The challenge, the vision and the value

Meaningful patient involvement in collaborative research projects

Jan Geissler, Patvocates // LeukaNET // CML Advocates Network
IMI impact on patient involvement, 7 Oct 2021

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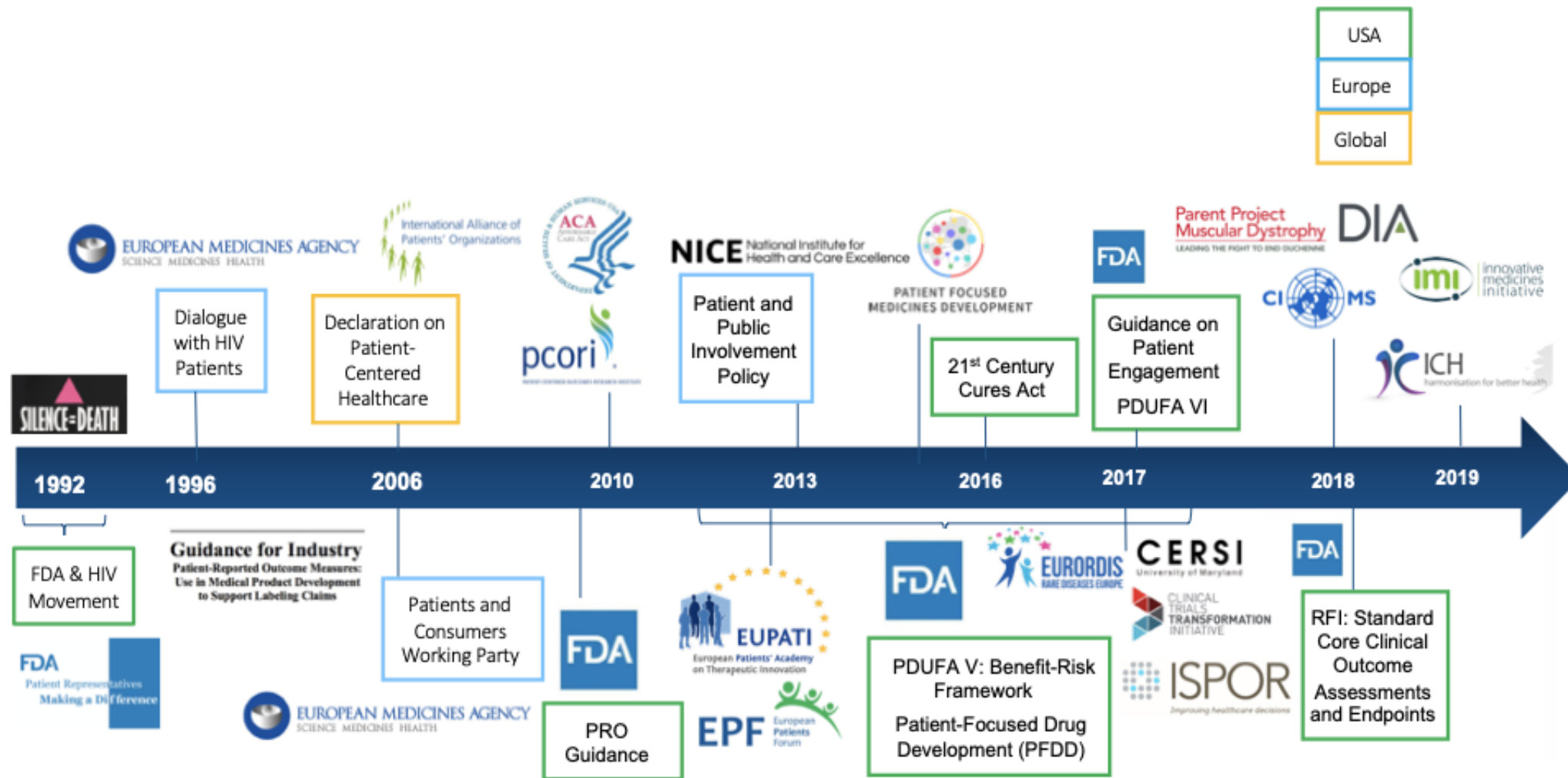# Why should academic researchers, industry and regulators involve patient advocates?

Move away from glossy statements on „patient centricity" towards real involvement of patients in plans, actions, and outcomes

- Gather insights into the day-to-day reality of patients

- Understand „patients' unmet need", "patient preferences", positive "benefit/risk" and „real value" to patients

- Design better clinical trials, services and info resources, leading to wiser investment of limited resources and more meaningful data
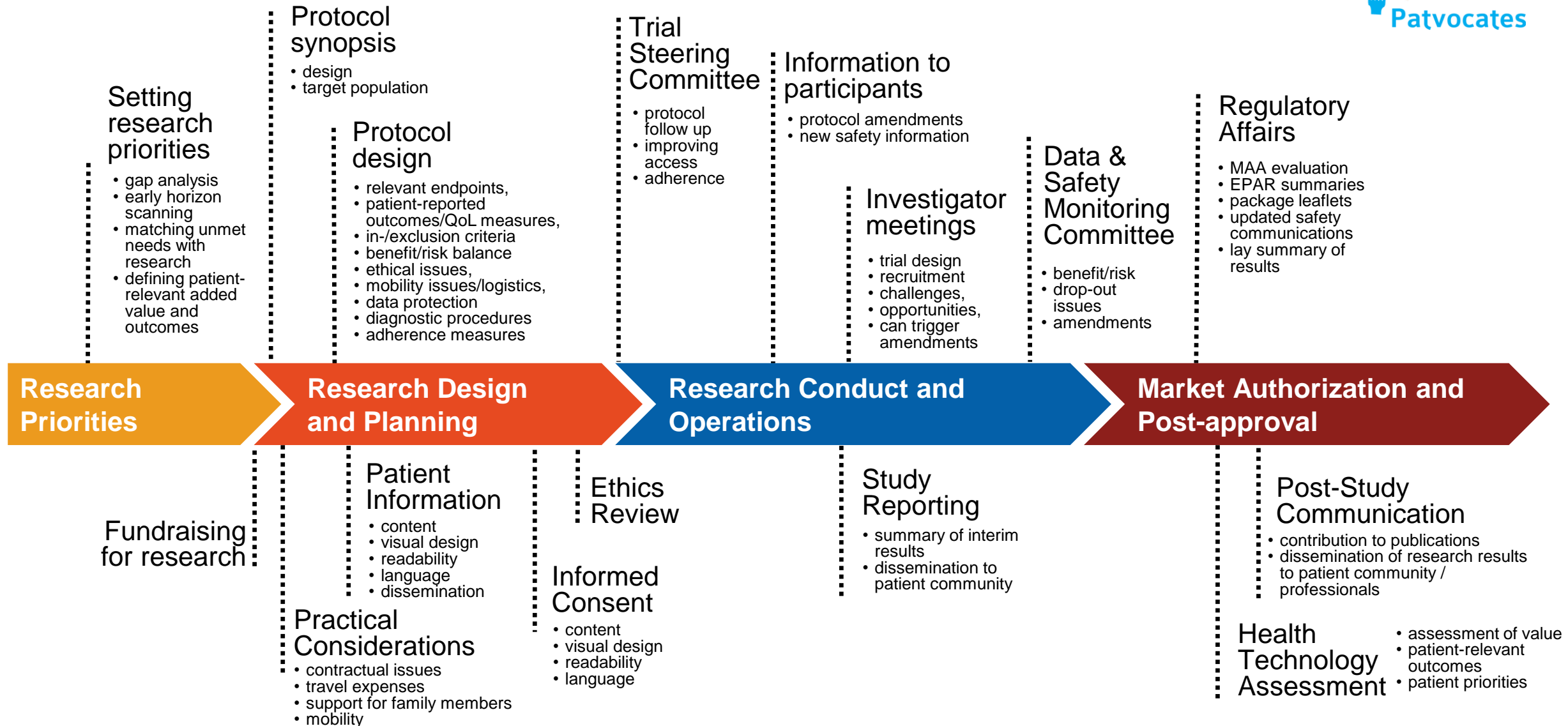
Patient involvement in R&D is not a new fashion!
Evolution from 1990s to strong maturity today

Source: PFMD / NHC (2020)

Patient involvement in clinical development in practice

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Setting research priorities
- gap analysis
- early horizon scanning
- matching unmet needs with research
- defining patient-relevant added value and outcomes

Protocol synopsis
- design
- target population

Protocol design
- relevant endpoints,
- patient-reported outcomes/QoL measures,
- in-/exclusion criteria
- benefit/risk balance
- ethical issues,
- mobility issues/logistics,
- data protection
- diagnostic procedures
- adherence measures

Trial Steering Committee
- protocol follow up
- improving access
- adherence

Information to participants
- protocol amendments
- new safety information

Investigator meetings
- trial design
- recruitment challenges,
- opportunities,
- can trigger amendments

Data & Safety Monitoring Committee
- benefit/risk
- drop-out issues
- amendments

Regulatory Affairs
- MAA evaluation
- EPAR summaries
- package leaflets
- updated safety communications
- lay summary of results

Research Priorities	Research Design and Planning	Research Conduct and Operations	Market Authorization and Post-approval

Fundraising for research

Patient Information
- content
- visual design
- readability
- language
- dissemination

Ethics Review

Study Reporting
- summary of interim results
- dissemination to patient community

Post-Study Communication
- contribution to publications
- dissemination of research results to patient community / professionals

Practical Considerations
- contractual issues
- travel expenses
- support for family members
- mobility

Informed Consent
- content
- visual design
- readability
- language

Health Technology Assessment
- assessment of value
- patient-relevant outcomes
- patient priorities

20% of 135 IMI projects had patient organisations in the consortium, 30% in patient advisory boards (60% by 2021)

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Risk – Benefit Appraisal: **PROTECT** International Alliance of Patients' Organizations	**Dementia:** **EPAD** • Alzheimer Europ	**Incorporating Real World data:** **GetReal** • International Alliance of Patients' Organizations	**Big data in Hematological Malignancies** • LeukaNET + Myeloma Patients Europe + MDS Alliance + Acute Leukemia Advocates Network + Childhood Cancer International + Lymphoma Coalition + CLL Advocates Network
Dementia: **PHARMA-COG** • Alzheimer Europe • Greek Association of Alzheimer's Disease and Related Disorders:	**Chronic Obstructive Pulmonary Disease (COPD):** **PRO- Active** • Astma Fonds Longstichting • British Lung Foundation	**Neurodegenerative diseases:** **AETIONOMY** • Alzheimer Europe	**Big data in HM** • LeukaNET + Myeloma Patients Europe + MDS Alliance + Acute Leukemia Advocates Network + Childhood Cancer International + Lymphoma Coalition + CLL Advocates Network • CML Advocats Network + MPN Advocates Network
Alzheimer's disease: **EMIF** • Alzheimer Europe	**Autism spectrum disorders:** **EU-AIMS** • Autism Speaks Inc.	**Recognising adverse drug reactions:** **WEB-RADR** • European Organisation for Rare Diseases	
Asthma: **U-BIOPRED** • European Lung Foundation • Netherlands Asthma Foundation • Asthma UK • European Federation of Allergy and Asthma • Lega Italiana Anti Fumo	**European Patients' Academy on Therapeutic Innovation:** **EUPATI** • European Patients Forum • European Organisation for Rare Diseases • European AIDS Treatment Group • Irish Platform for Patients' Organisations, Science and Industry • European Genetic Alliances Network • Vereniging Samenwerkende Ouder - en Patiëntenorganisaties • Genetic Alliance UK		**Patient engagement in R&D** **PARADIGM** • European Patients Forum • EURORDIS • Alzheimer Europe • European AIDS Treatment Group

The Innovative Medicines Initiative and patients - a partnership

A short guide to successful patient involvement in EU-funded research

Lessons learnt from the U-BIOPRED project

Patient engagement ecosystem has evolved: Trainings and tools

Training for industry
- EUPATI Essentials
- EUPATI Fundamentals
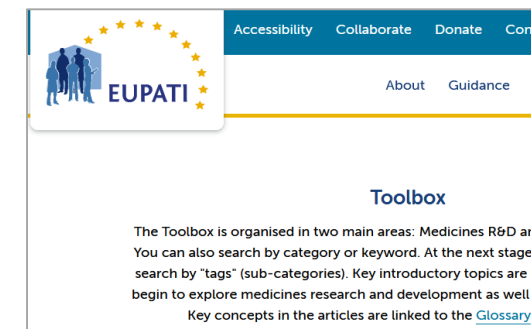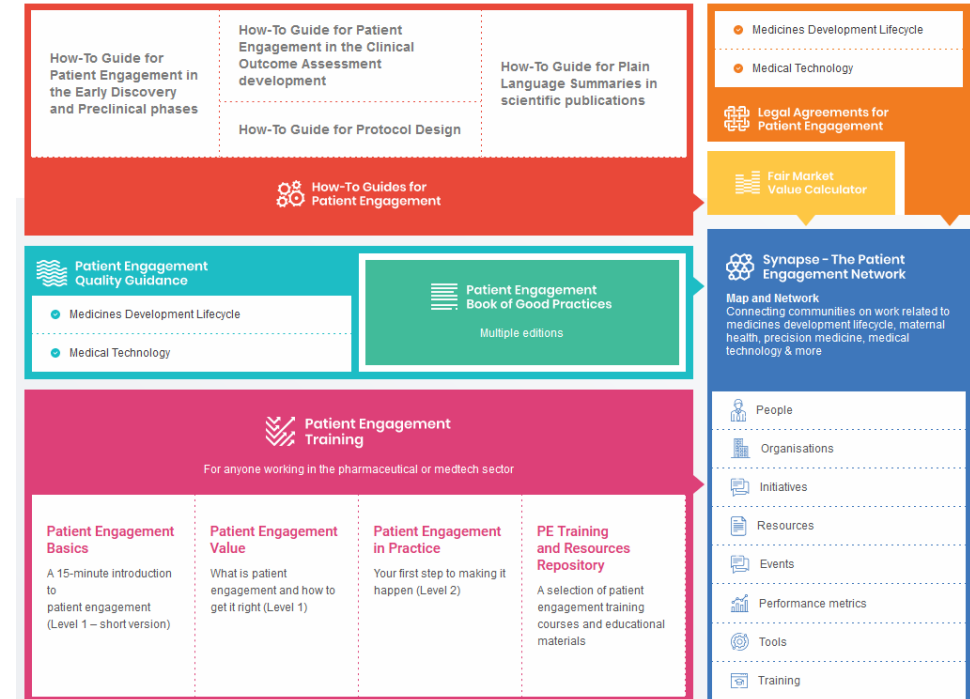- PFMD PE Training

Training for patient advocates
- EUPATI Patient Expert Training Course
- EUPATI Toolbox
- EUPATI National Platforms

Guidance, Frameworks and tools for patient engagement
- 4 EUPATI Guidances on Patient Engagement
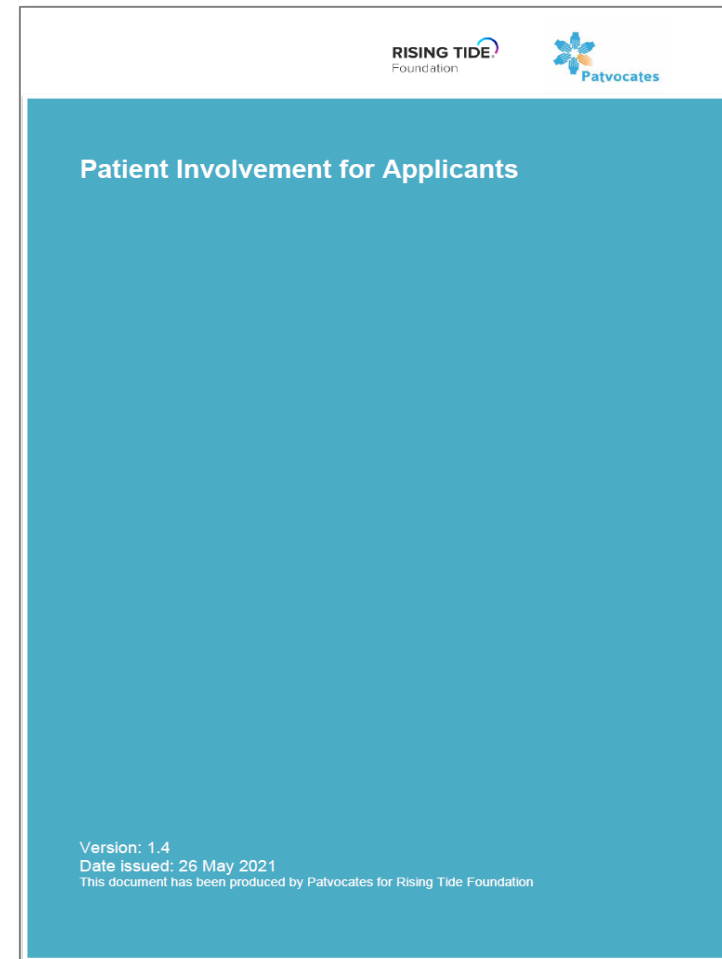- PARADIGM frameworks
- PFMD how-to-guides

Matchmaking & Best Practice Sharing
- PFMD Synapse

Practical guidance on patient involvement in collaborative research projects

- Checklist when planning patient engagement

- Examples of potential contributions of patients

- Organisational models of patient engagement in research projects

- Identification of the right patient organisation/patient advocates and resourcing the contributions

- Patient involvement plans

- Preparing the patient community for their contribution in the post-application, pre-launch phase

Patient Involvement for Applicants

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Available for download at
https://www.risingtide-foundation.org/clinical-cancer-research/program-/-core-belief

Involvement models
for collaborative research projects

Engagement model	Patient organization's / patient expert's role	Impact
Project coordinator, chair or co-chair	Leads and coordinates the research project	Very high
Steering committee member	Member of the governance board of the research project	Very high
Work package leader	Coordinates a specific work package in the project	High
Research project member	Full member of the research teams	Medium
Patient involvement hub	Full project member, coordinating all contributions from the wider patient community	High
Associated project partner	Partnership agreement with the research project (but usually no funding)	Small
Advisor / advisory board member	Providing advice in ethics committee, scientific/project advisory board, data safety monitoring board, but no governance or leadership role	Small

Available for download at
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The first and largest Public-Private Partnership for Big Data in Hematology

14 key targeted blood cancers

ALL · AML · Childhood HMs · CML · CLL · MDS · PV

ET · Myelo-fibrosis · NHL · HL · MM · WM · Amyloi-dosis

Patient input into HARMONY Core Outcome Sets, HARMONY Delphi Surveys, HARMONY Research proposals

HARMONY Patient Organisations Masterclass, HARMONY Communications about Big Data in Blood Cancer and about project results to patients and general audience

HARMONY Ethical Framework, De-identification Mechanisms

Patient input into core HARMONY tasks:
- Stakeholder Forum
- Clinical Value Framework
- Access Evidence Framework

ALAN — Acute Leukemia Advocates Network
Childhood Cancer International
CLLAN — CLL ADVOCATES NETWORK
THE MDS ALLIANCE · ALLIANCE · ALLIANZ · ALIANZA
MPe — Myeloma Patients Europe
LYMPHOMA COALITION EUROPE
CML Advocates Network
MPN Advocates Network
LeukaNET — Leukemia Advocates Network

All essential ingredients for patient engagement for more inclusiveness are here

- Shared purpose and **collaborative spirit**

- **Political and institutional will**

- Engagement **frameworks**

- **Capacity** to engage
  e.g. by patient organisations, companies and institutions

All essential ingredients are here.
Let's engage!

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