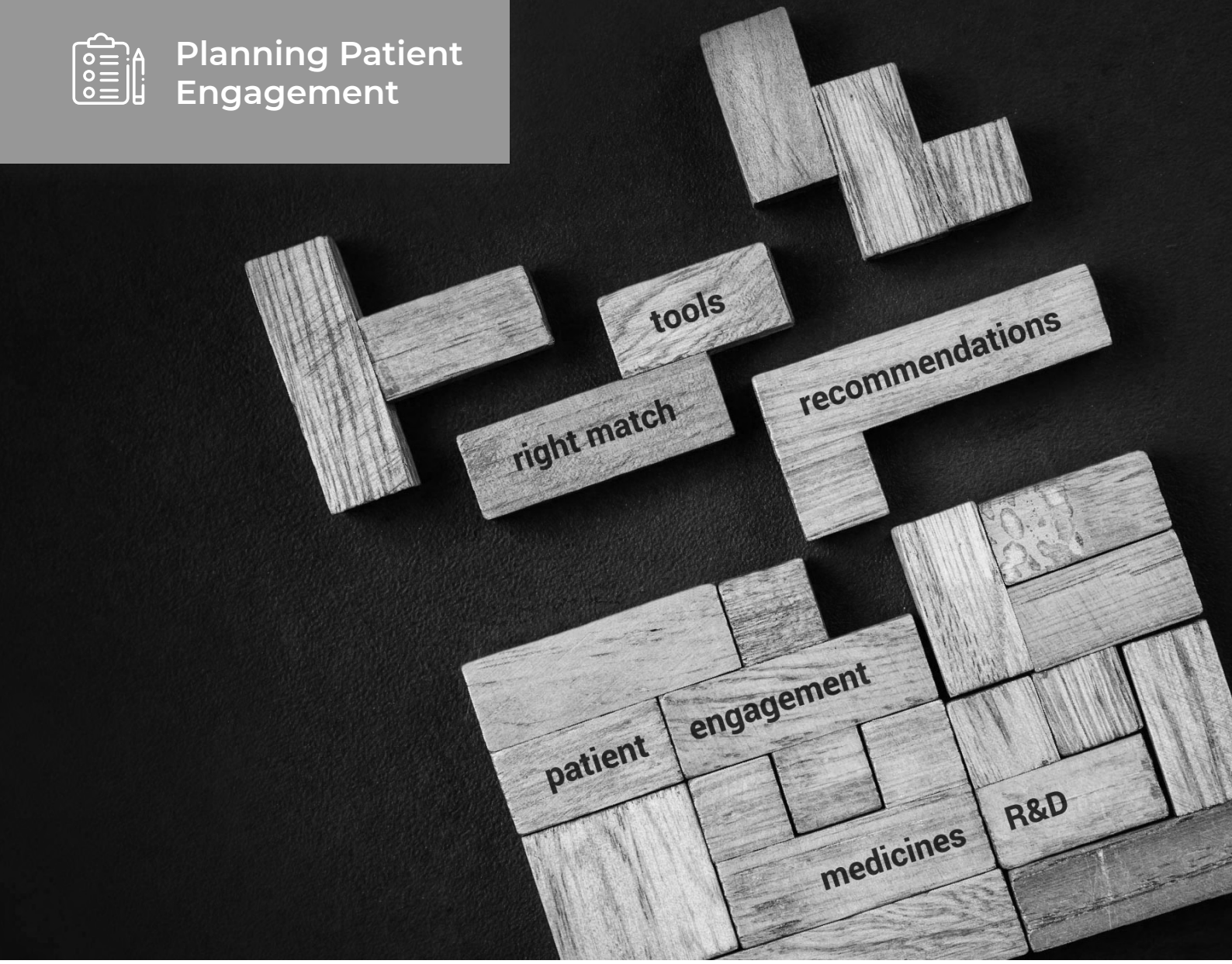
PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

innovative medicines initiative

Planning Patient Engagement



Patient engagement in medicines development:

Recommendations on how to find the right match for the right patient engagement activity

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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 work 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 document aims to provide guidance and recommendations to all stakeholders to address the gap identified about the '*selection of participants and adequate representation; clear description of the criteria followed to identify patient representatives needed*'.

During the analysis of existing practices and processes of patient engagement (PE) in the context of the PARADIGM project, a clear gap was identified under the theme 'Selection of participants and adequate representation'. This related to a description of the steps **followed to identify patient representatives between the engaging partners.**

Generally limited documentation exists on how patients and their representatives are selected prior to an engagement activity. This may be because co-design of selection criteria with the patient community is still evolving and is not yet standard practice, and this task is often managed internally within individual organisations, with published case studies of patient engagement not currently providing that information. The task may be outsourced by the pharmaceutical industry to various vendors providing patient engagement services.

This document aims to address this gap by **providing recommendations on the elements associated with identifying patients and their representatives to partner in patient engagement activities.**

This document specifically addresses what to consider when identifying the patients or their representatives' together with associated competencies to help match the right individual for the right activity.

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Key principles for the set of recommendations
To meaningfully conduct a patient engagement activity, the right matches (patients and/or their representatives) need to be identified.
The patient organisations - where they exist - are the first and key point of contact to co-create the engagement project, to avoid purely one-off activities and to start or consolidate efforts to build trust and collaboration with the patient community.
The patient organisations are key partners in the definition of the right competencies, skills and behaviours of the patients and their representatives.
A landscape analysis of the patient community helps to identify the most suitable partners to co-deliver a patient engagement activity. The aim is not to prevent any type of engagement activity, but to understand the degree of development of a patient organisation with regards to a specific activity.
Table 1 setting out competencies of patients and their representatives should be understood as a way to identify the best matches for a specific patient engagement activity. It could be also used as a self-assessment tool for patients and for patient organisations to assess the capabilities within their own organisations. When PO(s) are partnering, the identification of the patients and their representatives is the responsibility of the relevant POs and conflict of interest check should be performed by both partners.

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Recommendations

Diagram 1 below sets out the steps to follow when finding the right match for the right activity. Each step is then further explained in this section. (PO stands for patient organisation(s) and CoI for Conflict of Interest)

DIAGRAM 1: **Process steps**

STEP 1	STEP 2A	STEP 3	STEP 4A	STEP 5*	STEP 6*
By the engaging partner	By the engaging partner	By the engaging partner and the relevant PO(s)	By the relevant PO(s)	By the engaging partner and the relevant PO(s)	By the engaging partner and the relevant PO(s)
Outline of the patient engagement activity on shared purpose (scope, time, human and financial resources ...)	Identification of relevant PO to co-create the activity	Co-definiton of the competencies needed to carry out the patient engagement activity	Identification of patients or their representatives that match the needed competencies for the activity	Running the engagement activity	Measuring its impact and reporting back the the community
	or **STEP 2B**		or **STEP 4B**	*Not in the scope of these recommendations	
	By the engaging partner		By the engaging partner	**Box 1:** The identification of the patients and their rep is the sole responsibility of the relevant POs and both partners collaborate to make a final selection decision. A conflict of interest check is to be performed by the engaging partner.	
	If no PO exists, or a PO does not have the capacity, nor is interested, use alternative methods to create the activity with the patient community.		If no PO exists, or a PO does not have the capacity, nor is interested, use alternative methods to identify patients or the representatives that match the needed competencies for the activity (see box 1)		

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The recommendations in this document aim to make it easier to identify the right matches for the right patient engagement activity in medicines development, especially when initiated by the pharmaceutical and biotech companies (engaging partner), as identified in the gap analysis performed in the context of the PARADIGM project. However these recommendations can also be used by other medicine developers for patient engagement activities.

STEP 1: **Outline the patient engagement activity on shared purpose (scope, time, human and financial resources, …)**

To facilitate outreach to patients and their representatives, **the engaging partner** making the request needs to be able to structure their initial requirements:

- Outline the rationale, objective for the collaboration and describe the desired outcomes. A shared purpose[1] between the engaging partner and the patients or their representatives who will be involved in the patient engagement activity should be described.
- Describe what the activity is trying to achieve (e.g., inform decision making, build knowledge, assess if hypothesis are meaningful to the patient community, verify understanding or co-create materials to ensure patient friendly language is used, determining inclusion-exclusion criteria in a clinical trial protocol discussion or considering which endpoints are relevant for the patient).
- Describe what is expected from engaging patients, their representatives and their roles in the activity together with the time commitment required. Typically, time commitment will depend on the type of instruments used to capture the insights from patients and their representatives (see Annex 1).
- Outline interdependencies with other engagement activities planned or ongoing. Always try to limit duplication with other internal activities / projects of similar nature. Combining various activities to meet similar objectives will avoid overburdening patient organisations with numerous requests. Remember many POs rely solely or partly on volunteers, and therefore may have limited resources and capability to react to multiple requests.
- Provide best estimate on timelines including start date and deadline for results or findings from activity, however, include an indication of flexibility with dates (+/- weeks/months).

[1] A shared purpose. PFMD's Patient Engagement Quality Guidance, Available at: https://patientfocusedmedicine.org/peqg/patient-engagement-quality-guidance.pdf

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Patient identification may be the most time-consuming part of the project. This can take longer, depending on the disease under discussion, the type of activity, the capacity and capability of patients/patient organisations and other factors.

- If the engaging partner does not already have a relationship with the chosen patient/patient organisation(s), this must be established to start the process of building trust to foster future collaboration. However, it must be recognised that it will take additional time to build understanding and form a mutually beneficial relationship and this is desirable prior to specific requests for identifying the suitable match are made.
- Indicate what budget is required for the activity. The costs of the activity should be understood, and funding secured before any request is made to patients or patient organisations (or alternatives).
- Detail if the support of a facilitator (or a translator) is needed to perform the patient engagement activities. This is a requirement that we encourage to be mandatory in the case of children and young patients and other target groups where the role of caregiver is essential (e.g. elderly, people living with dementia) and for further guidance, refer to Enhancement EUPATI industry guidance: suggested working practices[2].

After the engaging partner has drafted the first version of the engagement activity for internal purposes to scope the activity, the co-creation shall begin with the support of the patient community. It appears that the POs - where they exist - are the first and key point of contact to co-create the patient engagement project, to avoid purely transactional elements and to start or consolidate efforts to build trust and collaboration efforts with the patient community.

STEP 2A & 2B: **Identify the right partner(s) -such as patient organisation(s)- to co-create the patient engagement activity**

It is important to highlight that in the case where a patient organisations does not exist, or do not have the capacity or do not wish to collaborate, then the engaging partner shall look for the perspective of the patient community through other means such as patient opinion leaders, online patient communities or, in some cases, patients with lived experience of the specific condition being discussed. The partner will vary according to the goal of engagement.

[2] Enhancement EUPATI industry guidance: suggested working practices http://imi-paradigm.eu/PEtoolbox/enhanced-eupati-guide

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The engaging partner needs to identify the appropriate (or suitable) POs to act as a partner to co-create the activity and support its implementation, by performing a landscape analysis - linked to the activity purposes - of the community that could integrate, as example, the following aspects[3]:

(The list below does not intend to capture the entire set of elements of a complete landscape analysis)

- **Overview of the community**
  - Number of patient organisations in different countries
  - International/regional umbrella organisations
  - Patient opinion leaders (both patient organisations leaders or online bloggers)
- **What is specific about the patient community?**
  - Core activities
  - Connectivity
  - Capacity and influence
  - Engagement with industry
  - Strategic partnership opportunities and areas on which to focus to create the biggest mutual value
  - Potential challenges faced in engaging with the community

However, it is clear that not all patient organisations have the same degree of development for a given activity, as this may depend on the activities they carry out on a daily basis (e.g. patient and caregiver support vs policy activities) or the main operational focus of the organisation (e.g. multi-stakeholder collaborations vs medical expertise).

Therefore, finding the appropriate partner in this early phase of the activity is important. Diagram 2 below is an example of potential assessment criteria that could be used to identify potential partners and assess the most suitable POs.

[3] Adapted from Roche's and Novartis' internal materials

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DIAGRAM 2: **Recommended topics to consider about the POs that could guide the decision about which are the best suitable POs to contact steps**

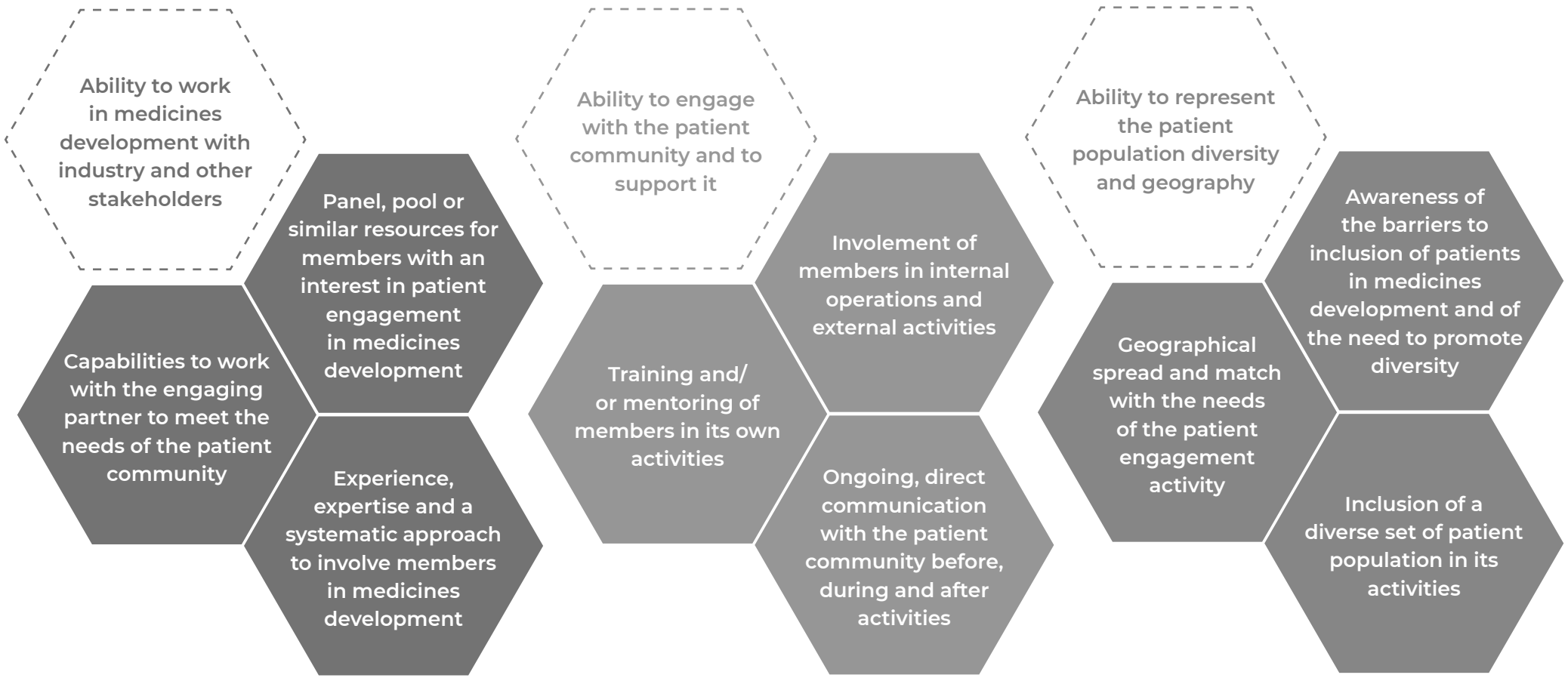

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Table 1: **Check list of the elements to consider before engaging with a patient organisation to work on a patient engagement activity**

Elements to consider before engaging with a patient organisation to work on a patient engagement acticity	Yes/No/Partially	Comments
Does the organisation have the experience, expertise and a methodology or a systematic approach about how to involve their members in medicines development?		
Does the organisation have the capabilities to work with the engaging partner to ensure that the patient engagement activity is suited to the needs of the patient community?		
Does the organisation have a panel, pool or similar resources which includes their members with an interest in patient engagement in medicines development?		
Does the organisation have well-established channels of communications with their members and beyond with the patient community at large?		
Does the organisation involve their members in their own internal operations and external activities?		
Does the organisation have direct contact with their members and the track record of mobilising them?		
Does the organisation have the resources to train and/or mentor their members in its own activities?		
Does the organisation have the geographical coverage that matches the needs of the patient engagement activity?		

Does the organisation address the inclusion of a diverse set of the patient population in its own activities?		
Does the organisation demonstrate an awareness of barriers to inclusion and of theneed to promote diversity in the context of patient engagement in medicines development		

Once the landscaping exercise is performed, the different approaches available to reach out to the identified potential partnering POs are via:

- Umbrella or disease specific organisation (international, regional, national)
- Databases[4], either open access (e.g. Orphanet[5], PFMD's Synapse[6]) or proprietary (e.g. EMA individual experts' stakeholder database)
- eYPAGnet[7] to engage children and young people through the YPAGs (young person's advocacy groups).
- Local company affiliates (or the headquarters when the engagement activity started)
- Search engines (such as Google)
- Alternatives:
  - Alumni of training programmes such as EUPATI (with the limitation that only EUPATI fellows and patients in the EUPATI network can be identified as right match in this way)
  - Investigators and hospitals who are conducting research with and for patients
  - Vendors providing patient engagement services that as part of their portfolio may include support to identify POs willing to engage in medicines R&D activities.
  - Country based services provided by public sector organisations for example, the National Institute for Health Research (NIHR) Patient Engagement in Clinical Development service

It is recommended that outreach to various groups and communities happen in parallel, rather than in sequence, thus avoiding tight timelines and mitigates the risk that patient identification is delayed. Nevertheless, it is important to have anticipated and put in place mitigation measures if and when there are more POs and/or individuals willing to engage. The outreach activity to identify patients and their representatives should be coherent with the engaging partner's capacity to effectively carry on with the activity. Throughout the process, the engaging partner need to continuously assess what progress is being made during the outreach phase and adjust the approach as appropriate in consultation with the partner PO or alternatives.

It is recommended both the engaging partners to work together to increase the PO's capability

[4] IMI PARADIGM's Recommendations on the required capabilities for patient engagement More information on the Table 8, Available at: http://imi-paradigm.eu/PEtoolbox/pe-capabilities

[5] Orphanet, Available at: https://orpha.net

[6] PFMD's Synapse, Available at: https://synapse.pfmd.org/

[7] This network has the recognition of EnprEMA (European Networks for Paediatric Research of EMA), Available at: https://www.eypagnet.eu/

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to support future activities. The capability building shall be initiated and organised by the patient community and medicine developers can financially support these activities, following existing guidance documents [8,9,10,11,12]. More information about the capabilities needed to conduct meaningful patient engagement activities for all stakeholders can be found in the IMI PARADIGM's D4.1 Recommendations on the required capabilities for patient engagement[13].

When conditions exist to prevent co-creation, due to the nature of the activity, external regulations or the capacities of the POs, the patient community may be involved with less integrated approaches at the most appropriate level and this should be decided between the patient community and the engaging partner.

STEP 3: **Co-define the competencies for the patient engagement activity**

As described above, patient organisations are usually recommended as a key partner in a patient engagement and should be involved in the co-creation of the PE activity. **The engaging partner and the relevant patient organisation** should work to drive the definition of the skills, behaviors and competencies of the patients and their representatives who will engage in the activity. More information can be found in the IMI PARADIGM's D4.1 Recommendations on the required capabilities for patient engagement[14].

Table 2 below can be used as a tool for individuals to self-assess their own competencies, as well as during the co-design phase to identify the level of competencies required for the activity (between 0 and 3 as per the definition in third column). The scoring can be done between 0 and 3 where #0 means that the competence is not needed and #3 highly desirable.

[8] EPFIA's Working together with patient groups, Available at: https://www.efpia.eu/media/412524/working-together-with-patient-groups-23102017.pdf

[9] EFPIA's Code of practices, Available at: https://www.efpia.eu/media/413022/efpia-code-2019.pdf

[10] EURORDIS Charter for Collaboration in Clinical Research in Rare Diseases, Available at: http://download2.eurordis.org.s3-eu-west-1.amazonaws.com/clinical_trials/charter-for-collaboration-in-clinical-research.pdf

[11] EUPATI's Guidance documents on patient involvement in R&D, Available at: https://www.eupati.eu/guidance-patient-involvement/

[12] IMI PARADIGM's Short Guidance 'General and stakeholder-specific considerations to manage competing interests and conflicts of interest', Available at: http://imi-paradigm.eu/PEtoolbox/conflict-of-interest

[13] IMI PARADIGM's Recommendations on the required capabilities for patient engagement More information on the Table 8, Available at: http://imi-paradigm.eu/PEtoolbox/pe-capabilities

[14] IMI PARADIGM's Recommendations on the required capabilities for patient engagement, Available at: http://imi-paradigm.eu/PEtoolbox/pe-capabilities

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An individual's willingness or ability to be transparent plus act with ethical integrity are important personal characteristics during patient engagement activities. The level of transparency someone needs to provide will depend on the different levels of engagement and type of activity. At the intermediate level, the declaration of interests in context of the activity is to be provided. A complete declaration of all interests, (irrespective of whether they are related to the activity or not) is to be provided when a high level of competency is needed.

The competency table is a key tool to identify the main attributes that would be the most suitable for a given patient engagement activity. Inclusivity of the different perspectives should be sought, in order to capture best the insights from the patient community. The engaging partner and the POs should work together to identify suitable matches, and ensure that there is no discrimination between populations.

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Table 2: **Competency table** [15]

Competencies		Definition	1-Low	2-Intermediate	3-High	Patient Engagement Activity	
						Co-designing the project	Patricipating in the project
Medical expertise	**Basic medical expertise**	Medical expertise, anatomic and psychological expertise; knowledge about medicines research and development process	Basic medical expertise (anatomy, physiology, use of treatments)	Extended medical expertise (medicines development, medical methodology)	In-depth medical knowledge in all aspects		
	Indication specific experetise	Knowledge about the indication/disease, treatments, care and life circumstances of those living with the disease	Basic indication, treatment and care expertise	Extended indication, treatment and care expertise	In-depth indication, treatment and care expertise incl. latest research results and treatment expertise		
System expertise	**Regulatory expertise**	Knowledge about regulatory processes, e.g. evaluation, authorization, reimbursement processes of therapies	Basic knowledge about the medicines approval process, related assessments and reimbursement procedures	Extended medical expertise (medicines development, medical methodology)	In-depth medical knowledge in all aspects		
	Public Health expertise	Knowledge about access and participation in the healthcare system (e.g. social law), knowledge about health policy	Basic knowledge about accessing and participating in the healthcare system	Extended indication, treatment and care expertise	In-depth indication, treatment and care expertise incl. latest research results and treatment expertise		
Methodological expertise	**Communication and representation**	Well-structured and solution-oriented communication skills (including digital and social media), and expertise to advocate appropiately for a patient community	Objective commmunication and appropiate conduct	Clear and focused communication with sound and consistent opinion	High level skills in communication, moderation and group interaction		

[15] Competency table adapted from EUPATI Germany by Geissler J, Bereczky T, Dierks ML, Schumacher-Wulf E, Schmitt C and Claussen C (2019/2020,unpublished)

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Table 2: **Competency table** [15]

Competencies		Definition	1-Low	2-Intermediate	3-High	Patient Engagement Activity	
						Co-designing the project	Patricipating in the project
Methodological expertise	**Negotiation skills and political interaction**	Expertise in political interaction, negotiation skills	No specific expertise in political interaction	Personal integrity and good negotiation skills	Sensible policy interaction and communication, strong negotiation skills and consistent positions		
Personal framework	**Personal experience**	Indication-specific expertise based on personal experience	Empathic relation to those affected	Affected indirectly (e.g. family member)	Directly affected by the condition		
	Community insight and involvement	Involvement in the patient community in a specific area.Ability to abstract from personal experience to represent a wider community	Direct interaction with other people living with the disease	Broad insights of different needs of a specific patient community. Frequent interaction with different community members	Structured approach on processes and decision making when interacting with people across a specific community. Ability to represent a community.		
	Capability to perform a task	Financial means or support to allow to perform a specific role; availability to perform specific tasks; Sufficient physical fitness	Ability to engage on a specific task within a strict time and effort limit	Ability to undertake continuous, recurrent interaction	Ability to carry out more complex, long-term tasks requiring a considerable commitment (efforts, time and health wise)		

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Addressing the patient community diversity

In this document, 'patients and their representatives' is understood as described in the EUPATI guidance for patient involvement in industry-led medicines development[16] (individual patients, carers, patient advocates, patient organisation representatives and patient experts). It is paramount that more than one patient is involved, and that the diversity of patient populations is reflected in the activity. This ensures the balance of opinions and views, and optimises the opportunity to match the methods (see Annex 1) to be used in the engagement. It is recognised that in some rare diseases this will be challenging if not impossible, so a pragmatic approach is key. This can also apply in the case of other conditions such as individuals living with dementia, where due to stigma, late diagnosis and negative impact of the disease and so on in some countries, it may not be feasible to find a sufficient number of patients willing to take part in patient engagement activities. Therefore, it is recommended that efforts should be made to include several patients, as far as the availability of interested patients allows. In other words, whenever possible, patient engagement activities should not rely on only one individual.

In order to capture the richness of the insights, seeking the support of 'mentors' (individuals more experienced in patient engagement activities) who could be a reference for newcomers and ensure continuity of knowledge sharing is advised. Where appropriate for the project for which a PE activity is planned, efforts should be made to gather and include the views from specialised or stigmatised groups, pregnant women, individuals with different social-economic backgrounds, different age, gender, stages of disease, cultural, religious backgrounds and beliefs.

When country specific insights are required, specific considerations should be given to provide a balanced representation of countries, languages and cultures, as there may be regional differences worth reflecting in the patient engagement activity.

More information can be found in the PFMD's Patient Engagement Quality guidance[17], in the IMI PARADIGM's D4.1 Recommendations on the required capabilities for patient engagement[18] and the National Health Council's Roadmap and Rubric[19].

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[16] EUPATI guidance for patient involvement in industry-led medicines R&D, Available at: https://www.eupati.eu/patient-involvement/guidance-for-patient-involvement-in-industry-led-medicines-rd/

[17] https://patientfocusedmedicine.org/the-patient-engagement-quality-guidance/ Section 3 on Representativeness of stakeholders

[18] Recommendations on the required capabilities for patient engagement http://imi-paradigm.eu/PEtoolbox/pe-capabilities

[19] https://www.nationalhealthcouncil.org/wp-content/uploads/2019/12/Representativeness%20in%20Patient%20Engagement.pdf

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STEP 4A & 4B: **Identification of patients or their representatives that match the needed competencies for the activity**

In a member-based PO, qualifying and understanding the membership through development and integration of software that can record the self-assessment of interest, the availability of individuals and their competencies for future engagement with any type of activity could be considered, with the deployment of Customer Relationship Management[20] type software.

This process could be supported by patient engagement managers in patient organisations. The IMI PARADIGM's D4.1 Recommendations on the required capabilities for patient engagement[21] states that among others that 'patient engagement 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 patient engagement activity.**
- [...]

Patient engagement functions in both industry and POs can be organised in a number of models (e.g. by types of activities) in which the engagement of patients is requested (e.g. clinical trial design) or by medical areas (i.e. by disease areas).'

Alternatively, patient engagement managers (or or related functions) in POs could use the outreach power of social media or online communities to identify and recruit patients or their representatives to take part in engagement activities.

Following co-creation of the patient engagement activity and the definition of key criteria (such as competencies, health status, level of availability, language skills, etc.) for the right matches, it is the sole responsibility of the relevant POs to identify the patients and their representatives and make a final selection decision based on the results of the identification process (refer to key principles).

A conflict of interest is to be performed by the engaging partner against related criteria as laid out in the IMI PARADIGM's Raising awareness on managing competing interests in a multi-stakeholder environment: Guidance to patients and engaging stakeholders[22].

[20] Customer Relationship Management, Available at: https://en.wikipedia.org/wiki/Customer_relationship_management

[21] Section 3 on Representativeness of stakeholders, Available at: https://patientfocusedmedicine.org/the-patient-engagement-quality-guidance/

[22] Tools for the management of competing interests and conflicts of interest. http://imi-paradigm.eu/PEtoolbox/conflict-of-interest

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If the patient organisation(s) are not successful with patient identification then the engaging partner may find alternatives, see step 2B. Success can and must only be defined at the co-creation phase between the partners and is best judged after the completion of the activity.

STEP 5 & 6: **Run the activity, measure its impact and communicate its outcomes of the activity to the partners and beyond**

As a reminder, the steps involved in running the patient engagement activity is not within the scope of the document. However, the type of instruments and short description on how and when to best use them to capture insights from patients and their representatives during activities are provided in Annex 1.

Measuring the 'return on engagement' of an activity is a key milestone that can be captured using the IMI PARADIGM's Monitoring and Evaluation framework[23].

The communication of the results of the identification and 'recruitment' of patients and their representatives to co-create and subsequently participate in the patient engagement activity can be reported and communicated to the patient community using the IMI PARADIGM's templates for patient engagement reporting and dissemination[24].

After the co-creation of the patient engagement project and the definition of key criteria (such as competencies, health status, level of availability, language skills,...) for the right matches, the final selection decision of patients based on the results of the identification process, can only be vetted by the engaging partner on conflict of interest reasons.

[23] IMI PARADIGM's Monitoring and Evaluation framework, Available at: http://imi-paradigm.eu/PEtoolbox/monitoring-evaluation

[24] Guidance for Reporting and Dissemination of Patient Engagement Activities, Available at: http://imi-paradigm.eu/PEtoolbox/reporting-and-dissemination

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Limitations

In conducting patient engagement activities, there are several steps that other global initiatives (such as PFMD's Patient Engagement Management (PEM) Suite[25], EUPATI's guidance documents[26] or the National Health Council's resources[27]) are covering, and therefore the 'how' to conduct a patient engagement activity is not in the scope of this document. It may be useful for patient organisations (POs) to consider their own competencies for a specific collaboration or developmental needs. Whilst important, it is also not in the scope of this document. Patients and POs may consider several elements before partnering with an engaging partner as laid out in the ABPI Sourcebook for industry working with POs and patients[28] or in the CRIG guidance for charities on working with industry [29].

[25] https://patientfocusedmedicine.org/pem-suite/

[26] https://www.eupati.eu/guidance-patient-involvement/

[27] https://nationalhealthcouncil.org/issue/patient-engagement/

[28] ABPI Sourcebook for industry on working with POs and patients https://www.abpi.org.uk/publications/working-with-patients-and-patient-organisations-asourcebook-for-industry/

[29] CRIG guidance for charities on working with industry http://slginvolvement.org.uk/wp-content/uploads/2019/09/Supporting-PPI-in-industry-ledresearch_Guidance-for-Charities_CRIG-HRCI-public.pdf

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Annex 1: **Methods and short description on how and when to best use them to capture insights from patients and their representatives**[30,31]

As described in the Recommendation section, the instruments to elicit the insights from patients or their representatives may, based on their specifics, have an impact on the patient matching and selection process. This may extend to the number of patients required to meaningfully capture a range of perspectives or on the competencies needed. Therefore, in this annex, you will find short descriptions of the most commonly used instruments.

In the description of the instruments below, the word 'data' should be understood as inputs, insights, contributions or opinions, and not clinical study data.

Focus groups

- A qualitative group discussion between participants (patients or representatives), which is typically conducted in-person and facilitated by a moderator with special expertise (e.g. researcher, psychologist). Focus groups can promote synergy by encouraging participants to comment, explain, disagree, and share their views. Information obtained from qualitative interviews through focus groups can help to understand the disease/treatments (e.g. pre-study experience, or development of patient journey) and inform the clinical development program (e.g. patient preferences, treatment expectations or interpretation of outcomes) or clinical outcomes assessment (COA) strategy (e.g. concept elicitation).
- Additionally, focus group interviews can enhance patient engagement in clinical trials or support the identification of additional needs for external stakeholders.
- While not generally as resource intensive as conducting one-to-one patient interviews, similar guidance to optimize research objectives and execution versus potential activity constraints is recommended.
- This patient engagement tool is most useful when a consensus is sought.
- Key considerations for focus groups may include research objectives, topic complexity, topic sensitivity (e.g. sexual health, mental illness, etc. are generally more sensitive topics not conducive to group settings), group size/number of participants per group, group diversity/

[30] Adapted from Novartis internal material

[31] Adapted from Transcelerate's Patient Protocol Engagement Toolkit (P-PET) User Guide
https://transceleratebiopharmainc.com/wp-content/uploads/2019/07/TransCelerate_P-PET-User-Guide_Version-1.pdf

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heterogeneity, and number of focus groups needed.

- Focus groups can be conducted to identify potential questions for survey development.

Qualitative interviews

- A semi-structured conversation between a patient their patient representatives and interviewer to collect rich, in-depth qualitative data related to the research question.
- This may include discussion about a patient's disease experiences, or their attitudes/perceptions towards treatment and its impact. This method is often used to develop patient-reported outcomes measures (PROMs) or to gain qualitative insights early in a clinical development program or later in the product lifecycle when gaps in knowledge emerge.
- Information obtained from qualitative interviews can help to understand the disease/treatments and inform the clinical development program or clinical outcomes assessment (COA) strategy.
- Additionally, qualitative interviews can support development decision points, enhance patient engagement with clinical trials or support data needs for external stakeholders.
- Qualitative interviews are of most value when rich, in-depth qualitative data are needed as direct evidence from patients, such as for regulatory and (certain) health technology assessment (HTA) submissions, or to provide feedback on the patient experience of clinical development programs.
- This type of primary patient research can be resource intensive, particularly in terms of costs and timelines. Careful consideration is needed to optimize for research objectives and any applicable resource constraints. It is recommended to execute due diligence in advance to identify potential existing data and information that may address research needs and to assess whether these data can be supplemented with other patient engagement tools (e.g. literature search).

Online patient surveys

- Online Patient Surveys are completed by patients or their representatives and can be used to collect quantitative data (discrete choices) or qualitative data (free-text answer boxes). Unlike bulletin boards, patients complete the survey independently, and do not interact with one another.
- Patient surveys can capture patient insights around the disease. The topic for the patient survey will be chosen according to the activity needs and can assess the unmet needs of patients.

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- Different target product profiles (TPPs) and value of specific attributes can be tested.
- Surveys can also capture clinical trial-related insights, such as general clinical trial awareness/motivation, patient input on specific protocol-related questions, or confirming understanding of the patient experience.
- Patient surveys are most valuable to assess stakeholder attitudes/perceptions and quantify disease burden/unmet needs; such surveys methods are indispensable when large datasets are required for quantitative analysis or when patient populations are geographically diverse.
- Cross-sectional surveys of large populations could be quick and economical while longitudinal initiatives may be prone to retention bias and higher budget.

Patient advisory panels (PAP)

- Consists of patient experts (patients, patient advocates and/or caregivers) who are convened to bring unique knowledge to inform teams regarding the patient perspective.
- PAPs can provide the patient perspective on a wide range of topics, including:
  - disease understanding
  - unmet medical needs
  - research needs prioritization
  - insights on the patient journey
  - key issues that patients are facing
  - advice on key development and clinical trial questions
  - review of product/value messages and patient-facing materials
  - psycho-societal and healthcare system challenges patients are facing
  - the role of patient organizations in providing support
  - define potential areas of collaboration with the patient communities.
- PABs may be used in combination with the other patient engagement tactics to facilitate the most appropriate data collection from patients.
- This patient engagement tool should be considered when patient-centric methods are needed to inform trial design, review product/value messages and patient-facing material and advise on appropriate patient involvement. This tool places patients as strategic advisors rather than research participants. It may be used in combination with other patient engagement tools to facilitate the most appropriate data collection from patients.

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- A longer-term alternative is patient councils, which are engagements over a specific period of time with a group of patient advisors. For example, a patient council might engage with a leadership team to provide the patient perspective on policy issues on a regular basis over the course of two or three years.

Community advisory board (CAB)

In short, Community Advisory Boards (CABs) are an innovative concept, developed some decades ago in the United States and more recently in Europe, to establish long-term relationships between the patient community and industry in order to encourage patient engagement and input in the medicines research and development lifecycle. For more information, see Community Advisory Board - Guidance document and templates[32] on the PARADIGM Patient Engagement Toolbox.

Holding public meetings

- Offers the opportunity to tailor a meeting with patients, their representatives, patient advocates, and a broader representative panel of decision-makers including clinicians, scientists, health authorities, and payers, to address specific objectives.
- Opportunity to support additional Patient-Focused Drug Development (PFDD) meetings. Other than U.S. Food & Drug Administration (FDA)-related PFDD meetings, there may also be health technology assessment (HTA) public forums, commissioning board public meetings involving patients, or similar, in your region of interest.
- Multi-stakeholder meetings that collect input from patients and patient representatives about the burden of disease and patient views on existing treatment options or might focus on topics beyond clinical development or regulatory context (e.g. policy/advocacy, digitalization & technology).
- Holding public meetings can be costly, and the objectives should be carefully considered to obtain the desired information. In contrast to passive searching, they allow to participate in agenda-setting. In contrast to other active tools, they benefit from an official, structured, recognized setting (e.g. by participation of the FDA).

Social media listening (SML)

- Analyzing online interactions among patients and their representatives, caregivers and healthcare professionals (HCPs), which provides insights related to patients' unmet needs,

[32] IMI PARADIGM's document 'Community Advisory Board - Guidance document and templates', Available at: http://imi-paradigm.eu/PEtoolbox/community-advisory-boards

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experiences with their disease, and behavioral, attitudinal and emotional constructs of patients at each stage of their journey. SML is also important to understand the words, language and terminology used by patients when talking about their disease with others.

- SML can offer an understanding of the patient experience with a specific disease state, such as disease burden, emotional and attitudinal lenses, disease sentiment and vernacular, treatment experiences and unmet needs, preferences or adverse events, and economic information (out-of-pocket expenses). They can also provide information on peripheral factors affecting patient decisions to switch therapy, customer/patient and HCP influencers, awareness of clinical trials (to inform clinical trial strategy for early drug development), or competitor-related insights.
- SML insights can be used to understand patient and caregiver unmet needs, design patient support services, track sentiment relating to a brand, or design patient-facing marketing materials to resonate with actual patient experience, where permitted. In the regulatory context (developing patient-reported outcomes [PROs]) formal interviews will always be necessary, but generally SML may bring fresh insights and help generate hypotheses for further research.

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Annex 2: **References**

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European Patients' Academy on Therapeutic Innovation (EUPATI)

https://eupati.eu/about-us/ focuses on education and training to increase the capacity and capability of patients and patient representatives to understand and meaningfully contribute to medicines research and development (development) and to improve the availability of objective, reliable, patient-friendly medical information for the public.

Patient Focused Medicines Development (PFMD)

https://patientfocusedmedicine.org/about-pfmd/ aims to transform the way in which we understand, engage, and partner with patients globally in the design and development of research and medicines by focusing on unmet patient needs.

National Health Council (NHC)

https://nationalhealthcouncil.org/, created by and for patient organizations 100 years ago, brings diverse organizations together to forge consensus and drive patient-centered health policy in the US…

Orphanet

https://www.orpha.net/consor/cgi-bin/index.php organization to gather scarce knowledge on rare diseases so as to improve the diagnosis, care and treatment of patients with rare diseases.

Synapse

https://synapse.pfmd.org/tool for managing PE initiatives and exploring the ecosystem, with people and related resources, organizations, events and initiatives in one place.

The European Young Person's Advisory Group Network (eYPAGnet)

https://www.eypagnet.eu/ provides a platform for children and young persons to have a voice within Europe and provide their opinions, and experience to a variety of issues in clinical trials, such as relevant end points, protocol design, formulations, age appropriate information and patient tools.

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Annex 3: **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)

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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)

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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)

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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)

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