The European Medicine Regulatory Network: Present and Future

X FORESIGHT TRAINING COURSE

CONTRIBUTION OF EXPERT PATIENTS IN THE ASSESSMENT PROCESS OF INNOVATIVE MEDICINE

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Expert Patient: the definition

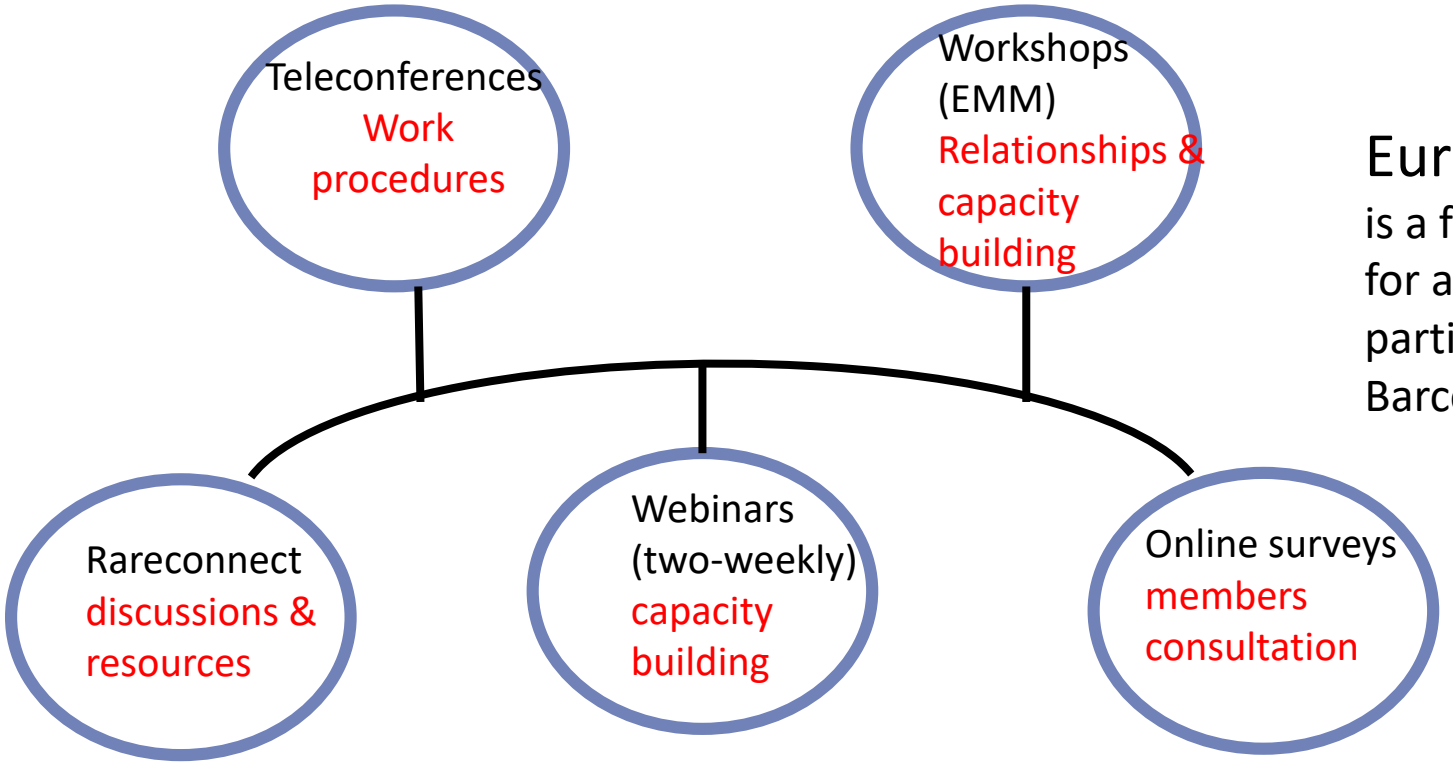
EXPERT PATIENT is defined a person that has built his capacity, competence and awareness not only about his disease. He's able to exert his rights thank to the achievement and knowledge of legislative, regulatory and ethical tools using them with appropriateness and competence. Furthermore, he's able to act for the advocacy of other people, sharing the same condition, that are not aware or competent in using achievements for personal benefit due to many reasons, for a poor education or difficult social context.

Expert Patient: the empowerment

- Joining the existing disease-specific associations at local, national and international level
- Becoming proactive in demanding to build capacities
- Active participation to workshops and conferences at local, national and international level

- EURORDIS - European Organization for rare Diseases -
- EUPATI - European Patients Academy -

Patient empowerment: EURORDIS mode



Eurordis Summer School

is a face to face one week event for a selected number of participants held every year at Barcelona (Spain)

Patient empowerment: EUPATI mode

EUPATI Training Course:

For Patients experts on Medicines' Research and Development

Specifically designed to meet the needs of patients and patients' advocates motivated to achieve knowledge on R&D

15 month training programme through an online platform and 2FtoF meetings

Giving a meaningful contribution to R&D process

Advocacy: the definition

Advocacy seeks to ensure that people, particularly those who are most vulnerable in society, are able to:

- Have their voice heard on issues that are important to them.
- Defend and safeguard their rights.
- Have their views and wishes genuinely considered when decisions are being made about their lives.

Advocacy is a process of supporting and enabling people to:

- Express their views and concerns.
- Access information and services.
- Defend and promote their rights and responsibilities.
- Explore choices and options

How patients' role is changing

FOCUS

POLICY

From passengers to co-pilots: Patient roles expand

Margaret Anderson* and K. Kimberly McCleary*

The premier position of medical research on the U.S. national policy agenda offers an unprecedented opportunity to advance the science of patient input and marks a turning point in the evolution of patient engagement.

Patients as key partners in rare disease drug development

Max G. Bronstein and Emil D. Kakkis

Rare disease drug development could benefit substantially from increased patient engagement and input to enhance understanding of the key aspects of disease impact, ways to measure these impacts and patients' perspectives on the benefit–risk profile of potential therapies.

BMJ

BMJ 2013;346:f2614 doi: 10.1136/bmj.f2614 (Published 14 May 2013)

EDITORIALS

Let the patient revolution begin

Patients can improve healthcare: it's time to take partnership seriously

Patients' engagement

Domecq *et al. BMC Health Services Research* 2014, **14**:89
http://www.biomedcentral.com/1472-6963/14/89

RESEARCH ARTICLE Open Access

Patient engagement in research: a systematic review

Juan Pablo Domecq[1,2,5], Gabriela Prutsky[1,2,5], Tarig Elraiyah[1,5], Zhen Wang[1,5,6], Mohammed Nabhan[1,5], Nathan Shippee[1,5,6], Juan Pablo Brito[1,4,5], Kasey Boehmer[1,5], Rim Hasan[1,5,8], Belal Firwana[1,5,8], Patricia Erwin[1,7], David Eton[1,5,6], Jeff Sloan[1,5,6], Victor Montori[1,2,4,5,6], Noor Asi[1,5], Abd Moain Abu Dabrh[1,5] and Mohammad Hassan Murad[1,3,5,6*]

According to this review Patients' engagement in Healthcare and research at British National Institute of Health is in place since 1996.

In USA the Patient Centered Outcomes research Institute (PCORI) has been established in 2010

Patients' engagement

- EMA – Adaptive Licensing Pilot Project
- European Reference Networks (ERNs)

Patients' engagement in EMA

EMA Adaptive Licensing Pilot Project launched in 2014

To bring medicines to patients

To maximize impact of new medicines on public health balancing a timely access to patients with providing a benefit-risk assessment

To promote an early multi-stakeholders dialogue

To gather patients' opinions and suggestions on real needs and concerns

EMA Adaptive Licensing Project

Three meetings of the Adaptive Licensing Discussion Groups (ALDG)

One was for Gene Therapy for thalassemia

Main goal for the drug developer for this meeting:

To obtain feedback on plans for parallel EMA/HTA Scientific Advice with particular focus on HTA aspects and involvement of patients representatives

This level of engagement for patients' representatives means that a cultural shift is ongoing as patients are considered as partners in research development

Patients' role is to work side by side with researchers and clinicians as the achievements have to be considered starting from real patients' needs

European Reference Networks **(ERNs)**

4.4.2011 EN Official Journal of the European Union L 88/45

DIRECTIVES

DIRECTIVE 2011/24/EU OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

of 9 March 2011

on the application of patients' rights in cross-border healthcare

17.5.2014 EN Official Journal of the European Union L 147/71

COMMISSION DELEGATED DECISION

of 10 March 2014

setting out criteria and conditions that European Reference Networks and healthcare providers wishing to join a European Reference Network must fulfil

(Text with EEA relevance)

(2014/286/EU)

17.5.2014 EN Official Journal of the European Union L 147/79

COMMISSION IMPLEMENTING DECISION

of 10 March 2014

setting out criteria for establishing and evaluating European Reference Networks and their Members and for facilitating the exchange of information and expertise on establishing and evaluating such Networks

(Text with EEA relevance)

(2014/287/EU)

ERNs Project

European Reference Networks (ERNs)

is a project launched in 2011 from the European Commission

The Networks should improve access to diagnosis, treatment and the provision of high-quality healthcare for patients with rare diseases

... to patients who have conditions requiring a particular concentration of resources or expertise

... and could also be focal points for medical training and research, information dissemination and evaluation, especially for rare diseases.

Patients' representatives in ERNs

European Reference Networks (ERNs) created on founding principles of patient-centred care, patient advocate empowerment, patient engagement

European Patient Advisory Groups:

- Forum for dialogue, unity & solidarity to optimise involvement of patients
- Representativeness to engage into application and governance of rare diseases ERNs
- Open to members & non-member patient groups in EU
- Aligned with RD ERN scope
- Composed of elected ePAG representatives & member organizations
- Democratically established where there is an ERN application & progressively expanded (a work in progress; perfectible next years)
- Can become formal members of ERN boards (role & function agreed on with respective clinical leads)
- Ensure two-way, vertical flow of information between ERN and ePAG, reflecting patients viewpoint & supporting ERNs including governance & clinical & operational delivery,

Role of patients' representatives

Decision making structures, for all relevant aspects of the EN strategy, policy, organization, processes

Identification of expert centres Evaluation of how the ERN act on feedback from patients

Best opinion **patient perspective on the needs**

Planning, assessment and evaluation

Patient-centric approach in both delivery of clinical care, service improvement and strategic development

Transparency in quality of care, safety standards, clinical outcomes and treatment options

Ethical issues and concerns

Application of **personal data rules, compliance of information consent and management of complaints**

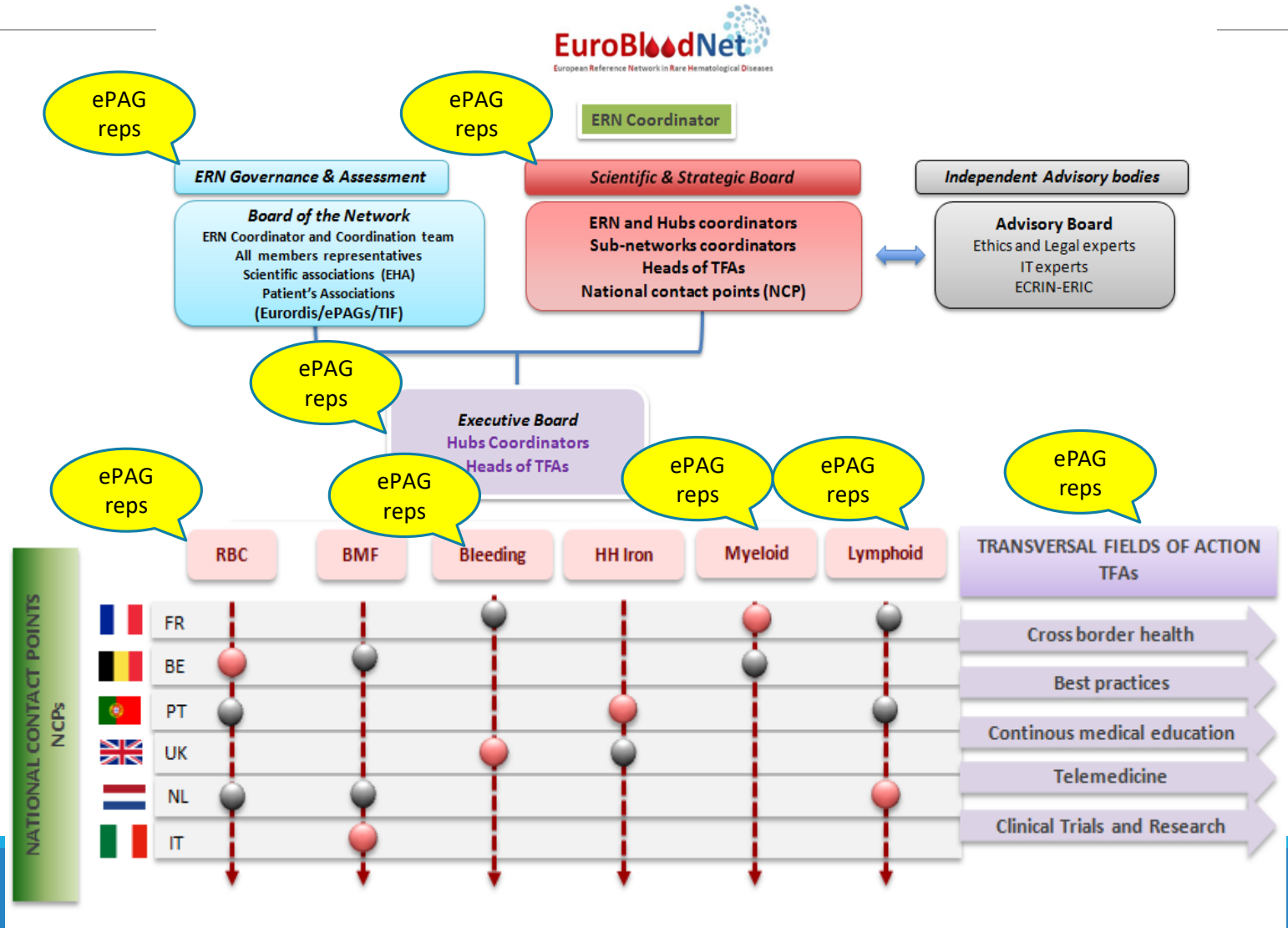
Feedback and evaluation of **patient experience**

Development and dissemination of **patient information, policy, good practice, care pathways and guidelines**

Reviewing the performance, quality indicators, access times to diagnostic and treatment, clinical outcomes of diagnosis and treatment.

Contribute to the research

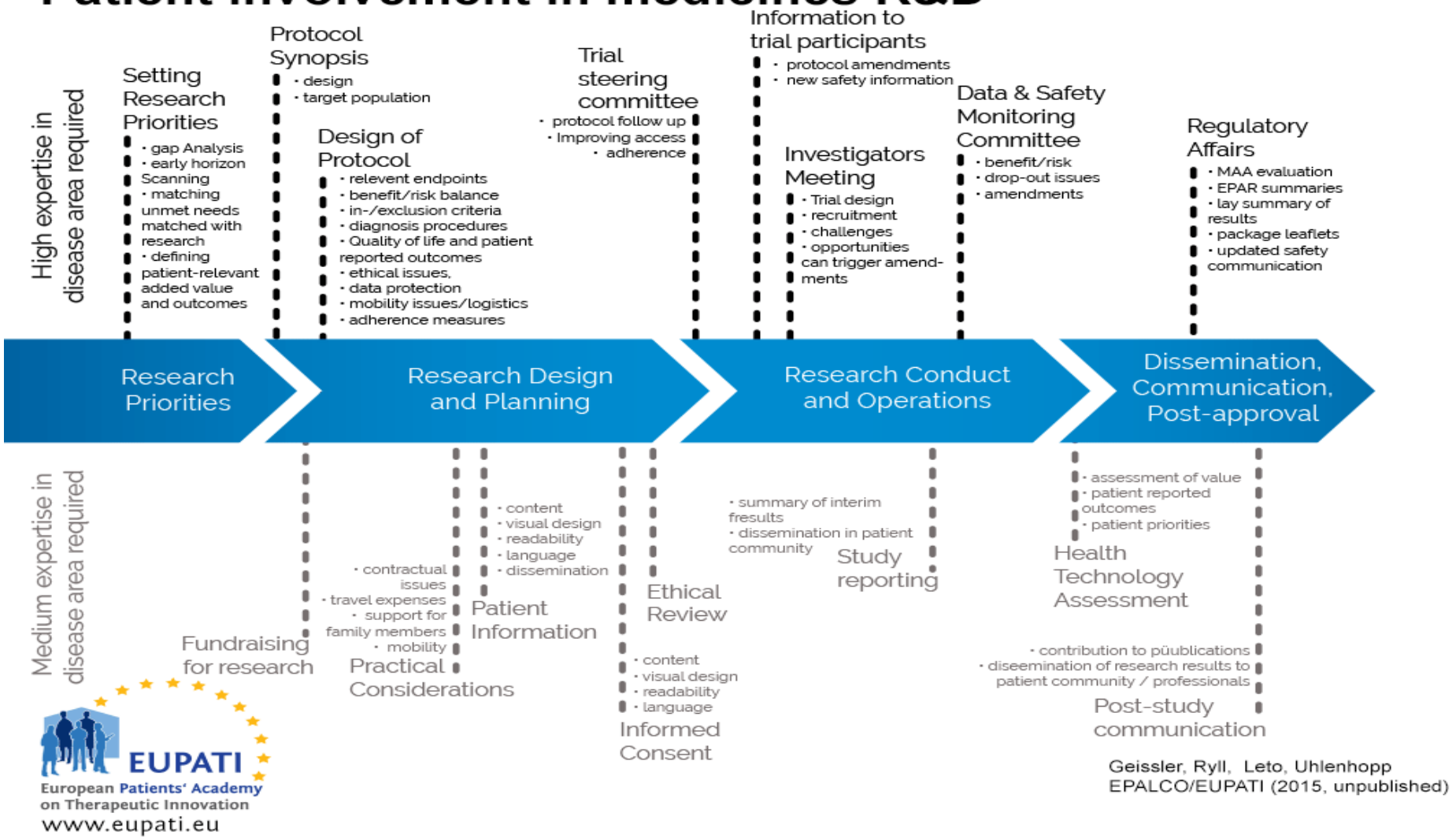
Where may patients' advocate organizations contribute?



Patients' involvement in R&D

- Pharmaceutical industry-led medicines R&D
- Ethics Committees
- Regulatory Authorities
- Health Technology Assessment (HTA)

Patient involvement in medicines R&D

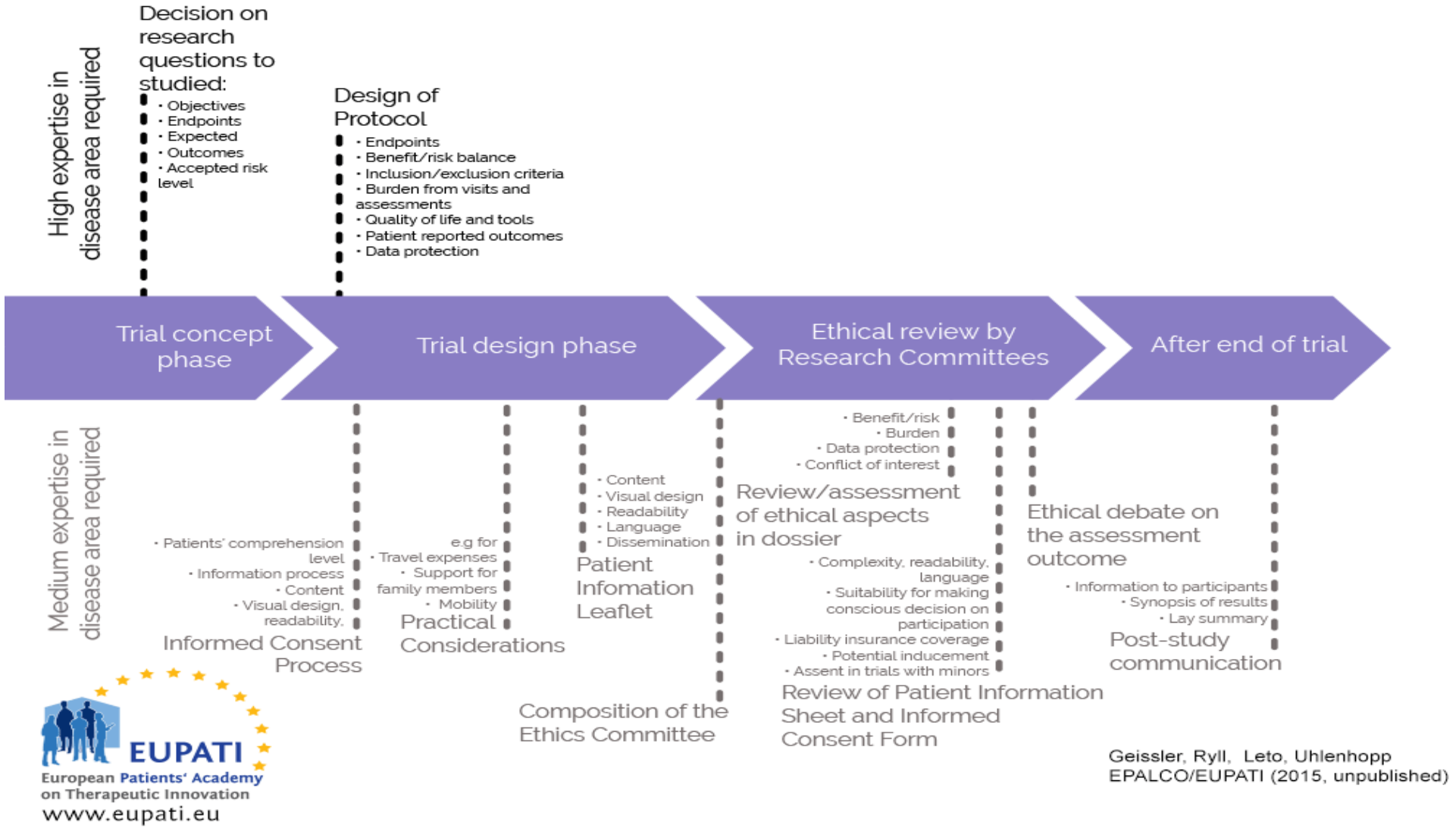


Geissler, Ryll, Leto, Uhlenhopp
EPALCO/EUPATI (2015, unpublished)

Patients' involvement in Clinical Trials

- **Relevance**: Patients have knowledge, perspectives and expertise to contribute to ethical deliberations
- **Fairness**: Patients have the same rights to contribute to ethical review of CTs as other stakeholders and have access to knowledge that enable effective engagement
- **Equity**: Patients can contribute to equity by seeking to understand the diverse needs of patients with particular health issues, balanced against the requirements of industry
- **Capacity building**: Patients involvement address barriers to involving patients in ethical review and build capacity for patients and ethics committees to work together

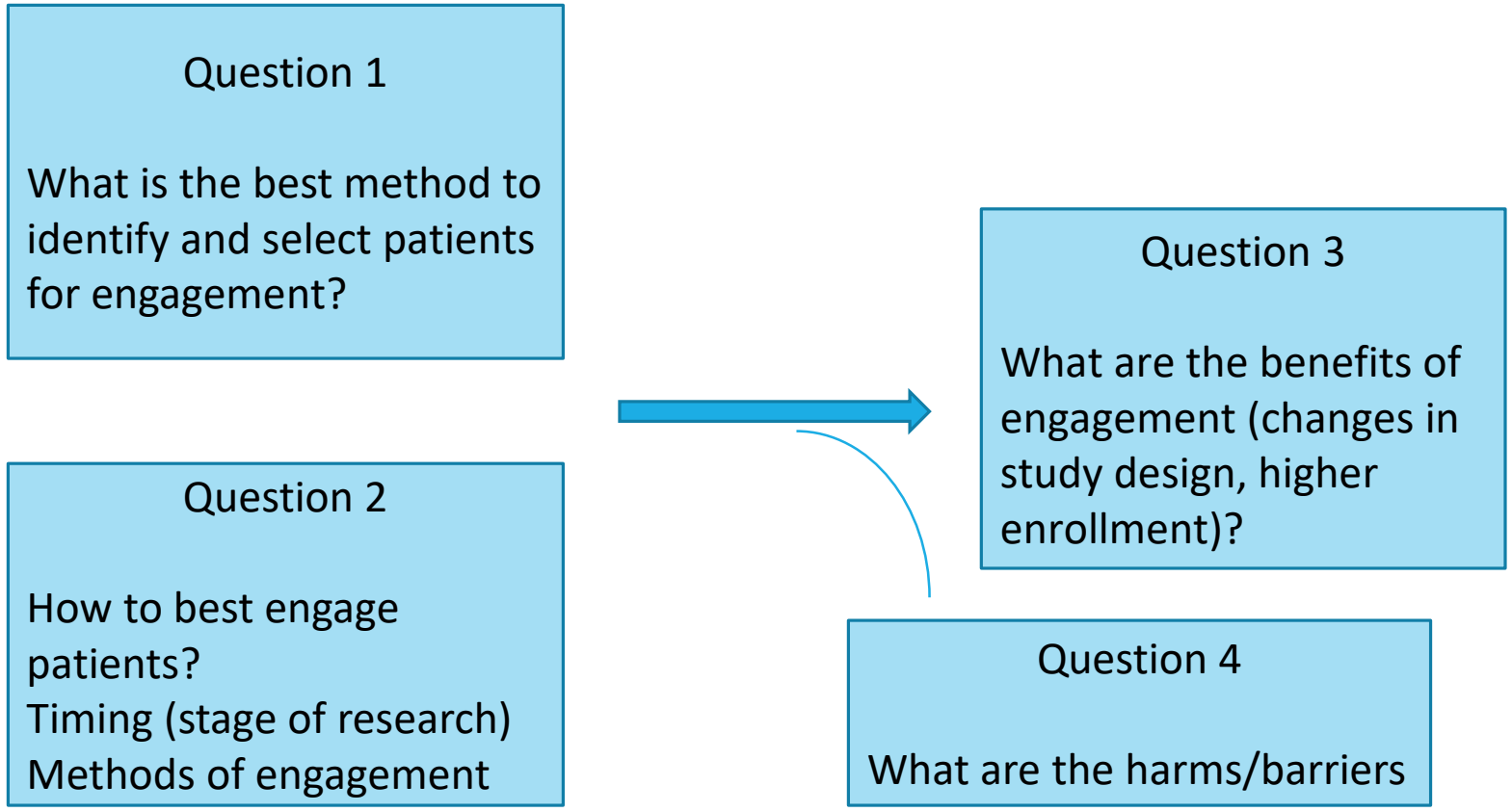
Patient involvement in ethical review



EUPATI
European Patients' Academy on Therapeutic Innovation
www.eupati.eu

Geissler, Ryll, Leto, Uhlenhopp
EPALCO/EUPATI (2015, unpublished)

Patients' engagement in Research



Patients' level of expertise

Individual patients **with a specific disease can provide valuable input** to the patient information sheet and informed consent/assent form with a view from outside and **can comment on aspects of a trial that will affect quality of life and the burden for participants**. However, they might not be research-naïve and it is argued that this could affect the value of their input. **It can be difficult** for research-naive patients **to take part in discussion of other ethical topics that involve scientific and/or methodological complexity**.

Patient advocates **have an in-depth knowledge of living with the disease** from their own experience **(grass root)** and might **have a level of understanding of research and medicines development for this disease**. Their **contribution** to ethical review of trials for other diseases **will be limited to a general patient perspective**.

POs representatives are either patient with a specific disease and **actively engaged in a relevant PO** and are exposed to the disease experience of many individuals. **They are knowledgeable about the needs, desires and opinions of this community** and thus will be relatively representative. Since POs exist to support their members and to lobby for their interests it is important to ensure that the PO **representative in the ethics committee is aware of the obligation to provide un-biased advice**. Their **contribution** to ethical review of trials for other diseases **will be limited to a general patient organization perspective**.

Patient experts **have personal experience** of living with the disease and/or the **combined knowledge from working with members of their PO**. In addition, they **have a comprehensive understanding of all aspects of the medicines development process, and can actively participate in all aspects of the ethical debate on the same level as the other ethics committee members**. They are not joining the ethics committee in a representative role but have much exposure to other cases due to their activities in their PO. **Their contribution to ethical review of trials for other diseases could also be valuable because of their knowledge of R&D.**

Current status of patients' involvement

Patients' involvement in ethical considerations on CTs as early as in trial design and protocol preparation stage can be beneficial to strengthen awareness on ethical issues.

Involvement at this stage can ensure the focus on patient is maximized while late involvement at the time of ethical review of CTs, protocol details have been decided yet.

Ethical review can ensure the acceptability of risk/benefit balance, patient protection elements as well as patient's information during the informed consent process. The addition of patients' specific expertise can be a relevant expansion of committee's expertise.

Participation of at least one patients' representative in ethical committees is longstanding practice and undisputed value but varies within European MS. Somewhere patients' participation is required by law and conditions are clearly defined. In other countries is still to be implemented.

Current status of patients' involvement

Although there is appreciation of the benefit of patient involvement there is no agreement on the role and most suitable patient profile: patient expert, patient advocate, patient organization representative or individual patient.

Finding patients willing to contribute to the ethical review is a challenge for ethics committees, and this is the case across Europe. There is no established match-making process.

Involving patients with specific diseases can be logistically challenging, while involving patients who advise on all kinds of diseases requires a level of knowledge beyond their personal disease.

There is disagreement about how far patients with a particular disease can and want to be representative for other patients with this disease, and whether there is potential for bias because of their personal interests. The independence of representatives from patient organizations has been questioned on the grounds that their personal interests and financial support from the pharmaceutical industry might lead to conflicts of interest.

Pan-European capacity of suitable patient experts is currently scarce.

Conclusions

A cultural shift is strongly demanded from patients: patients' representatives must be included deeply in the medicine development process.

Cultural shift means that unless we won't be able to consider the patient in a new light considering him as an added value an not only a consumer and unless the target of research will be a disease rather than a person we don't achieve any real progress.

Patients have built their capacities through POs' training courses, attending workshops and educational events at local, national and international level for gaining the knowledge needed for contributing with a meaningful activity in R&D and in CTs ethical review.

Patient representatives have been already involved in some of EMA and FDA Commissions but in many cases the representatives are not patients with the required knowledge and experience of living a specific-disease conditions.

There are still ongoing speculations on the role of patients and patients' representatives in R&D and in ethical review of CTs for the difficulties to give an official recognition to patients' role.

Nothing about us

Without us

Thank You