients combined with little or no expertise in medicine's development may influence the diversity and inclusiveness (i.e. the same patient(s) are the ones repeatedly engaged). Likewise, in the case of dementia, certain cancers and a number of other debilitating or neurological conditions, the involvement of people at more advanced stages of the disease may be challenging. In this sense, knowing the difficulties and barriers for engagement of a given community will help to overcome them.

GEOGRAPHICAL AND GENDER DIVERSITY

Depending on the type of PE activity, it may be important to capture the differences that may exist across geographic regions and that could influence the quality of the engagement. In addition, it is a principle of justice to provide equal opportunities regardless of the geographical area of origin. The breadth of geographical spread is also important when it comes to the representativeness of patient networks (i.e. European or global patient organisations). On other occasions, the geographical distribution of a disease will determine the degree of geographical diversity of the patients engaged.

Balanced gender representation should be sought where appropriate. However, it should be acknowledged that some chromosomal diseases affect one sex and not another, and that the burden of patient's care in daily life falls usually on female carers[1] and therefore equal sex representation might not be feasible or even advisable.

Table 3. Representativeness

[1]EURORDIS-Rare Diseases Europe (2017). Juggling care and daily life: The balancing act of the rare disease community. Survey of over 3000 people, conducted through the EURORDIS survey initiative Rare Barometer Voices: eurordis.org/voices

4. Transparency in communication and documentation

Under the overarching concept of transparency, this section describes the specific capabilities that stakeholders organising PE activities should have in the following domains of trust and transparency:

- Legal agreements and confidentiality
- Managing competing interests
- Establishing codes of conduct and rules of engagement
- Reach-out to and interact with patients and patient organisations

4.1 Legal agreements and managing confidentiality

Collaboration between the patient community and other stakeholders, especially the pharmaceutical industry, often requires a written agreement that states not only the terms and scope of the collaboration but also includes the roles and responsibilities of each partner, how the collaboration is supposed to take place and, the financial contributions.[1,2]

The need to protect commercial and confidential information should be balanced with a company being sufficiently open and transparent to enable meaningful and informed trust-based PE. Confidentiality Agreements (CAs) and Non-Disclosure Agreements (NDAs) allow the sharing of sensitive information with patient organisations, which must abide by the agreements they sign with sponsors to enable open communication.

Confidentiality also applies to sensitive (non-public) information that exists within patient organisations, who may have legitimate reasons to protect what they consider confidential. In this case sponsors must also abide by NDAs signed with patient organisations[3] and other stakeholders involved in PE (academic researchers, regulatory agencies, HTA bodies) may need to adhere likewise.

Regarding vulnerable populations, it is important to highlight the legal capacity to sign a legal document or contract on behalf of children and young people, people with dementia, people living with a mental health condition and other vulnerable groups such as incarcerated populations.

[1]EFPIA Code of Practice on Relationships between the pharmaceutical industry and patient organisations. Initially approved in 2007. Amended by decision of the General Assembly in June 2011. Last accessed 22 Apr 2019. https://www.efpia.eu/media/24310/3c_efpia-code-of-practice-on-relationships-pharmaluspt-orgs.pdf
[2]Guiding principles on reasonable agreements between patient advocates and pharmaceutical companies. https://www.mpeurope.org/legalagreements/wp-content/uploads/2019/03/Legal_Agreements_A5_3mm-bleed_PRINT_v2.pdf. Last accessed on 15 Apr 2019
[3]Clinical Trial Transformation Initiative (CTTI) recommendations: effective engagement with patient groups around clinical trials. Available at: https://www.ctti-clinicaltrials.org/files/pgctrecs.pdf

The approach taken may differ, but it always should respect the autonomy of the person. In the case of young people, the framework developed by the European Young Person's Advocacy Group (eYPAGnet) involves minors as a group on behalf of the institution they belong to. Balanced gender representation should be sought where appropriate. However, it should be acknowledged that some chromosomal diseases affect one sex and not another, and that the burden of patient's care in daily life falls usually on female carers and therefore equal sex representation might not be feasible or even advisable.

Table 4.1 Legal agreements and managing confidentiality

4.2 Managing competing interests

Everyone has interests. Interests generate responsibilities and one should be aware of those responsibilities. Different interests can come into competition or conflict if undisclosed or unmanaged as they can result in potentially biased decision-making, a lack of objectivity and serious damage to the reputation of individuals or organisations, and ultimately cause incorrect decisions during medicines development[1].

In the case of patient engagement, it is essential to protect the process and the integrity of the parties involved (i.e. the patient and the engaging stakeholder). It is the engaging stakeholder who/that defines what constitutes a conflict of interest for a particular process. Competing interests and conflicts of interest can be defined as follows:

- Competing interests as those interests that may affect an individual's impartiality but that do not constitute a conflict per se. They should be declared for transparency purposes; and
- Conflict of interest as a situation in which the individual's judgement may be perceived as being affected by a secondary interest, as defined by the engaging stakeholder(s).

Effective PE involves participation of patients in interlinked processes from defining unmet needs to meaningful input on clinical endpoints or regulatory scientific advice and HTA assessments. Thus, patients do not always engage with one stakeholder at a time, but multiple, simultaneous interactions may occur. Therefore, they are likely to find themselves at the cross-roads between different stakeholders and different types of interactions.

In order to effectively discriminate between competing interest and conflicts of interests, the 'engaging' organisation has to put in place a process aimed at collecting information on the interests of the patients (declaration of interests form), a process/policy to assess it, and put in place the appropriate subsequent measures.

Table 4.2 Managing competing interests

[1]Raising awareness on managing competing interests in a multi-stakeholder environment: Guidance to patients and engaging stakeholders. http://imi-paradigm.eu/PEtoolbox/conflict-of-interest

4.3 Codes of conduct and rules of engagement

A code of conduct (also named code of ethics or code of practice) for a group or an organisation defines the rules of behaviour for the members of that group or organisation. For instance, a code of conduct of a professional organisation defines its mission, values and principles and establishes the standards of professional conduct (internally and externally).

The rules of engagement describe how a particular stakeholder arranges patient involvement across the medicines development process. They give legitimacy to the process of engagement and help clarifying the involvement of each stakeholder in the project or activity - they are publicly available and revised periodically. Examples of such rules exist at all stakeholder levels (see Additional resources).

Some differences in terms of process capabilities exist between patient organisations, pharmaceutical companies, regulatory agencies and HTA bodies or academia. There are often significant differences in the capacities and resources they are able to deploy. Also, stakeholders may have to respect different legislative and compliance frameworks - regulatory agencies and healthcare providers may be limited to national legislative scopes, while companies, academic institutions and patient groups often operate in multi-national environments. This complication needs to be considered in order to create a level playfield for all stakeholders involved.

The code of conduct and rules of engagement should be clear and transparent, known by all members of the organisation and available to the public.

Table 4.3 Codes of conduct and rules of engagement

4.4 Establish contact and interact with patients and patient organisations

Identify the right match for the patient engagement activity[1] is an essential step in the patient engagement process. The capabilities described in Table 4.4 may apply to all functions within stakeholder organisations directly involved in PE. However, they are of major importance for patient engagement functions who usually have a referent and expert role both for patients and patient organisations and the relevant functions in their own organisation.

Table 4.4 Establish contact and interact with patients and patient organisations

[1]D4.2 Recommendations on how to find the right match for the right patient engagement activity. http://imi-paradigm.eu/PEtoolbox/identification-of-patient-representatives

5. Continuity and sustainability

Under the umbrella of continuity and sustainability, we have included the capabilities related to financial compensation of patient participants and measurement of patient engagement impact.

5.1 Financial compensation

Covering expenses incurred during or as part of participating in any PE activities is considered good practice. Some organisations may also offer payment to the persons contributing to PE activities for their time, skills and expertise. Different rules may exist at the national, or even organisational level, determining who is eligible for payment, what aspects can be covered, and how much patients can be paid for their involvement. However, examples of expenses that are typically offered include: expenses related to travel, accommodation and subsistence, replacement carer costs, administration costs, and fees of conferences. For some vulnerable patient groups, planning for and covering the expenses of a person who can provide support for travelling or during the activity is essential to enable their engagement. Covering expenses in advance (so that patients are not left out of pocket or put at risk of being financially worse off as a result of their involvement) will facilitate the participation of patients and other stakeholders.

Receiving payment(s) may have implications for the Income Tax or National Insurance contributions of some patients, particularly those receiving state benefits or a pension. National rules and laws may differ between countries. The way that reimbursement of expenses is settled should not create barriers that deter patients from being involved. Whenever possible and while respecting the applicable laws, as well as stakeholders' policies, alternative rewards or benefits, or other possibilities, should be offered to patients who prefer for any reason not to receive a direct payment.

All these expense and payment considerations should be addressed in a clear and easy policy (or policies) prior to starting, and throughout the PE activity, and according to existing guidances[1,2], (see also Additional resources). The application of different terms, conditions, or procedures by different departments or groups of any stakeholder should be avoided unless fully justified by compliance reasons.

Table 5.1 Financial compensation

[1]EFPIA Code of Practice on Relationships between the pharmaceutical industry and patient organisations. Initially approved in 2007. Amended by decision of the General Assembly in June 2011. Last accessed 22 Apr 2019. https://www.efpia.eu/media/24310/3c_efpia-code-of-practice-on-relationships-pharmapluspt-orgs.pdf

[2]Guiding principles on reasonable agreements between patient advocates and pharmaceutical companies. https://www.mpeurope.org/legal_agreements/wp-content/uploads/2019/03/Legal_Agreements_A5_3mm-bleed_PRINT_v2.pdf. Last accessed on 15 Apr 2019

5.2 Measuring patient engagement impact

There is increasing interest to demonstrate the added value or "return on engagement" of involving patients in decision-making across the medicine's development spectrum[1]. Moreover, demonstrating the value (of a particular activity or framework) to any given stakeholder is considered among the essential factors to ensuring the success and sustainability of a project or an organisation[2].

PARADIGM has developed a monitoring and evaluation framework that considers the context (i.e. political, cultural, institutional, etc.) and mechanisms (such as the type of engagement) and their respective effects on outcomes and impact of patient engagement at three decision-making points in medicines development. Human and organisational capabilities required to measure impact are based on the elements and dimensions covered in the framework.

Table 5.2 Measuring patient engagement impact

[1] Vat LE, Finlay T, Jan Schuitmaker-Warnaar T, et al. Evaluating the "return on patient engagement initiatives" in medicines research and development: A literature review. Health Expect. 2020;23(1):5-18. doi:10.1111/hex.12951
[2] Internal PARADIGM assessment review of existing sustainability models.

6. What to consider when engaging potentially vulnerable populations

Involving potentially vulnerable patient groups in often considered a challenge and, as a consequence, these patients have historically largely been excluded from PE activities, or indeed sometimes another person has been invited on their behalf (e.g. a relative or carer).

Although patient involvement may, in some cases require extra effort, patients are the ones living with the condition and their contribution can be extremely powerful and important. Close relationships with patient organisations linked to the condition, or other organisations with expertise in how to involve the particular vulnerable group, could help to address the challenges of how to plan and execute the involvement of these patient groups in a meaningful way.

The list of capabilities described may be required by staff involved in patient engagement with potentially vulnerable populations, such as children and young patients and people living with dementia and their carers, and other underrepresented groups such as migrants and non-settled populations, incarcerated people or substance users, people with mental health disorders other than dementia. The sections Respect and accessibility and also Representativeness of all stakeholders also covers the capabilities required when engaging vulnerable groups.

Table 6. Specific capabilities to consider when engaging with vulnerable populations

Recommendations

This document describes a set of capabilities required by all stakeholder organisations involved in the planning, implementation, reflection and evaluation of PE activities. Each stakeholder can use these recommendations to analyse their own organisation's capabilities at a given moment and consider the elements described here to further develop or adapt the capability model existing in their own organisations.

In general, stakeholder organisations involved in PE are expected to:

1. Equip their organisations with organisational functions holding the core competency set and the core set of processes, tools and systems to be able to effectively carry out these priority areas: the aims and objectives of the PE activity are agreed and understandable by all stakeholders, aligned to patients' needs and, that the right patient population matching these objectives is selected.
2. This core set of capabilities should be expanded, where needed, to cover all relevant areas of PE from initially building the engagement framework to evaluating a particular activity for further improvement.

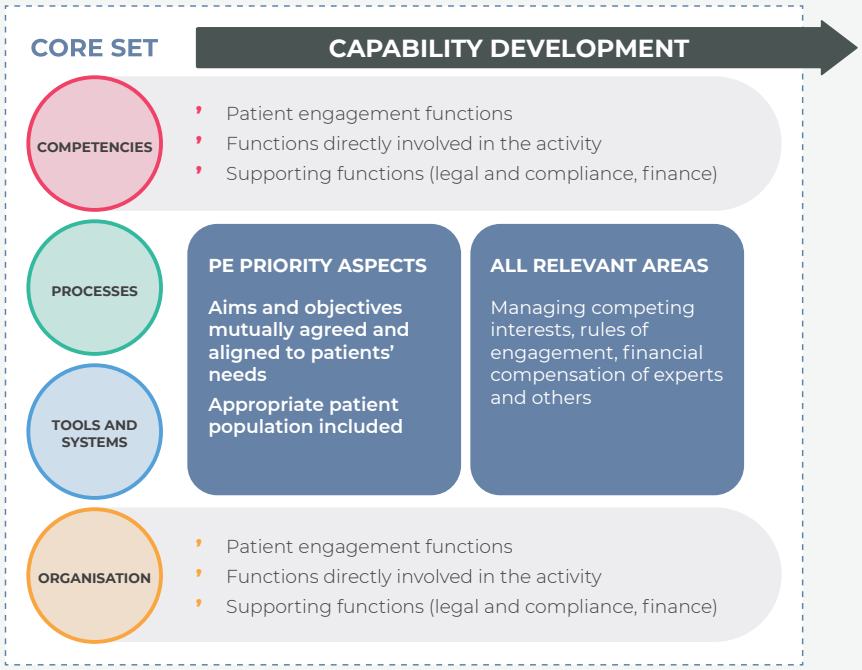

Figure 3. **Overview diagram of the capability framework. Core set of capabilities to carry out PE priority aspects can be further expanded to cover all relevant areas of PE**

3. Across organisations, the individual competencies should rely on the functions directly involved in the activities and more specifically will be concentrated within the functions dedicated to PE, which will act as the experts on the area of engagement and will act as a single point contact centralizing internal and external interactions.
4. Supporting functions (e.g. legal and compliance, and finance) will be also required to hold certain competencies in the areas of interest related to their specific function (e.g. managing competing interests, establishing a financial compensation framework).
5. It is for stakeholder organisations to decide whether other functions require competencies for PE based on their particular involvement, or on the organisational culture.

Limitations

- This is a reference tool for all stakeholders considering PE activities. The list of capabilities is not intended to be exhaustive.
- This tool is not intended to be prescriptive and will not give detailed step-by-step advice. The recommendations should be used according to specific circumstances, national legislation or the unique needs of each interaction. They should be adapted for individual requirements, and to each stakeholder organisation, using best professional judgment. Similarly, this document does not address the specific competencies of the patient participants in any specific patient engagement activity.
- It is not expected that every individual nor all the staff of an organisation involved in a PE activity will have all the required competencies. Given their central role in the process of PE, patient engagement functions will have most of the individual competencies listed in the document. Other functions within an organisation may require such competencies when involved in PE activities. It is beyond the remit of the recommendations here to specify which roles and functions should be involved at each moment, as these may change depending on the PE activity and/or the organising stakeholder.
- Although most of the capabilities described here can be applied across the medicine's lifecycle and extended to the post-authorisation phase, specific situations (e.g. PE with payers or healthcare professionals) may require a set of specific capabilities which are beyond the scope of these recommendations.
- The list of additional resources aims to help the reader to identify relevant sources of information, but is not intended to be exhaustive.

Glossary

Disclaimer: The terms used here have been defined or agreed upon within the context of this project.They should not be considered as exhaustive, finite or purposely exclusive of other considerations, but are representative of the specific focus of this project and its actions.

Code of conduct:

Collection of rules and regulations that include what is and is not acceptable or expected behaviour (PARADIGM)

Community Advisory Board:

Community Advisory Board (CAB) refers to a group of patients who offer their expertise to sponsors of clinical research and who advise several sponsors in their field. CABs are autonomous bodies, not related to the sponsor or chosen by them. (EURORDIS)

Confidentiality Agreement (CA)/Non-disclosure agreement (NDA):

Legal contract between at least two parties that outlines confidential material, knowledge, or information that the parties wish to share with one another for certain purposes but wish to restrict access to. (https://en.wikipedia.org/wiki/Non-disclosure_agreement)

Consultancy:

Advice provided on company- or academia sponsored clinical trial protocols including related documents, regulatory documents or information about the products under discussion (e.g. medicinal products, biomarkers), strategic initiatives and other projects of commercial or academic relevance (PARADIGM)

Design of clinical trials:

Designing protocols, discussing patient burden, discussing patient related outcomes (PARADIGM)

Early dialogues with regulators and Health Technology Assessment bodies:

Early (multi-stakeholder) discussions between industry, HTA agencies and/or regulators (and in some contexts with payers) to discuss developmental plans for a medicinal product and to ensure they meet the requirements.

* *Early dialogue is not a decision-making time for any party. In practice it more closely resembles consultation with the chance for feedback and input (two-way communication).* (PARADIGM)

Health Technology Assessment (HTA):

Systematic evaluation of the properties and effects of a health technology, addressing the direct and intended effects of this technology, as well as its indirect and unintended consequences, and aimed mainly at informing decision making regarding health technologies. HTA is conducted by interdisciplinary groups that use explicit analytical frameworks drawing on a variety of methods. (http://htaglossary.net/health+technology+assessment)

Health technology assessment (HTA) body:

A body that undertakes or commissions health technology assessment to form recommendations or advice for healthcare funders and decision-makers on the use of health technologies (PARADIGM)

Healthcare professional (HCP):

This category of stakeholders is broad and heterogeneous as it encompasses general practitioners, nurses, clinical investigators/academics, pharmacologists, etc. (PARADIGM)

Medicine developer:

Includes any organisation involved in the research, development, manufacture, marketing and/or distribution of medicinal products and/or any other health products such as medical devices or digital solutions. Clinical/contract research organisations (CROs) or consultancy companies providing advice or services relating to the above activities, fall under the definition of medicines developers. Research organisations including universities and learned societies (i.e. an organisation that exists to promote an academic discipline, profession) are also included in the definition of medicines developers (PARADIGM)

Medicines development/medicines research and development (R&D)/ medicines lifecycle (in PARADIGM these terms are used interchangeably):

A medicines lifecycle comprises research and discovery, development (preclinical and clinical), marketing authorisation, post-approval, HTA, pricing and reimbursement, commercialization, lifecycle management and Pharmacovigilance until deregistration. (PARADIGM, adapted from: EUPATI; European Commission; EFPIA; Frontiers 'The Life Cycle of Health Technologies. Challenges and Ways Forward, Iñaki Gutiérrez-Ibarluzea et. al. 2017')

Memorandum of Understanding (MoU):

Type of agreement between two (bilateral) or more (multilateral) parties. It is not legally binding, but it expresses willingness between the parties to take forward a common line of action. (https://www.investopedia.com/terms/m/mou.asp)

Participating organisation/engaging partner:

An organisation which is organising and/or participating in a PE activity (PARADIGM)

Patient covers the following definitions:

- **"Individual Patients"** are persons with personal experience of living with a disease. They may or may not have technical knowledge in R&D or regulatory processes, but their main role is to contribute with their subjective disease and treatment experience.
- **"Carers"** are persons supporting individual patients such as family members as well as paid or volunteer helpers.
- **"Patient Advocates"** are persons who have the insight and experience in supporting a larger population of patients living with a specific disease. They may or may not be affiliated with an organization.
- **"Patient Organization Representatives"** are persons who are mandated to represent and express the collective views of a patient organization on a specific issue or disease area.
- **"Patient Experts"**, in addition to disease-specific expertise, have the technical knowledge in R&D and/or regulatory affairs through training or experience, for example EUPATI Fellows who have been trained by EUPATI on the full spectrum of medicines R&D.

(The European Patients' Academy on Therapeutic Innovation (EUPATI)

Patient community:

Patients, patient representatives including their family and carers, patient advocates and patient organisations (PARADIGM)

Patient engagement:

The effective and active collaboration of patients, patient advocates, patient representatives and/or carers in the processes and decisions within the medicines lifecycle, along with all other relevant stakeholders when appropriate (PARADIGM)

Patient organisations:

Patient organisations are defined as not-for profit organisations which are [patient-]focused, and whereby patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies (EMA 2018a)

Payer:

Institution, organisation or individual paying for healthcare or health services (PARADIGM)

Pharmaceutical industry:

The pharmaceutical industry is comprised of many public and private organizations that discover, develop, manufacture and market medicines for human and animal health. In short, the term "industry" is used to refer to the pharmaceutical industry (PARADIGM)

Policy-maker(s) (or policymaker(s)):

A member of a government department, legislature, or other organization who is responsible for making new rules, laws, etc. (https://dictionary.cambridge.org/dictionary/english/policymaker)

Regulatory authority (or regulatory agency or in short 'regulators'):

A body that carries out regulatory activities relating to medicines, including the processing of marketing authorisations, the monitoring of side effects, inspections, quality testing and monitoring the use of medicines. (EMA)

Representative for pharmaceutical industry:

An employee of the pharmaceutical industry designated to represent the company position in project/consortium/body (PARADIGM)

Research priority setting:

Providing opinion, providing evidence and/or being part of a group that decides what is important to research. Design of clinical trials (PARADIGM)

Three main decision-making points:

The term, 'decision-making points' is defined as the key points in the development lifecycle of medicinal products. The three decision-making points relevant to PARADIGM are: research priority setting, design of clinical trials and early dialogues with regulators and Health Technology Assessment bodies (PARADIGM)

Vulnerable / underrepresented groups:

Children and young patients, people living with dementia and their carers. This definition can also include underrepresented groups (e.g. migrant and non-settled populations, substance users, incarcerated people and people with mental health disorders other than dementia). (PARADIGM)

Terms related to the capability model

Adaptability:

Refers to the ability to respond to a change in circumstances or environment, in other words it shows the ability to learn from experience.

Capability

Combination of capacity and infrastructure (processes, tools and systems, and organisational structure).

Capacity:

Combination of competencies and availability of resources (human, financial, and organisational).

Competence:

Status of having acquired all competencies and ability of applying them effectively.

Competency:

Combination of knowledge, skills and behaviours of an individual. Competencies can be acquired with training and/or through personal experience, in turn leading to overall competence.

Organisation:

For the purpose of this document, it refers to the organisational structure (functions). It can also refer to the favourable organisational culture that can enable ethical and meaningful engagement.

Processes:

For the purpose of this document it describes how things can be done, and they can change in accordance with internal policies, regulations, technologies and other influences. Despite their adaptability, processes must be well defined and include how to interact with other stakeholders.

Tools and systems:

For the purpose of this document, they are the instruments necessary to perform or guide the implementation of a specific task. These can vary from technological tools to the ability to use certain systems in agreement with the other stakeholders.

Transferability:

Refers to knowledge, functions, processes and tools, in part or in whole that should be designed in a way that they can be transferred between one stakeholder organisation to another to provide lateral support and knowledge exchange.

Annex 1: **Role of patient engagement functions**

Results from the assessment of needs and expectations regarding PE[1], showed that the patient community would like to have "one-to-one support especially from the organising stakeholder and from a person/group with in-depth knowledge about the area of engagement". In addition, the majority of industry respondents declared that despite having a dedicated PE function in the organisation, their actual involvement in PE activities in the 3 decision-making points was rather limited. Other sources have also shown the need for a coordinating function for PE in research[2].

Nevertheless, it must be emphasised that PE not only occurs in the PE department or related functions, but expanded to the functions directly performing PE. On an organisational level, it should be enabled through permissive implementation and supportive cultural structures and ethos, supported by top management.

PE functions in the different stakeholder groups may act as a single point of contact either as a nominated person or a department and take responsibility to:

- Identify the right patients for the PE activity.
- Operationalise and manage PE throughout the process from start-to-finish.
- Handle requests for collaboration that can cover a very wide range of activities.
- Ensure maintenance of the quality of the PE process among the different functions involved (e.g. Clinical research team).
- Establish and/or implement the defined framework for PE.
- Be accountable towards the processes.
- Act as reference/ expert in patient engagement within their organization.
- Raise awareness about and foster PE.
- Provide support to other functions on PE.
- Organise training on PE for the organisational functions directly involved in PE.

Patient engagement functions might be organised by types of activities in which the engagement of patients is requested (i.e. clinical trial design) or by medical areas.

For example, pharmaceutical companies may have a PE department dedicated to rare

[1] Faulkner et al. manuscript submitted for publication.
[2] Crocker S et al. PIRRIST: A patient and public involvement (PPI) intervention to enhance recruitment and retention in surgical trials Presentation slides at PIRRIST seminar held in Oxford on 12th March 2019. NIHR Oxford Biomedical Research Centre & Nuffield Department of Primary Care Health Sciences, University of Oxford.

diseases, while regulatory agencies may have staff dedicated to engaging with patients and staff dedicated to engaging with academics and/or clinicians. Similarly, PE functions in patient organisations could be organised by activity or stakeholder.

In this context, PE functions may act as "knowledge brokers"[3,4] to bridge the knowledge gap between the patient participants in PE activities and the relevant functions in the R&D process. And similarly, some competencies related with knowledge translation in health care[5] are common to those required by PE functions: understanding the context, understanding the research process, how to share available knowledge in an accessible way, establishing trusting relationships and engaging with others, leadership skills, facilitating knowledge exchange among stakeholders and facilitating collaboration and co-creation.

[3] Bornbaum CC, Kornas K, Peirson L, Rosella LC. Exploring the function and effectiveness of knowledge brokers as facilitators of knowledge translation in health-related settings: a systematic review and thematic analysis [published correction appears in Implement Sci. 2015;10:171]. Implement Sci. 2015; 10:162. Published 2015 Nov 20. doi:10.1186/s13012-015-0351-9

[4] Bornbaum CC, Kornas K, Peirson L, Rosella LC. Exploring the function and effectiveness of knowledge brokers as facilitators of knowledge translation in health-related settings: a systematic review and thematic analysis [published correction appears in Implement Sci. 2015;10:171]. Implement Sci. 2015; 10:162. Published 2015 Nov 20. doi:10.1186/s13012-015-0351-9

[5] Mallidou AA, Atherton P, Chan L, Frisch N, Glegg S, Scarrow G. Core knowledge translation competencies: a scoping review. BMC Health Serv Res. 2018;18(1): 502. doi:10.1186/s12913-018-3314-4