Patient engagement & projects

EURORDIS – Rare Diseases Europe's experience

Open Academy – Joint session

Thursday 5th June 2025 - Barcelona

Virginie Hivert – EURORDIS Head of Therapies & Access

Our mission

EURORDIS works across borders and diseases to improve the lives of all people living with rare diseases.

OV
ER
1000

MEMBER PATIENT
ORGANISATIONS

7
COUNTRIES
(27 EU
5
4
COUNTRIES)
NATIONAL ALLIANCES OF
RARE DISEASE
7
7
EUROPEAN &
PATIENT ORGANISATIONS
INTERNATIONAL
FEDERATIONS
7
OF SPECIFIC RARE DISEASES

199
FOUND
ED IN
7

6
MILLION
euro
BUDGET

Outreach to
over
2500

PATIENT
GROUPS

OV
ER
65

VOLUNTEER PATIENT
ADVOCATES

40

TEAM MEMBERS,
WITH OFFICES IN
PARIS, BRUSSELS,
BARCELONA

90’s – The roots of patient activism – the HIV outbreak

- When talking about patient engagement in medicines life-cycle, we must remember that major principles have been established by the **HIV community**
- These basis (e.g., ethical principles), as well as some methodologies developed at the time are still in use nowadays such as the **Community Advisory Boards (CABs)**
- '*CABs were formed by people living with HIV to help shape and consult on clinical research. This could be considered as the beginning of AIDS treatment activism*'
- First delegation of patients to the European Medicines Agency for a **dialogue with regulators in 1996**

2000 – Launch of the Orphan Regulation

- **Rare diseases** – Pioneers of patient involvement in regulatory advice and decision-making
- Regulation (EC) No 141/2000 laying down the creation of the **COMP** - First committee with patients as **full Members** (3 seats for Patient representatives)
- Followed by patients included in EMA Management Board 2005, WG with Patients & Consumers / PCWP (2003 /2006), PDCO (2007 – 3+3), CAT (2008 – 2+2) and PRAC (2012 – 1+1)
- **2005:** Patients included in Scientific Advice discussions with sponsors for orphan medicines (**Protocol Assistance**) – extended to all medicines in 2013
- More and more **EMA activities** including patient input (Review of Orphan Summaries from 2002, Patient leaflets from 2007, CHMP Oral explanations from 2014, First public hearing in 2017, CHMP early dialogue with patient and healthcare professional organisations, etc)

2008 – First edition of the EURORDIS Summer School

- COMP first Chair (Josep Torrent-Farnell) and first vice-Chair (Yann Le Cam, EURORDIS CEO) – together they created the **EURORDIS Summer School** for Patient representatives
- More than 800 patients trained to Medicine R&D since then
- **2014** - first time we had researchers at the Summer School
- Partners in IMI EUPATI for all diseases → Foundation
- In parallel, evolution of the Summer School into EURORDIS **Open Academy** with Winter School for translational research, Digital School, etc
- **Capacity building programmes – EJP-RD/ERDERA**
- **Open Academy:** MRD (Medicines R&D) and SITR (Scientific Innovation & Translational Research)

2018 – EURORDIS Black Pearl Awards given to EFPIA & companies having committed to IMI PARADIGM

PARADIGM is a public-private partnership and is co-led by the European Patients' Forum and EFPIA.

PARADIGM's mission is to provide a unique framework that enables structured, effective, meaningful, ethical, innovative, and sustainable patient engagement (PE) and demonstrates the 'return on the engagement' for all players.

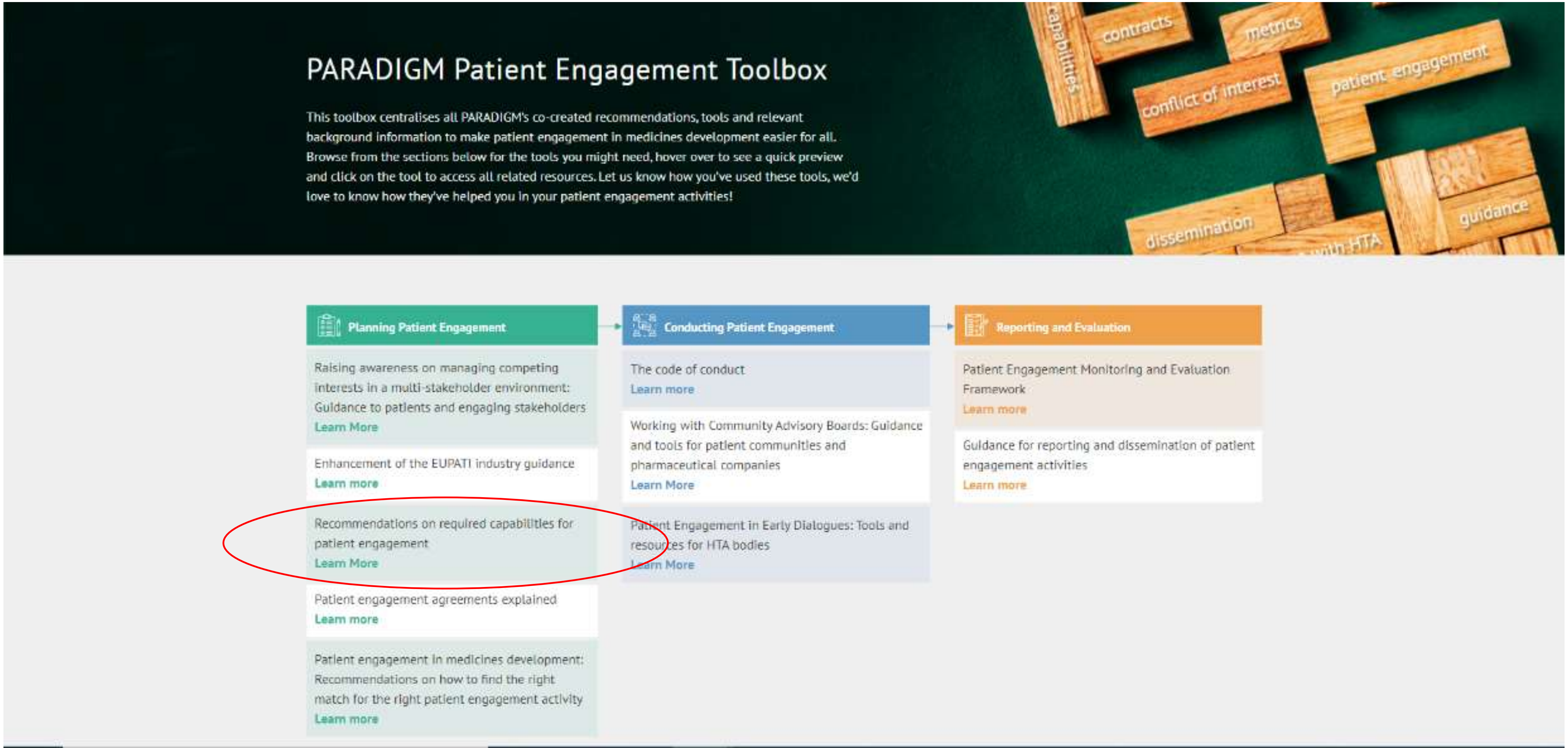
The objective is to develop much needed processes and tools for three key decision-making points: **research priority setting, design of clinical trials and early dialogue**. Building on advances at international level, PARADIGM will integrate the needs, perspectives and expectations of all actors (including vulnerable populations) involved and will also produce a set of metrics to measure the impact of patient engagement.

Advancing meaningful patient engagement in the life cycle of medicines for better health outcomes.

PARADIGM Patient engagement toolbox – Recommendations/How-to do PE & Managing competing interests

- Research & priorities setting
- Design of clinical trials
- Early dialogues with regulators and HTA bodies

Recommendations on required capabilities for patient engagement



PARADIGM Patient Engagement Toolbox

This toolbox centralises all PARADIGM's co-created recommendations, tools and relevant background information to make patient engagement in medicines development easier for all. Browse from the sections below for the tools you might need, hover over to see a quick preview and click on the tool to access all related resources. Let us know how you've used these tools, we'd love to know how they've helped you in your patient engagement activities!

Planning Patient Engagement

Raising awareness on managing competing interests in a multi-stakeholder environment: Guidance to patients and engaging stakeholders
Learn More

Enhancement of the EUPATI industry guidance
Learn more

Recommendations on required capabilities for patient engagement
Learn More

Patient engagement agreements explained
Learn more

Patient engagement in medicines development: Recommendations on how to find the right match for the right patient engagement activity
Learn more

Conducting Patient Engagement

The code of conduct
Learn more

Working with Community Advisory Boards: Guidance and tools for patient communities and pharmaceutical companies
Learn More

Patient Engagement in Early Dialogues: Tools and resources for HTA bodies
Learn More

Reporting and Evaluation

Patient Engagement Monitoring and Evaluation Framework
Learn more

Guidance for reporting and dissemination of patient engagement activities
Learn more

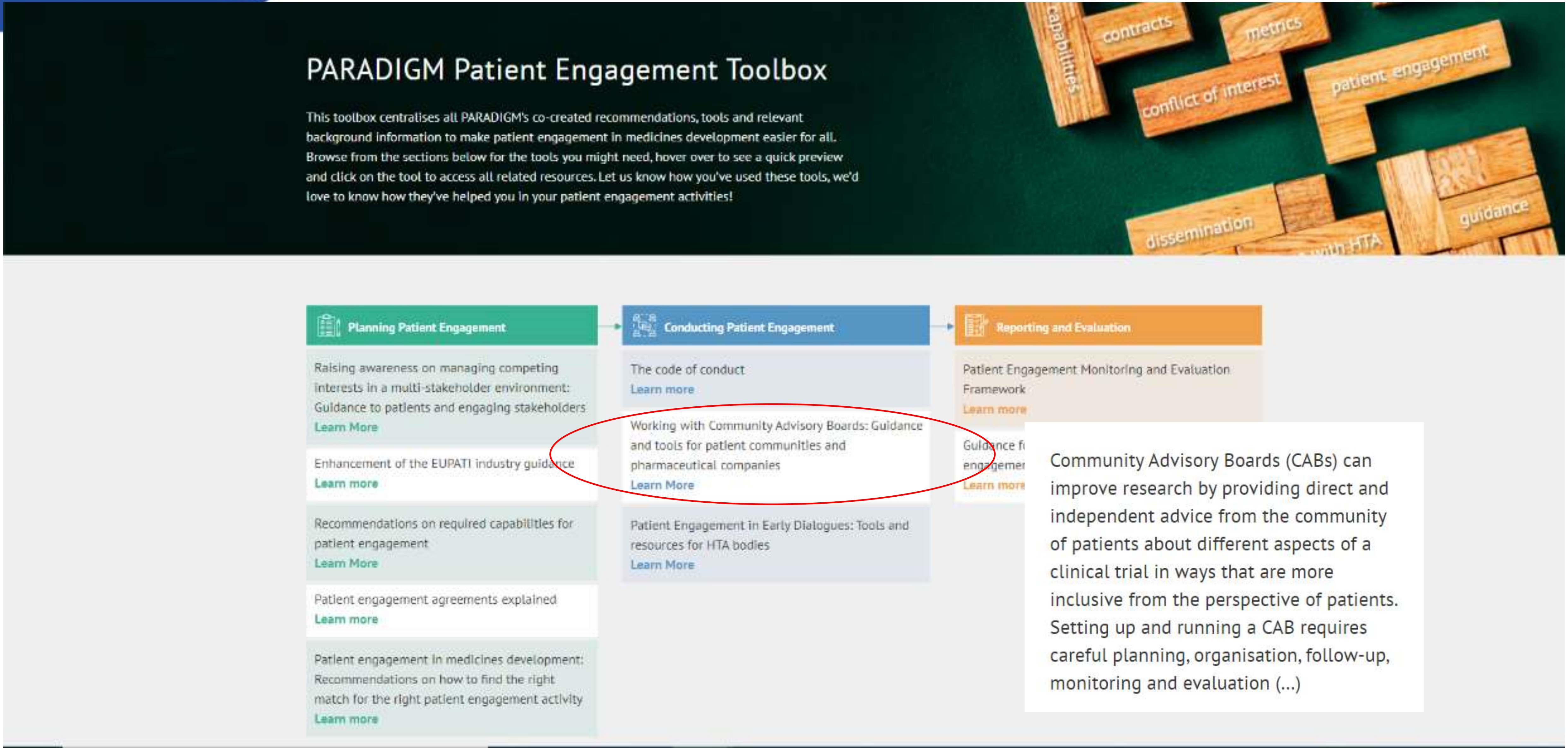
Patient engagement functions

Single point of contact both internally and externally hence initiating, facilitating and overseeing the engagement process

- ✓ 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.

Community Advisory Boards



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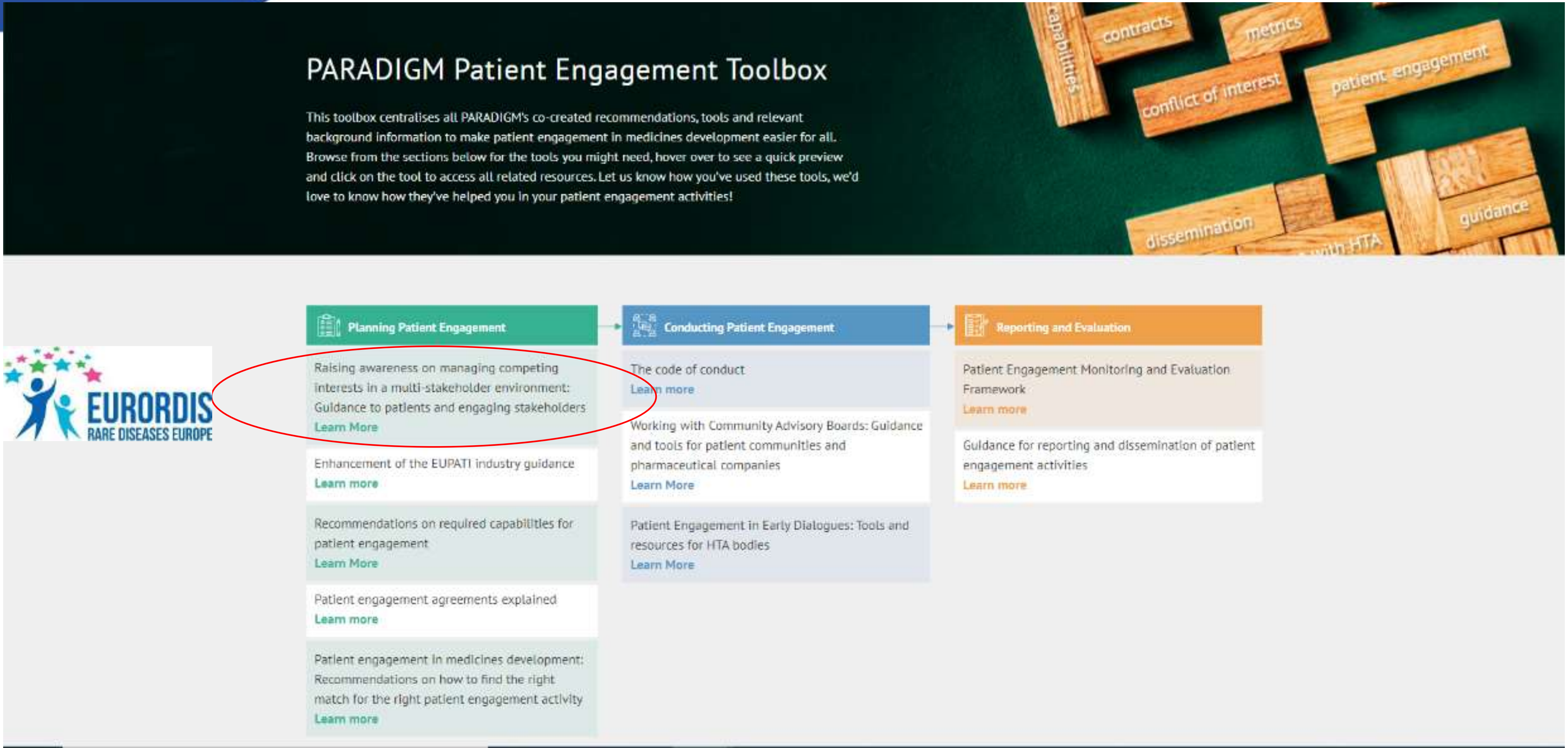
Guidance f
engagemen
Learn more

Community Advisory Boards (CABs) can improve research by providing direct and independent advice from the community of patients about different aspects of a clinical trial in ways that are more inclusive from the perspective of patients. Setting up and running a CAB requires careful planning, organisation, follow-up, monitoring and evaluation (...)

Managing Competing Interests



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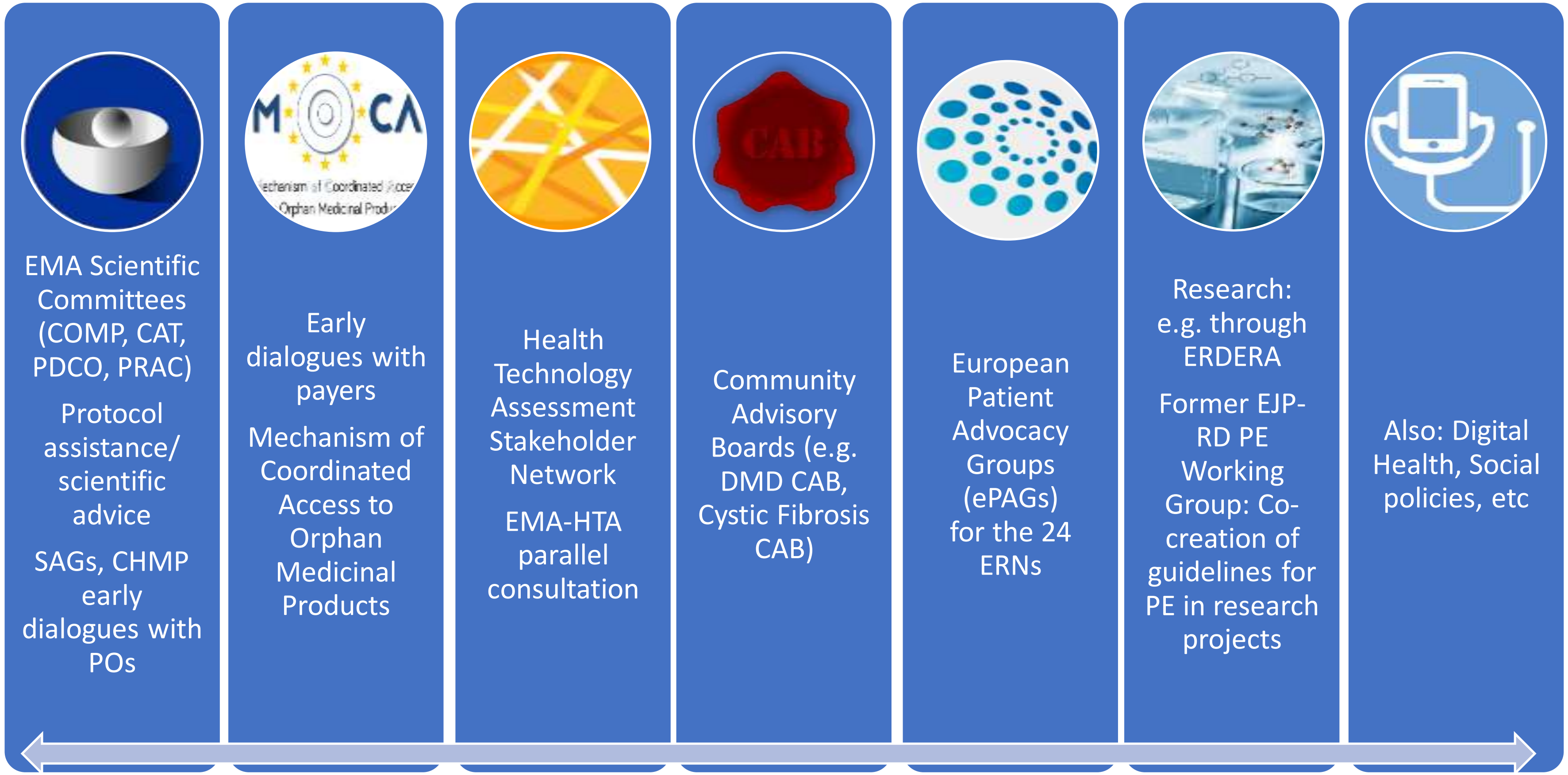
EURORDIS experience with patient engagement, projects and initiatives

Multiple forms of Patient Engagement



EMA Scientific Committees (COMP, CAT, PDCO, PRAC)

Protocol assistance/ scientific advice

SAGs, CHMP early dialogues with POs

Early dialogues with payers

Mechanism of Coordinated Access to Orphan Medicinal Products

Health Technology Assessment Stakeholder Network

EMA-HTA parallel consultation

Community Advisory Boards (e.g. DMD CAB, Cystic Fibrosis CAB)

European Patient Advocacy Groups (ePAGs) for the 24 ERNs

Research: e.g. through ERDERA

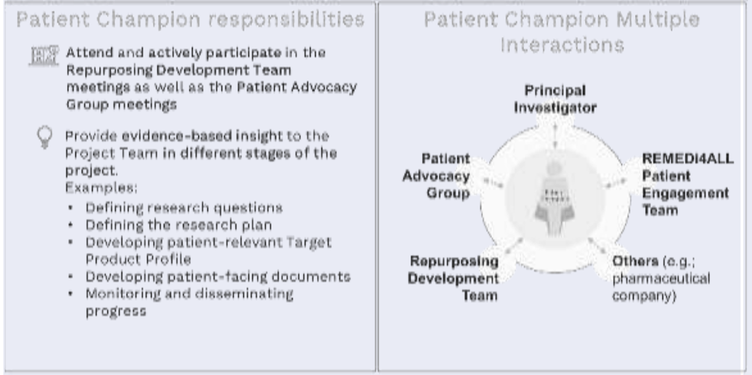
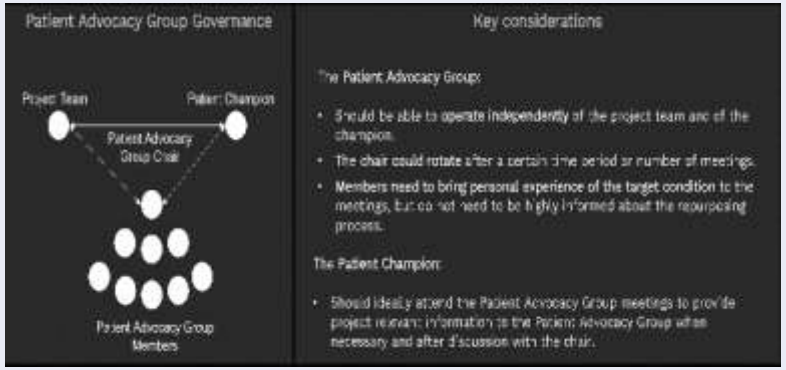
Former EJP-RD PE Working Group: Co-creation of guidelines for PE in research projects

Also: Digital Health, Social policies, etc

From Patient Engagement to Co-creation

Years	4 projects to advance PE in clinical trials design and set-up		Outputs/PE tools for co-creation
2018-2021	PARADIGM Patients Active in Research and Dialogues for an Improved Generation of Medicines	Public-private partnership (IMI) - Advancing meaningful patient engagement in the life cycle of medicines for better health outcomes.	PARADIGM toolbox e.g. **recommendations** on required capabilities for patient engagement
2018-2025	conect 4children COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN	Public-private partnership (IMI) - Better medicines for babies, children and young people through a pan-European clinical trial network.	Children and families play an active role in clinical trial development + organisation of **multi-stakeholder meetings**
2022-ongoing	REPURPOSING OF MEDICINES 4ALL	EU-funded project (Horizon Europe) – Platform to drive forward the repurposing of medicines in Europe.	Patient **champions**, Patient Advisory Groups (**PAGs**) & **multi-stakeholder meetings**
2025 onwards	Realise D compRehensive mEthodological and operational Approach to cLinical trialS in ultra-rarE DIseases	Public-private partnership (IHI) - cutting-edge operational and methodological tools and resources that can dramatically advance the evaluation of new treatments for people living with a rare and ultra-rare disease.	Patient Advisory Groups (**PAGs**) & **multi-stakeholder meetings**

Co-creation in action

Patient Champions	Patient Advisory Groups (PAGs)	Multi-stakeholder meetings (MSMs)
Patient, carer or patient representative in a **duet** with project PIs contribute to **Repurposing Development Team Meetings** (RDT) and provides input in defining research questions and plan, developing Target Product Profile and patient-facing documents and in monitoring and disseminating progresses	PAGs guarantee **representative, robust and balanced range of insights** into the diseases. InR4ALL, they act as an additional source of input for the Patient Champion, the project team and the wider consortium. In RealiseD, they will provide input into methodologies, statistical and operational approaches for the defined use cases. PAGs should be able to **operate independently** of the project team and the PIs/champion	Dialogue and constructive interaction between **ALL relevant stakeholders**: clinicians, academics, patients and patient representatives, pharmaceutical companies, regulators, HTA, payers, etc to share information and advance learning to inform on the current **state of art** in research and therapeutic development for a given disease and possibly address a specific **problem statement**
**Patient Champion responsibilities** Attend and actively participate in the Repurposing Development Team meetings as well as the Patient Advocacy Group meetings Provide evidence-based insight to the Project Team in different stages of the project. Examples: • Defining research questions • Defining the research plan • Developing patient-relevant Target Product Profile • Developing patient-facing documents • Monitoring and disseminating progress **Patient Champion Multiple Interactions** 	**Patient Advocacy Group Governance** **Key considerations** The Patient Advocacy Group: • Should be able to operate independently of the project team and of the champion. • The chair could rotate after a certain time period or number of meetings. • Members need to bring personal experience of the target condition to the meetings, but do not need to be highly informed about the repurposing process. The Patient Champion: • Should ideally attend the Patient Advocacy Group meetings to provide project relevant information to the Patient Advocacy Group when necessary and after discussion with the chair.	 J Crohns Colitis. 2022 Sep 21;17(2):249–258. doi: 10.1093/ecco-jcc/jjac135 **Paediatric Inflammatory Bowel Disease: A Multi-Stakeholder Perspective to Improve Development of Drugs for Children and Adolescents** Nicholas M Croft, Lissy de Ridder, Anne M Griffiths, Jeffrey S Hyams, Frank M Ruemmele, Dan Turner, Katharine Cheng, Irja Lutsar, Marco Greco, Zuzanna Gołębiewska, Floriane Laumond, Maria Cavaller-Bellaubi, Adam Elgreey, Tara A Altepeter, Chrissi Pallidis, Koen Norga, Robert Nelson, Wallace Crandall, Gilles Vassal Author information • Article notes • Copyright and License information PMCID: PMC10024546 PMID: 36130114

EURORDIS colleagues

conect4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

REPURPOSING OF MEDICINES 4ALL

Realise D
compRehensive mEthodological and operational Approach to cLinical trialS in ultra-rarE Diseases


Maria

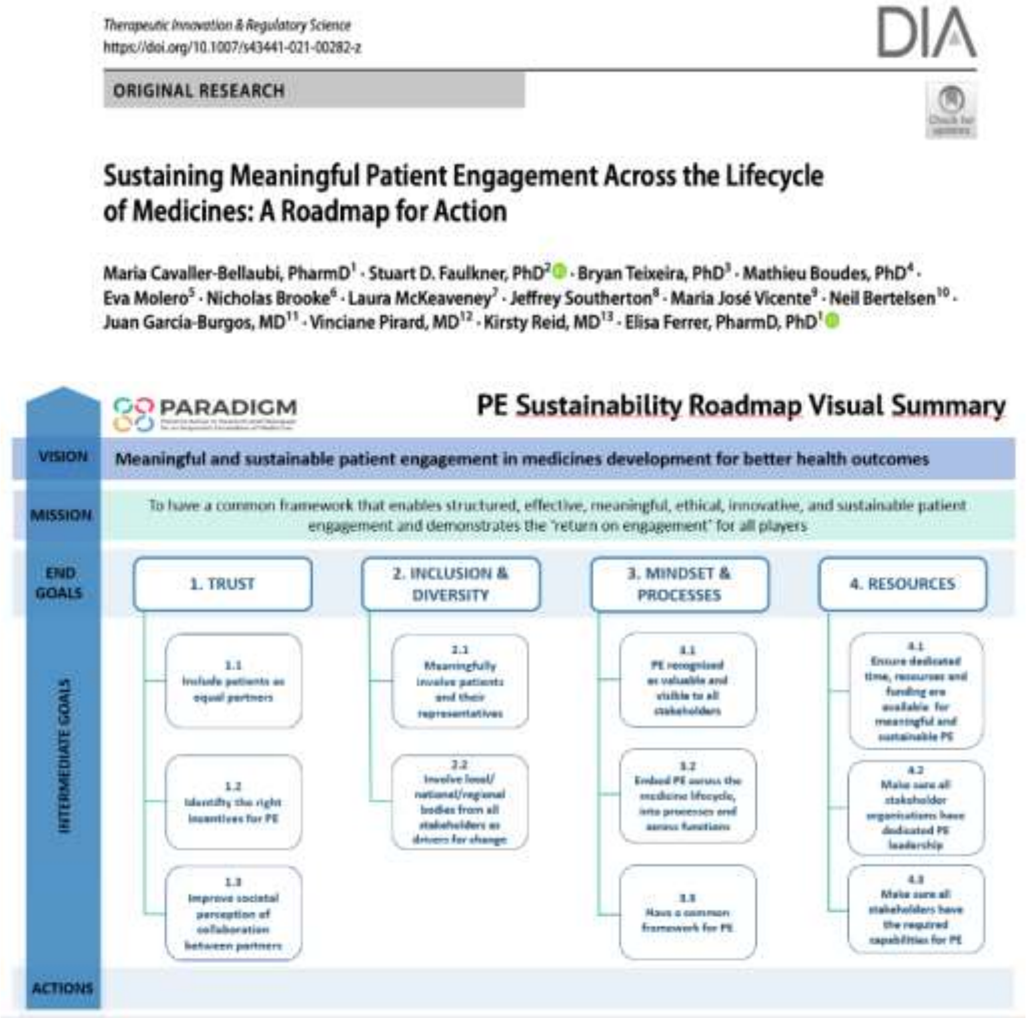

Gulcin


Claudia


Judit


Rita

Further reading



Therapeutic Innovation & Regulatory Science
https://doi.org/10.1007/s43441-021-00282-z

DIA

ORIGINAL RESEARCH

Sustaining Meaningful Patient Engagement Across the Lifecycle of Medicines: A Roadmap for Action

Maria Cavaller-Bellaubi, PharmD[1] · Stuart D. Faulkner, PhD[2] · Bryan Teixeira, PhD[3] · Mathieu Boudes, PhD[4] · Eva Molero[5] · Nicholas Brooke[6] · Laura McKeaveney[7] · Jeffrey Southerton[8] · Maria José Vicente[9] · Neil Bertelsen[10] · Juan Garcia-Burgos, MD[11] · Vinciane Pirard, MD[12] · Kirsty Reid, MD[13] · Elisa Ferrer, PharmD, PhD[1]

PARADIGM

PE Sustainability Roadmap Visual Summary

VISION Meaningful and sustainable patient engagement in medicines development for better health outcomes

MISSION To have a common framework that enables structured, effective, meaningful, ethical, innovative, and sustainable patient engagement and demonstrates the 'return on engagement' for all players

END GOALS

1. TRUST	2. INCLUSION & DIVERSITY	3. MINDSET & PROCESSES	4. RESOURCES
1.1 Include patients as equal partners	2.1 Meaningfully involve patients and their representatives	3.1 PE recognised as valuable and visible to all stakeholders	4.1 Ensure dedicated time, resources and funding are available for meaningful and sustainable PE
1.2 Identify the right incentives for PE	2.2 Involve local/national/regional bodies from all stakeholders as drivers for change	3.2 Embed PE across the medicine lifecycle, into processes and across functions	4.2 Make sure all stakeholder organisations have dedicated PE leadership
1.3 Improve societal perception of collaboration between partners		3.3 Have a common framework for PE	4.3 Make sure all stakeholders have the required capabilities for PE

Cavaller-Bellaubi *et al.*
Research Involvement and Engagement (2024) 10:125
https://doi.org/10.1186/s40900-024-00658-z

Research Involvement and Engagement

RESEARCH **Open Access**

A matrix tool to foster patient engagement in children, adolescents and young adults: report from a multistakeholder workshop

Maria Cavaller-Bellaubi[1*], Eva Degraeuwe[1,2], Johan Vande Walle[1,2,4], Elke Gasthuys[3,4] and Agnieszka Prytula[1,2] on behalf of the expert group

Fig. 1 Paediatric Patient Engagement Matrix tool. Elements to consider when engaging the paediatric population in paediatric drug development

Thank you for your attention!

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