The challenge, the vision and the value

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Meaningful patient involvement in collaborative research projects

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

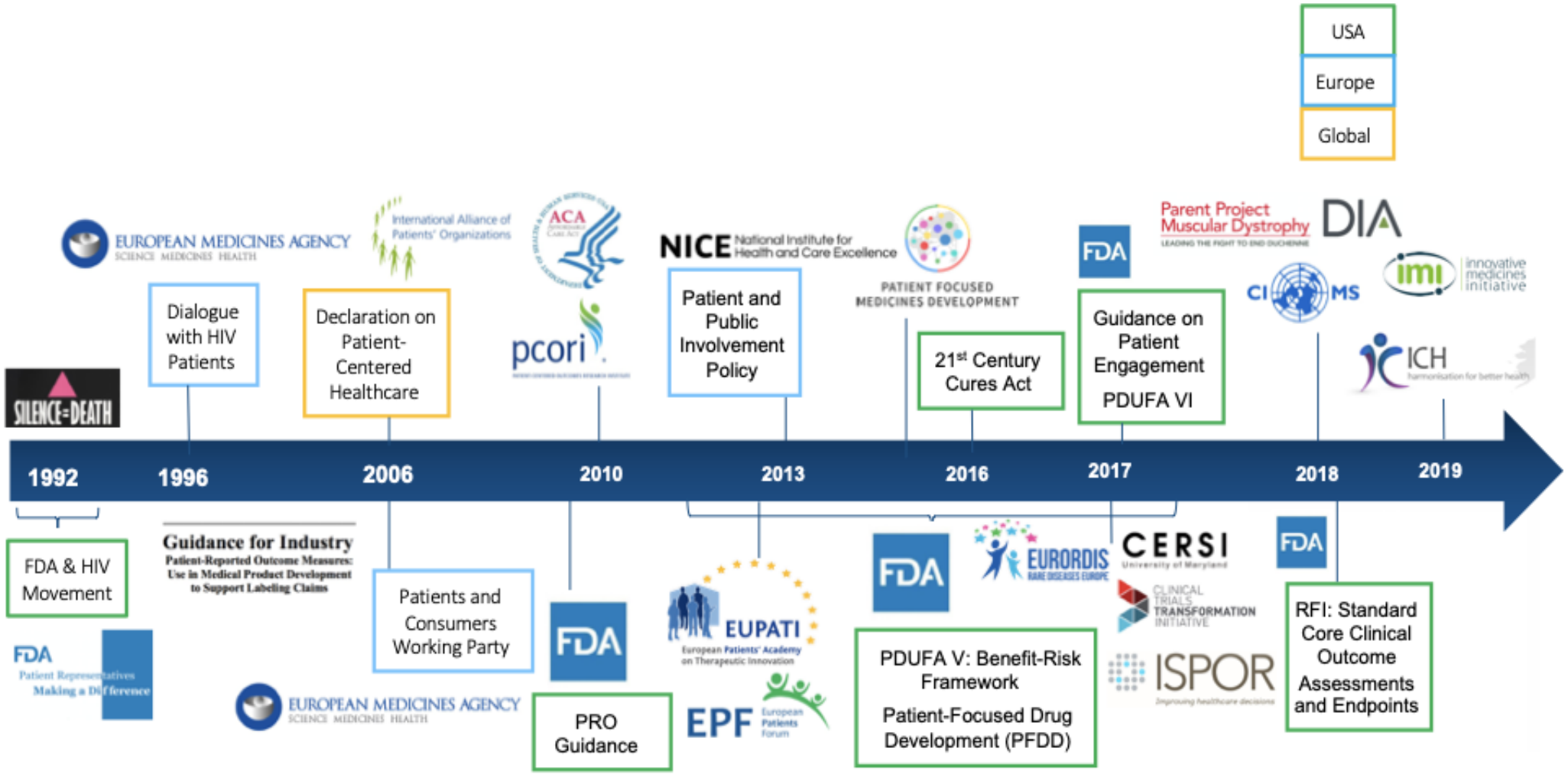
Why should academic researchers, industry and regulators involve patient advocates?

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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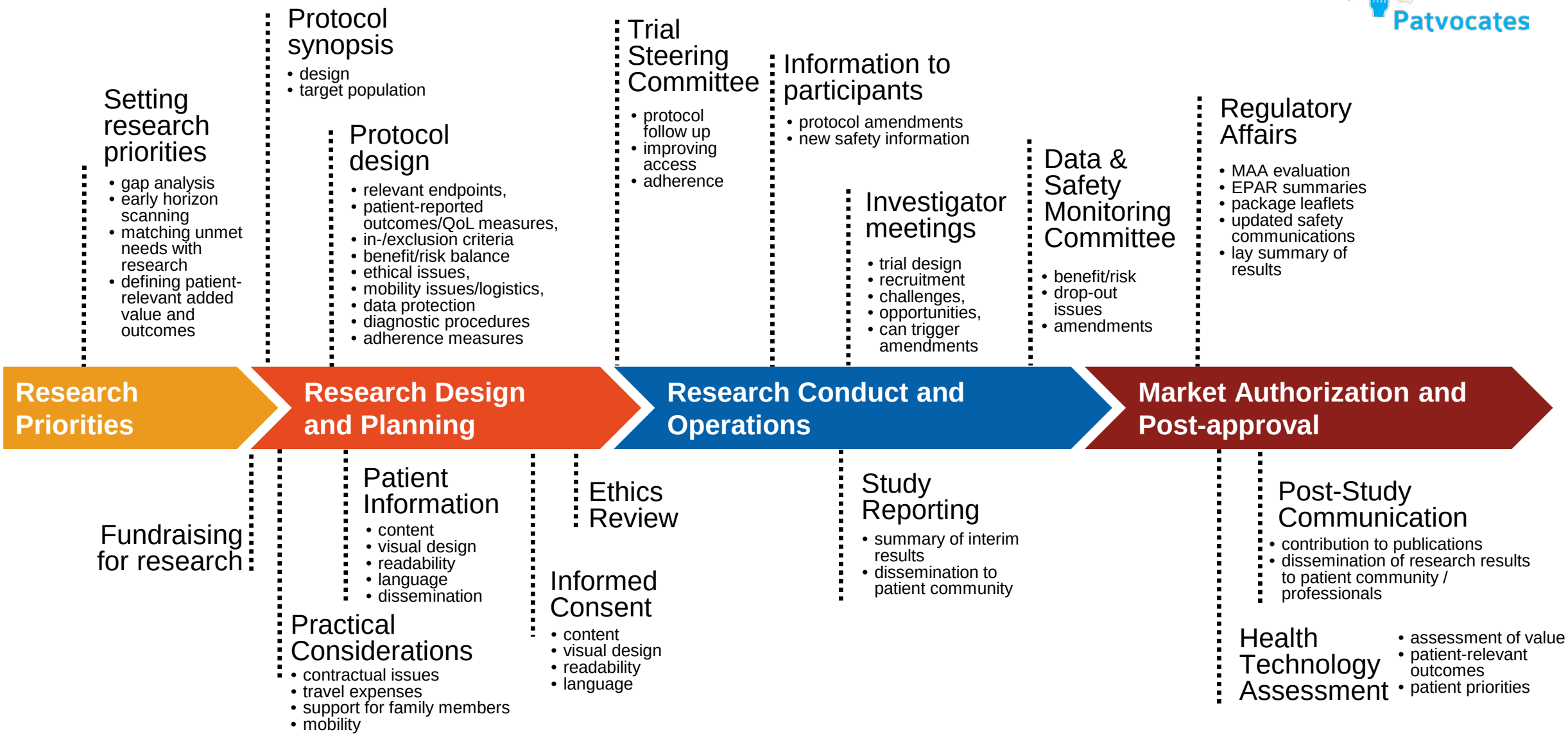

Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Geissler, Ryll, Leto, Uhlenhopp, Therapeutic Innovation & Regulatory Science (2017), doi: 10.1177/2168479017706405, and at www.eupati.eu

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

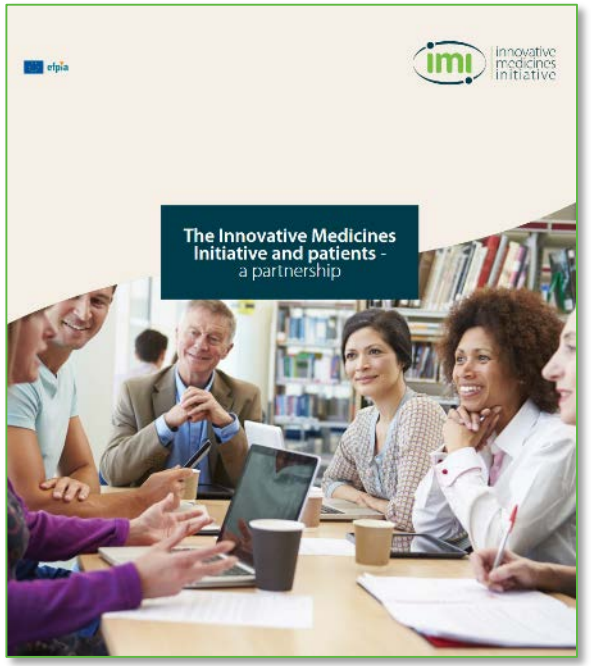
Autism spectrum disorders:
EU-AIMS
- Autism Speaks Inc.

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



Source: IMI (2019)

Patient engagement ecosystem has evolved: Trainings and tools

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Training for industry

- EUPATI Essentials
- EUPATI Fundamentals
- PFMD PE Training

Training for patient advocates

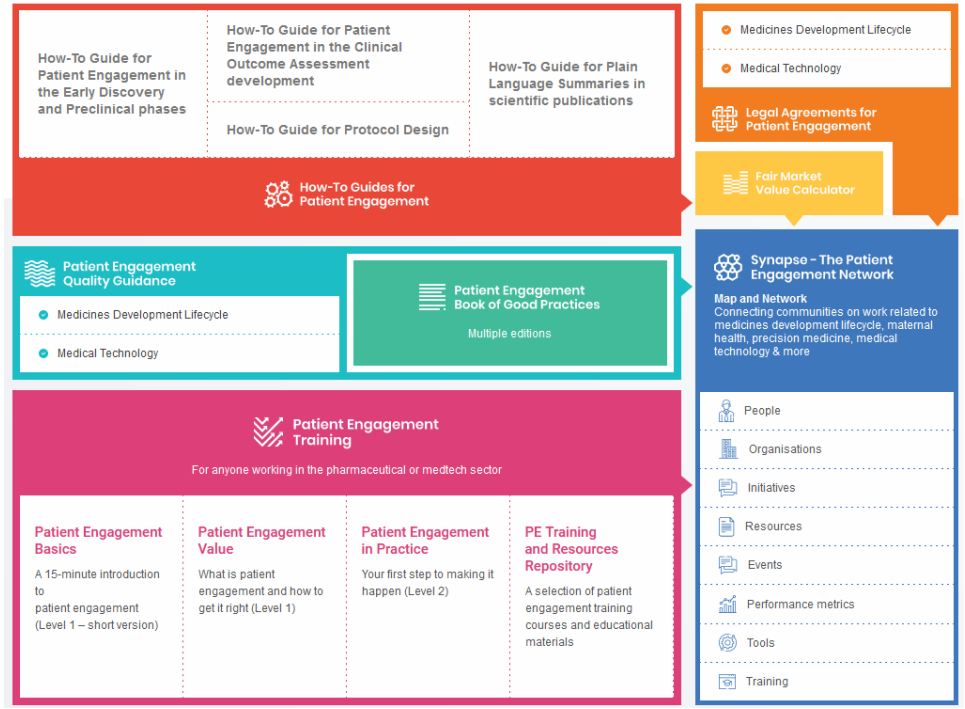
- EUPATI Patient Expert Training Course
- EUPATI Toolbox
- EUPATI National Platforms

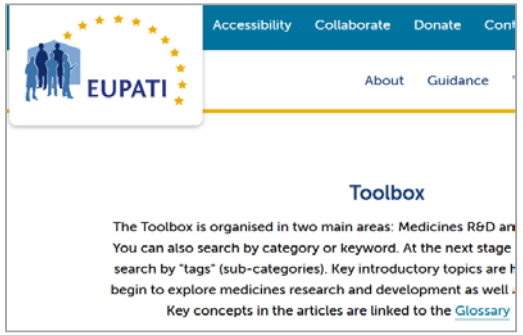
Guidance, Frameworks and tools for patient engagement

- 4 EUPATI Guidances on Patient Engagement
- PARADIGM frameworks
- PFMD how-to-guides

Matchmaking & Best Practice Sharing

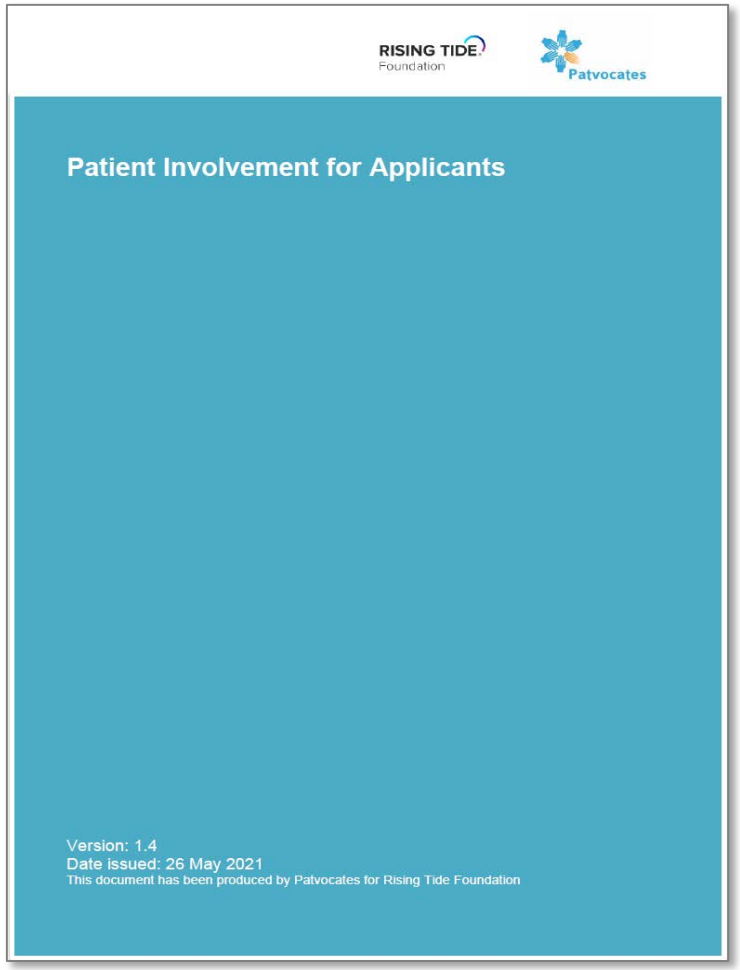
- PFMD Synapse





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



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

Involvement models for collaborative research projects

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RISING TIDE

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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HARMONY ALLIANCE

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

14 key targeted blood cancers

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

LeukaNET Leukemia Advocates Network

ALAN

Childhood Cancer International

CLLAN CLL ADVOCATES NETWORK

THE MDS ALLIANCE

MPe Myeloma Patients Europe

LYMPHOMA COALITION EUROPE

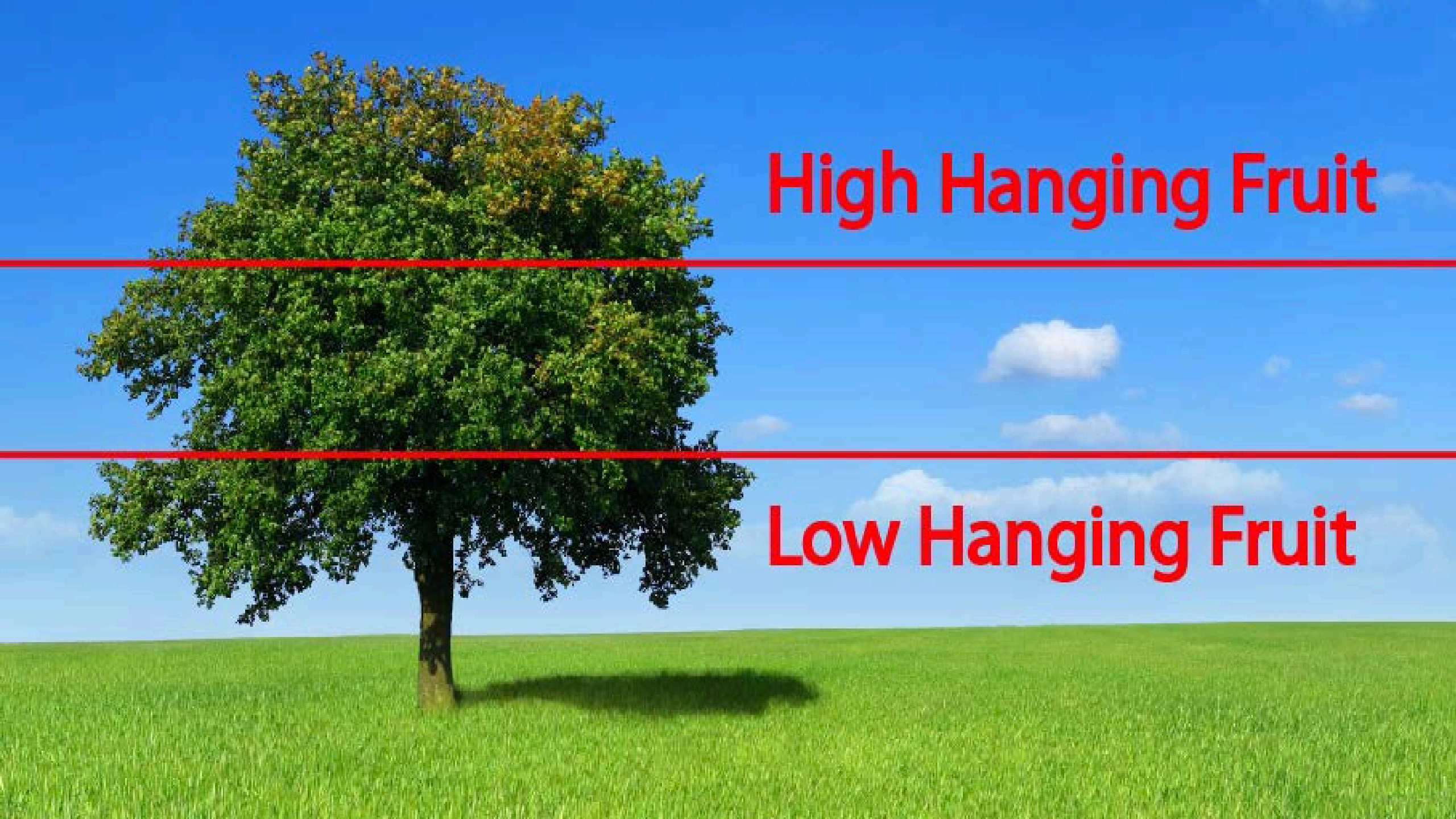
CML Advocates Network

MPN Advocates Network

HARMONY

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


High Hanging Fruit
Low Hanging Fruit

All essential ingredients are here. Let’s engage!

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