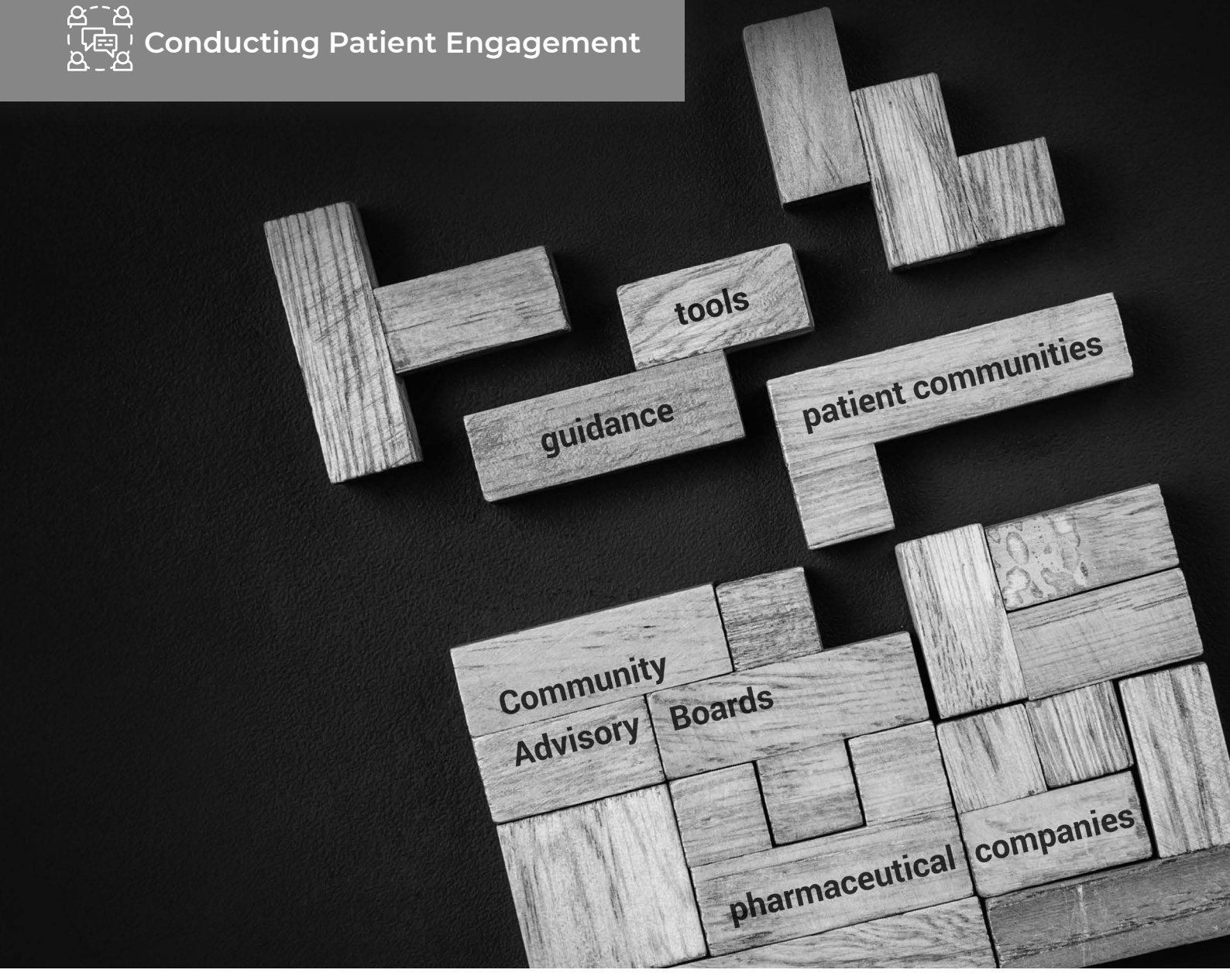
PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

innovative medicines initiative

Conducting Patient Engagement



Working with Community Advisory Boards:

Guidance and tools for patient communities and pharmaceutical companies

PARADIGM is receiving funding from the Innovative Medicines Initiative Joint Undertaking 2. This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA

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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 toolkit?

The PARADIGM toolkit on Community Advisory Boards (CABs) contains 8 different tools. It is intended for patient communities and pharmaceutical companies interested in setting up, running or collaborating with CABs.

Tool 1: **"Guidance"**

This tool contains three different sections. Readers can consult the entire guidance document as a general guiding tool for CABs or use the separate sections according to their needs and specific focus in engaging with patient communities. The main information presented in each section includes:

1.1. "Basic information about CABs"

- This section provides a general description of CABs and information about some of the existing CABs and related programmes in Europe. It focusses on the work of three patient communities in Europe (i.e. HIV, rare diseases and oncology) and includes brief information about different CABs within these patient communities (e.g. two examples of CABs on HIV, three on oncology and seven within a CAB related-programme for rare diseases). One of the examples (ECAT), is specific to Eastern Europe and Central Asia (EECA region).

- It is targeted at patient communities, industry and anyone with a general interest in CABs.

1.2. "Points to be considered by patient communities when setting up or running a CAB"

- This section includes six relevant topics to consider for setting up and running a CAB. For each of the topics, practical information is provided about how existing CABs are addressing the topic. One example of an existing CAB from each of the three patient communities (HIV, rare diseases and oncology) is presented for each topic.

  By providing different examples and approaches from different patient communities, it is hoped that it can help other patient communities in developing and finding their own ways of working considering the needs of their community and capabilities. It does not provide recommendations or prescriptive guidelines about how to establish or run a CAB.
- It is targeted at patient communities which may want to establish or are already running a CAB.

1.3. "Points to be considered by pharmaceutical companies collaborating with CABs"

- This section provides information and relevant points to consider, from the perspective of industry, when invited to collaborate with a CAB.
- It is targeted at representatives from the industry who are already collaborating or would like to start a collaboration with CABs.

Tool 2: **CABs "At a Glance"**

This tool is a brief summary ("a one-pager") which provides a high-level overview of how a CAB operates.

Tool 3: **"Comparative table for three existing CABs"**

This tool is a table providing information about three different approaches for setting up or running a CAB. The examples included are ECAB (HIV/AIDS), CML CAB (oncology) and EuroCAB programme (rare diseases).

Tool 4: **"Checklist of tools and resources"**

This tool includes a list of templates and documents which can be useful when working with CABs. It also includes information about where it may be possible to find examples of the templates/ documents.

Tool 5: **"Reflective questions and tracking tool"**

This tool contains a set of "reflective questions" and a "tracking table". It aims to stimulate reflection about different aspects to consider when setting up/running or collaborating with a Community Advisory Board (CAB) from the perspective of the different stakeholders involved. It can be used in combination with Tool 1 and Tool 3.

Tool 6: **"Value-adding factors of a CAB from a pharmaceutical company perspective"**

While CABs have been established to encourage patient engagement and input in the medicine research and development lifecycle, it is clear that they offer a unique tool to have input in the development process for a company. This tool gives an overview of how and what CABs can deliver.

Tool 7: **"Practical briefing guidance for industry"**

This tool refers to what company representatives need to consider and how to get prepared for participating in a CAB meeting.

Tool 8: **Examples of successful outcomes of CABs and industry interactions**

This tool describes four case examples of how CABs have been instrumental to provide timely patient input to change the course of the studies leading to successful outcomes.

Limitations

This toolkit focuses on the work of CABs which are initiated and driven by the patient community and collaborate with industry. However, it has to be considered that other types of CABs (e.g. set up by researchers or academia or not in the scope of medicines' development and access) and other types of collaboration between industry and patients (e.g. industry-initiated advisory boards, steering committees, councils, panels) exist.

This toolkit is intended to be a set of instruments for the initiation and development of CABs derived from the experience of two European CABs and one CAB programme. This not an exhaustive list of resources. Readers are invited to consult additional material that might have been developed by CABs run by other patient groups.

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.

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.

(Wikipedia 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.

(HTA glossary 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: https://toolbox.eupati.eu/resources/making-a-medicine-step-7-phase-ii-proof-of-concept/ European Commission: https://ec.europa.eu/competition/sectors/pharmaceuticals/cycle.html EFPIA: https://www.efpia.eu/about-medicines/ Frontiers 'The Life Cycle of Health Technologies. Challenges and Ways Forward, Iñaki Gutiérrez-Ibarluzea et. al. 2017' https://www.frontiersin.org/articles/10.3389/fphar.2017.00014/full)

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. (Investopedia 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) https://www.frontiersin.org/articles/10.3389/fmed.2018.00270/full)

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 Community Advisory Boards

CAB member

A person living with or affected by a medical condition or representing the interest of people living or affected by a medical condition. In this context, we refer to persons with an interest in research and medicines development, who have joined a Community Advisory Board following the appropriate process.

CAB chair

Member elected for a period of time to guide, supervise and govern the CAB activities for a determined period.

Eastern Europe and Central Asia

Different sources define borders of the EECA Region in different ways. In this document we refer to the geographical scope of WHO Europe including 17 countries as being part of the EECA Region.

Pharmaceutical company

A commercial business licensed to research, develop, market and/or distribute medicines, most commonly in the context of healthcare.

Representative of industry

For the purpose of this document, it is a person designated by pharmaceutical company to liaise with the CAB and/or to attend the CAB meetings.