RARE CANCERS EUROPE

Joining forces for action

Patient Involvement in Clinical Trials

RCE-ESMO-ESO Training Course for Rare Cancer Patient Advocates

Jan Geissler, Markus Wartenberg

LOGISTICS FOR THIS SESSION

- All questions should be addressed via the chat box only. We will also take questions and comments from the floor during the discussion time. Please use the ”raised hand system” in Zoom.

- We kindly request to put your camera on during the discussion time.

- For any technical issues, please send us an email at: rarecancerseurope@esmo.org and we will get back to you.

- The above information will also be posted in the chat box, so everyone is aware.

Agenda of today

- What is a clinical trial?
- What input can patient advocates provide into clinical trial design and conduct?
- (Why should +) How can patient advocates be more involved in clinical trials?
- Discussion

Jan Geissler
WECAN, EUPATI, Patvocates

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SPAEN Sarcoma Patients EuroNet
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NCT Essen-Cologne (CCCE, WTZ)

Disclosures Jan Geissler

- Contributions to IMI-funded EU consortium projects HARMONY and EUPATI by EFPIA companies

- Support for Community Advisory Boards (CABs) provided by Amgen, Alexion, Celgene, Incyte, Janssen, Jazz, Novartis, Pfizer, Servier and Takeda

- Advisory roles and consultancy on patient engagement to Amgen, Alnylam, Bristol-Myers Squibb, Bayer, Biomarin, Janssen, Novartis, Pfizer, Roche, Servier, Takeda, UCB

What is a clinical trial, and how can patient advocates provide input?

Jan Geissler

Workgroup of European Cancer Patient Advocacy Networks (WECAN)
European Patients' Academy (EUPATI)
Patvocates

What is a clinical trial?

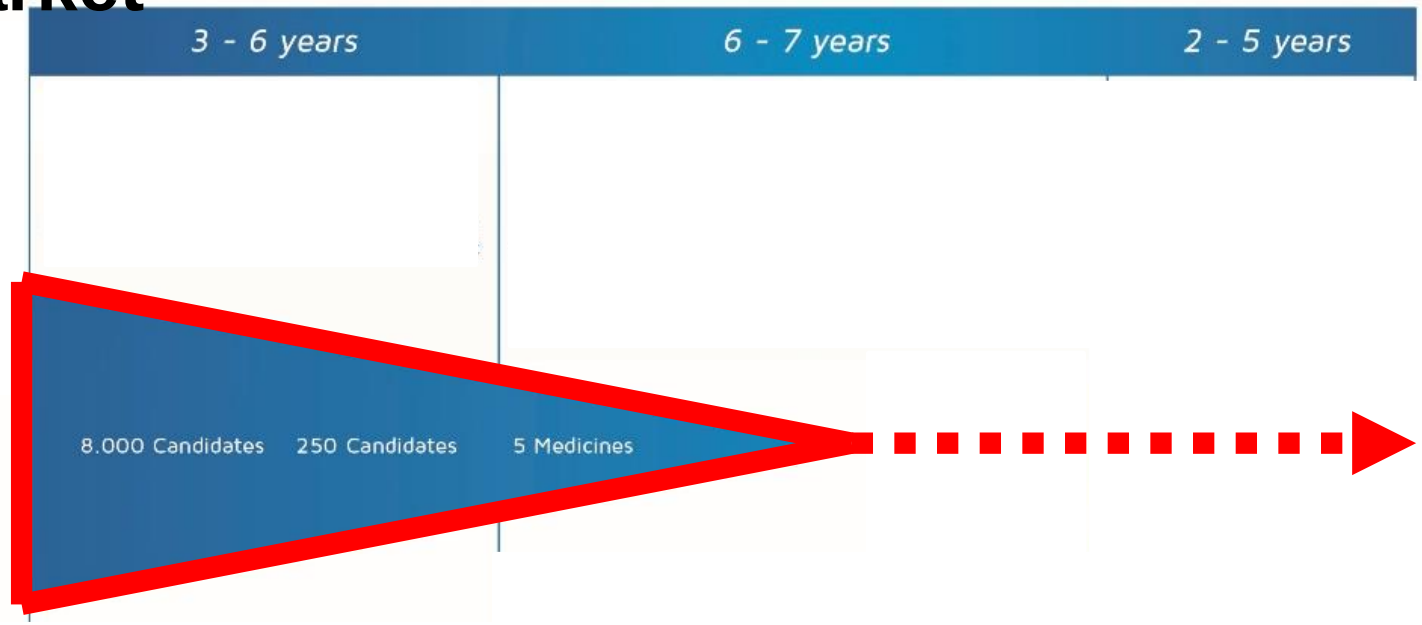
- A clinical trial is a clinical study in **which (human) participants are assigned according to a pre-defined therapeutic strategy or plan (protocol) to receive a health-related intervention, such as a medicine, in order to investigate its effects on health outcomes**, usually compared to another (or sometimes no) treatment.

- Clinical trials are used to **evaluate clinical practices** that do not fall within the current practices, or to **evaluate a new medicine** (investigational medicinal product).

- Clinical trials are used to **generate data on the safety and efficacy** of the intervention.

- Clinical trials are conducted only after a **regulatory authority approval and ethics committee review**.

In medicines development, of 8.000 molecule candidates, only 5 ever get into human clinical trials, and only 1 makes it to market



It takes over 12 years before a new medicine can be made available to patients and reimbursement starts.

Only about 2% of substances evaluated in early research make it to the market as new medicines

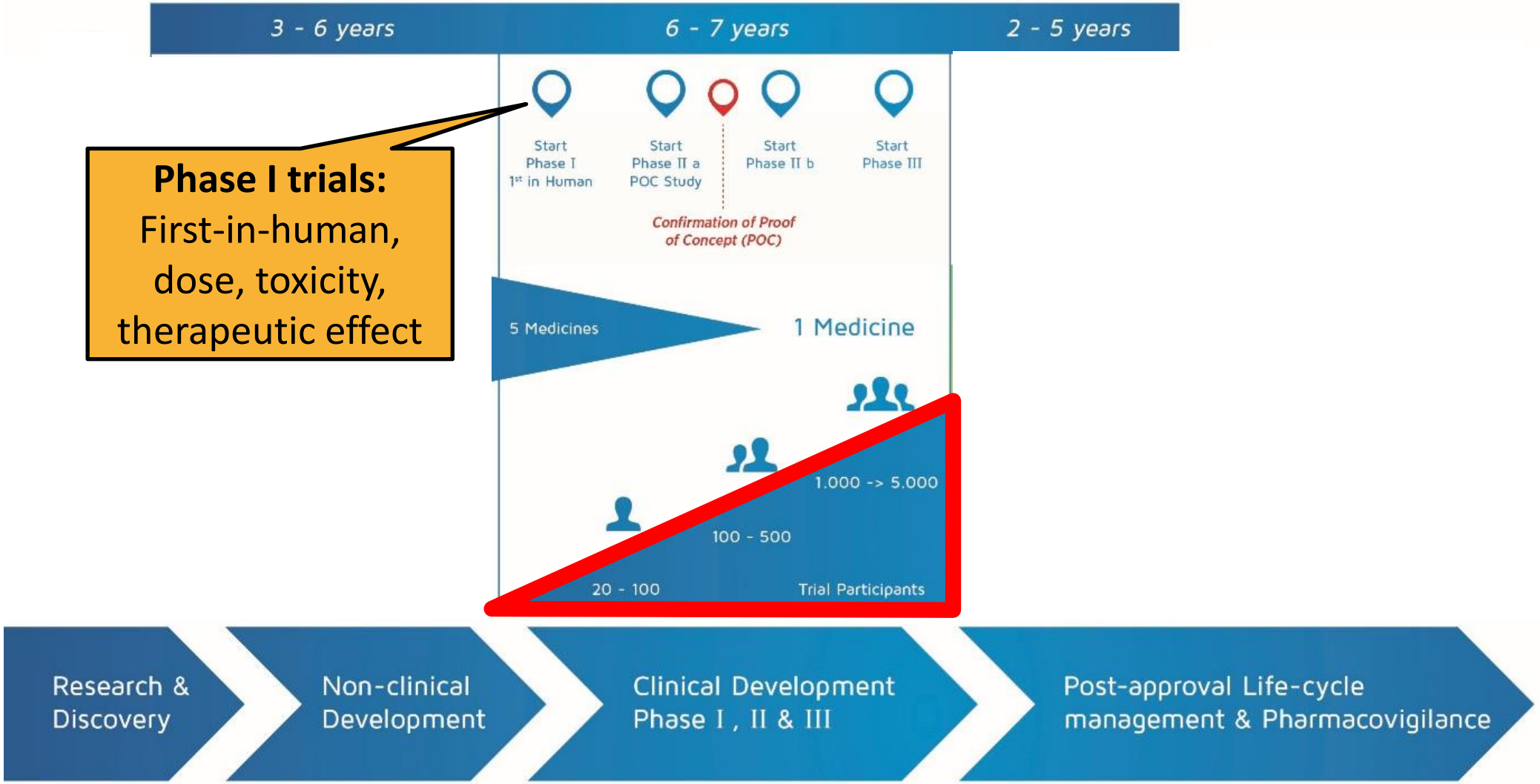
Source: EUPATI.eu

Phases of medicines research & development

Source: EUPATI.eu

In different phases of clinical trials, dosing, safety and efficacy are tested in an increasing number of patients

Phase I trials:
First-in-human, dose, toxicity, therapeutic effect



Source: EUPATI.eu

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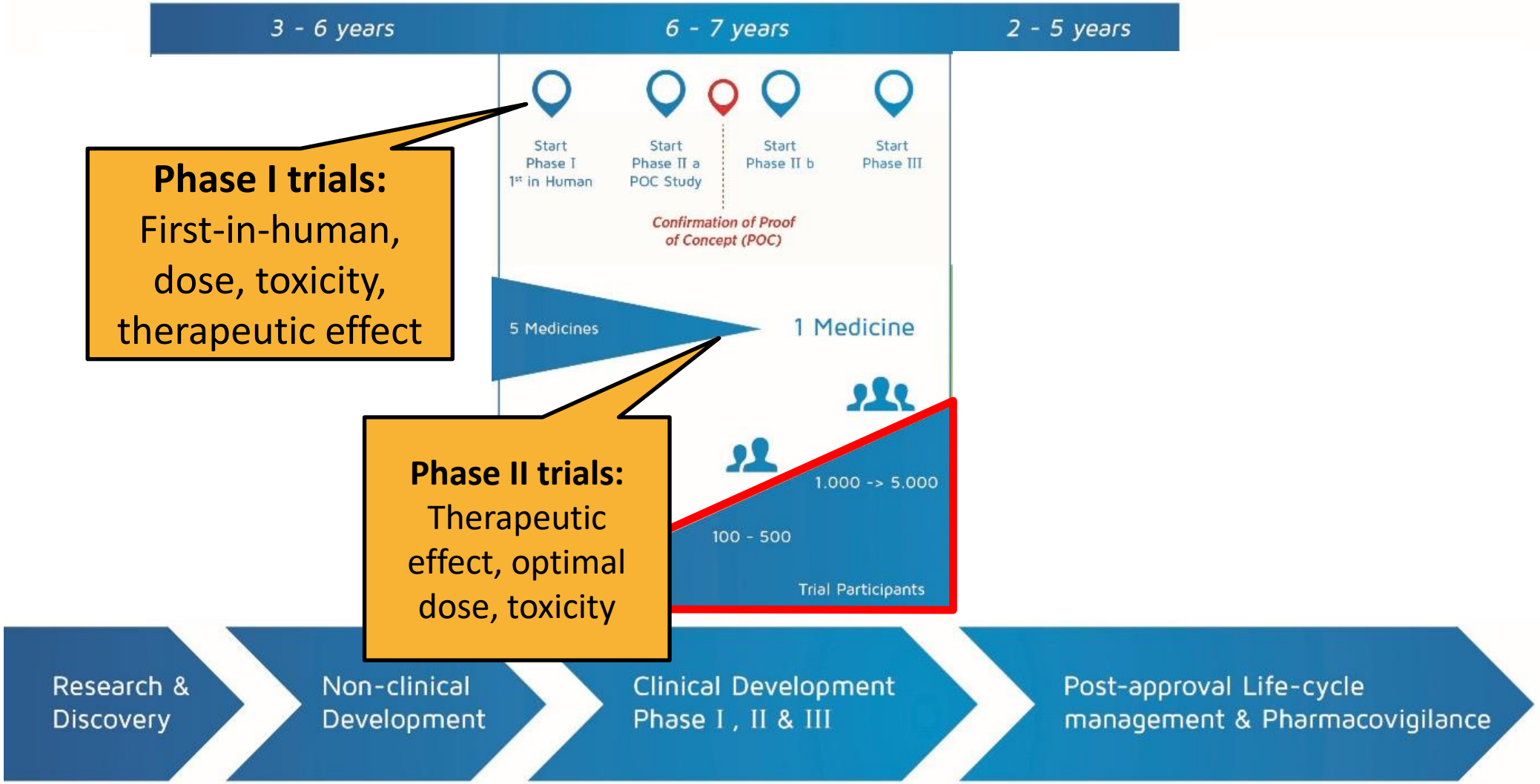

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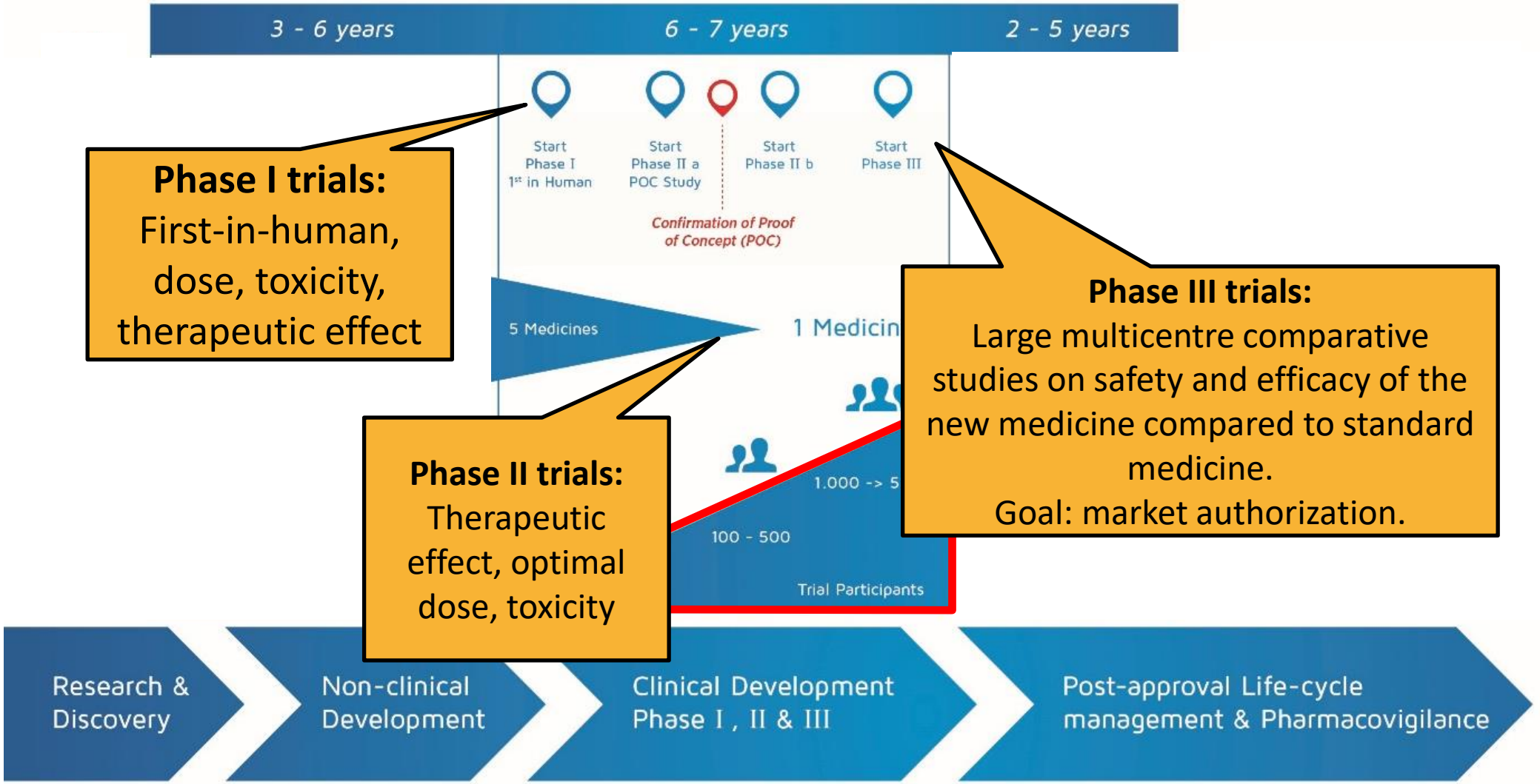


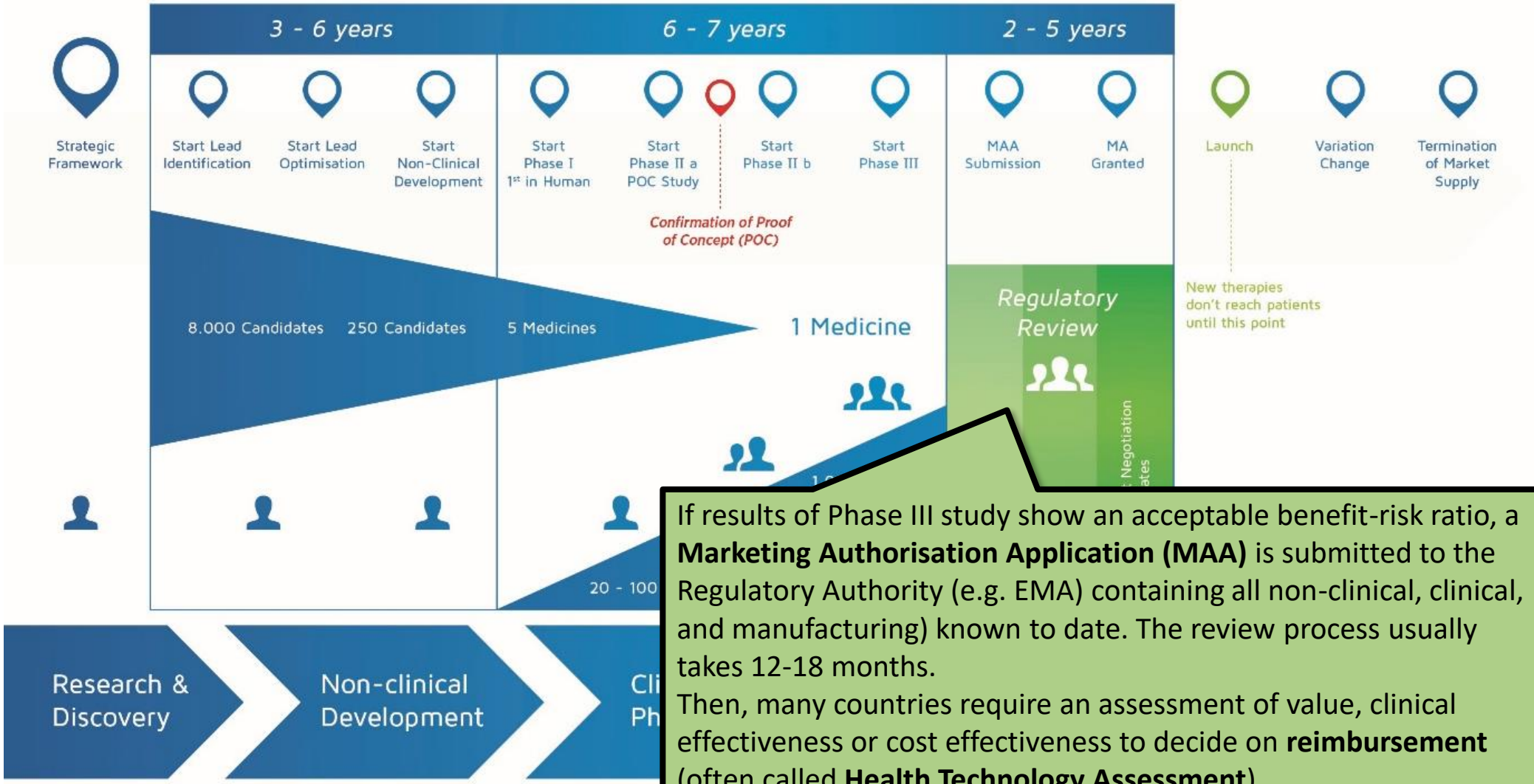
Phase I trials:
First-in-human, dose, toxicity, therapeutic effect

Phase II trials:
Therapeutic effect, optimal dose, toxicity

Phase III trials:
Large multicentre comparative studies on safety and efficacy of the new medicine compared to standard medicine.
Goal: market authorization.

Source: EUPATI.eu

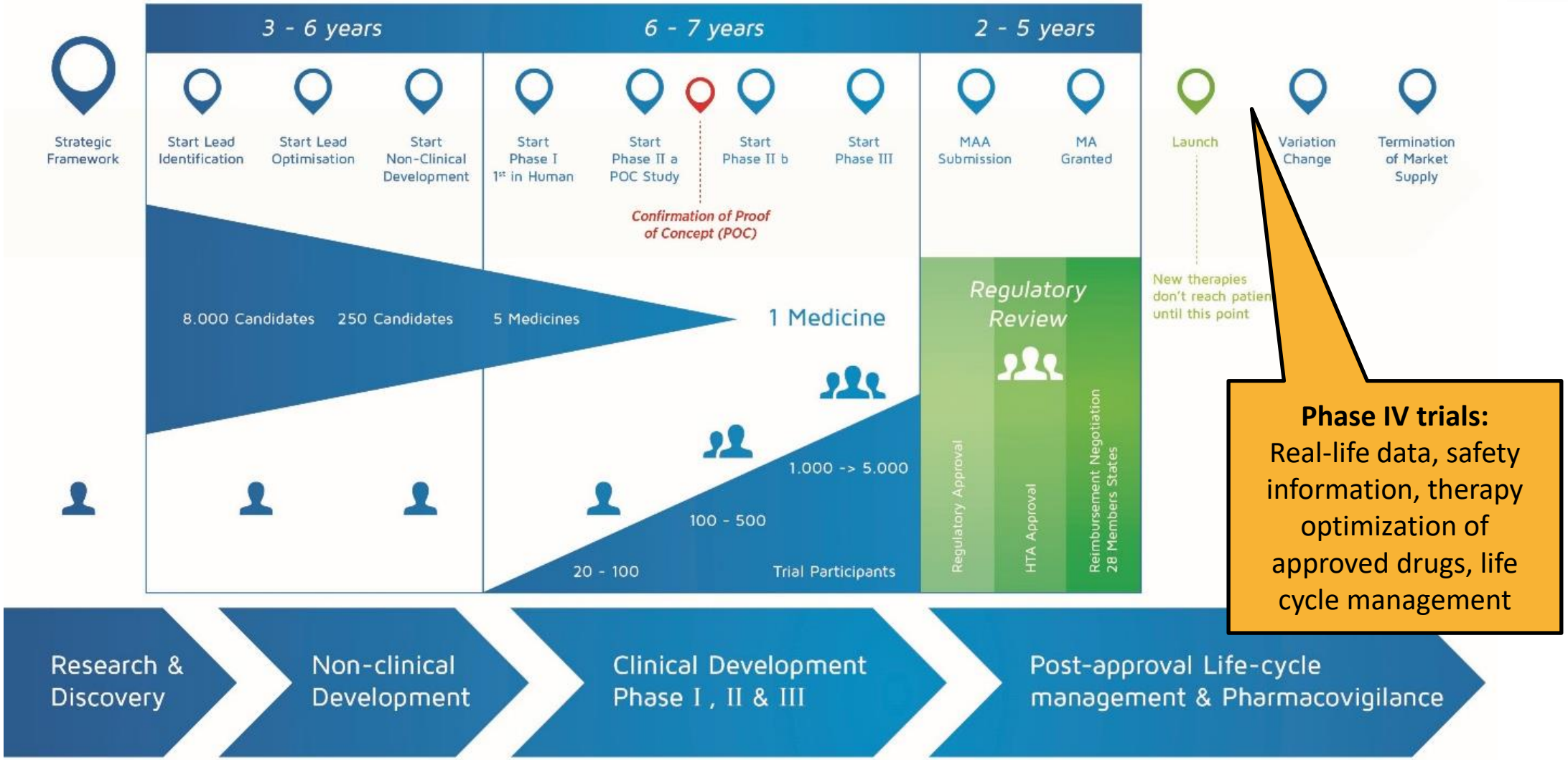
Regulatory review: Marketing authorization



If results of Phase III study show an acceptable benefit-risk ratio, a **Marketing Authorisation Application (MAA)** is submitted to the Regulatory Authority (e.g. EMA) containing all non-clinical, clinical, and manufacturing) known to date. The review process usually takes 12-18 months.
Then, many countries require an assessment of value, clinical effectiveness or cost effectiveness to decide on **reimbursement** (often called **Health Technology Assessment**)

Source: EUPATI.eu

After regulatory approval decision, late-phase trials optimize therapy and collect more (safety) data



Phase IV trials:
Real-life data, safety information, therapy optimization of approved drugs, life cycle management

Source: EUPATI.eu

(Why should +) How can patient advocates be more involved in clinical trials?

Markus Wartenberg

SPAEN Sarcoma Patients EuroNet (www.sarcoma-patients.eu)

Deutsche Sarkom-Stiftung / German Sarcoma Foundation (www.sarkome.de)

NCT Essen-Cologne (CCCE, WTZ Patient Advisory Board)

Declaration of Interests

2019 - 2021:

- *No direct or personal payments from researching industry*
- *No consultancy or financial research relationships*

Sustaining Partnerships, grants, project-sponsorships or honoraria only directly to the organisations:

- *SPAEN Sarcoma Patients EuroNet e.V. (since 2009)*
- *German Sarcoma Foundation (since Jan. 1st, 2020)*

K. Oliver: Some of the Challenges of Rare Cancers…

- Prevention and screening mostly irrelevant
- Late or incorrect diagnosis
- Lack of information and patient groups
- Widespread, small populations of patients
- Shortage of local medical expertise
- Incomplete registries and tissue banks
- Methodological barriers (in traditionally-designed clinical trials)
- Not enough clinical trials for rare cancers
- Regulatory barriers
- Reimbursement challenges for treatments
- Inequitable access to therapies and care
- Stigma

Rare cancer (five year relative) survival is worse at 48.5% than common cancer at 63.4%. *

*** Lancet Oncol. 2017 Aug: 18 (8) 1022-1039**

With kind approval from Kathy Oliver, IBTA

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Several of these challenges have directly or indirectly to do with deficits in cancer research!

With kind approval from Kathy Oliver, IBTA

Engagement of Patient Groups in Clinical Research…

In Germany today: Only rarely in Oncology…

WECAN Academy

- Clinical studies: Basic knowledge (information transfer)
- Publication of current or planned trials
- Making studies more understandable to patients
- Supporting the recruitment e.g. in rare "subtypes" or reg. spec. mutations
- **Patient Groups & Clinical Trials**
- Cooperation with information / education "Informed Consent"
- Quality of Life Aspects
- Publication/ dissemination of study results
- **Research-Involvement??? In a very early stage in Germany…**

Why “Patient Involvement in Clinical Research”?

And why should patient advocates be interested in?

The meaning of medicine & health care >>> support to / benefits for people! Patients (relatives) must be the focus – they are the real "customers“

- Everyone can be a patient / relative tomorrow (including doctors, researchers, politicians!)
- Patients = are a cross section through our society
- Patients are citizens, voters, taxpayers, contributors!!! They/We have rights to be involved, to be listened to!

Patients bear the ultimate risk in clinical trials: The "risk for their lives" – through e.g.

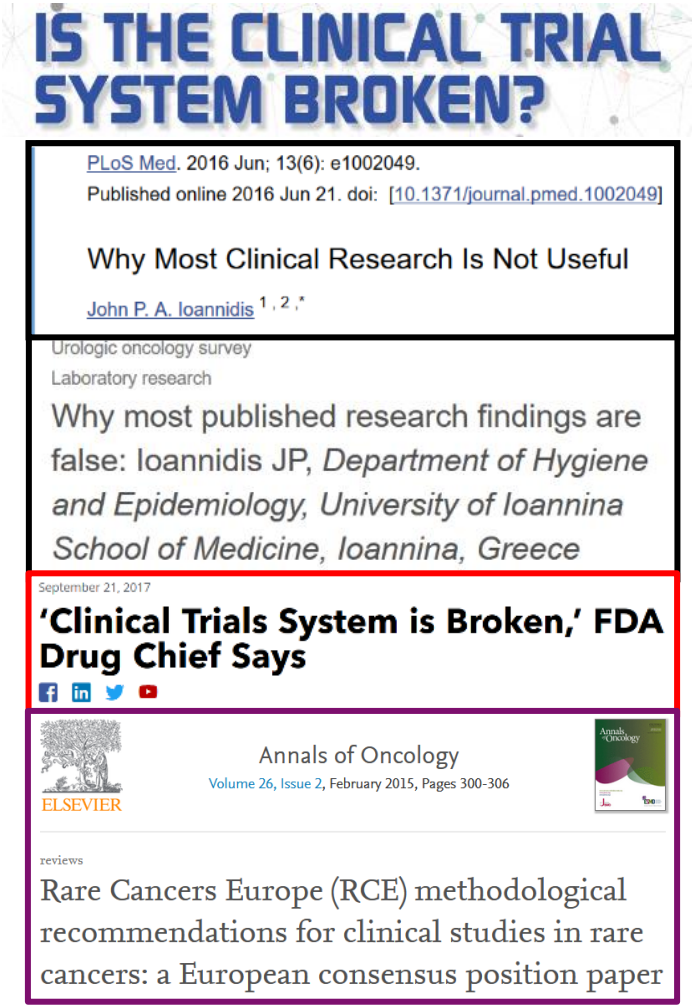
- new drugs in clinical trials
- wrong research questions
- inappropriate study designs
- missing / insufficient information
- delays in research & development
- lack of access to therapies due to cost…
- etc.

Patients know best – what it means to have a disease, to live with a special condition!

- Patients are the real experts!
- The views of doctors and patients can differ
- Rare Cancers: Patients often know more than their doctors
- Patient Groups have access to the very first source of information – to the patients / relatives
- New field: Personalised medicine / precision oncology…

Increasing criticism of the “old” study system…

- Focus on the patient: often ignored / neglected
- Results of Clinical Trials:
  often far away from real life and real needs
- Over 1 million studies published worldwide:
  Some experts complain: 85% are useless, waste of money
- A lot of resources are spent into:
  Doing things right – instead of – doing the right things
- Challenges with Clinical Trials in Rare Cancers:
  By definition - clinical evidence - is more difficult
  to build in rare than in frequent cancers…
- Difficult: Gaps between FDA/EMA approvals and HTA
- Often research is driven by company objectives,
  financial interests, academic reputation/carrers/publications,
  research funds, utilization of study units, etc.



Trying to describe a vision…

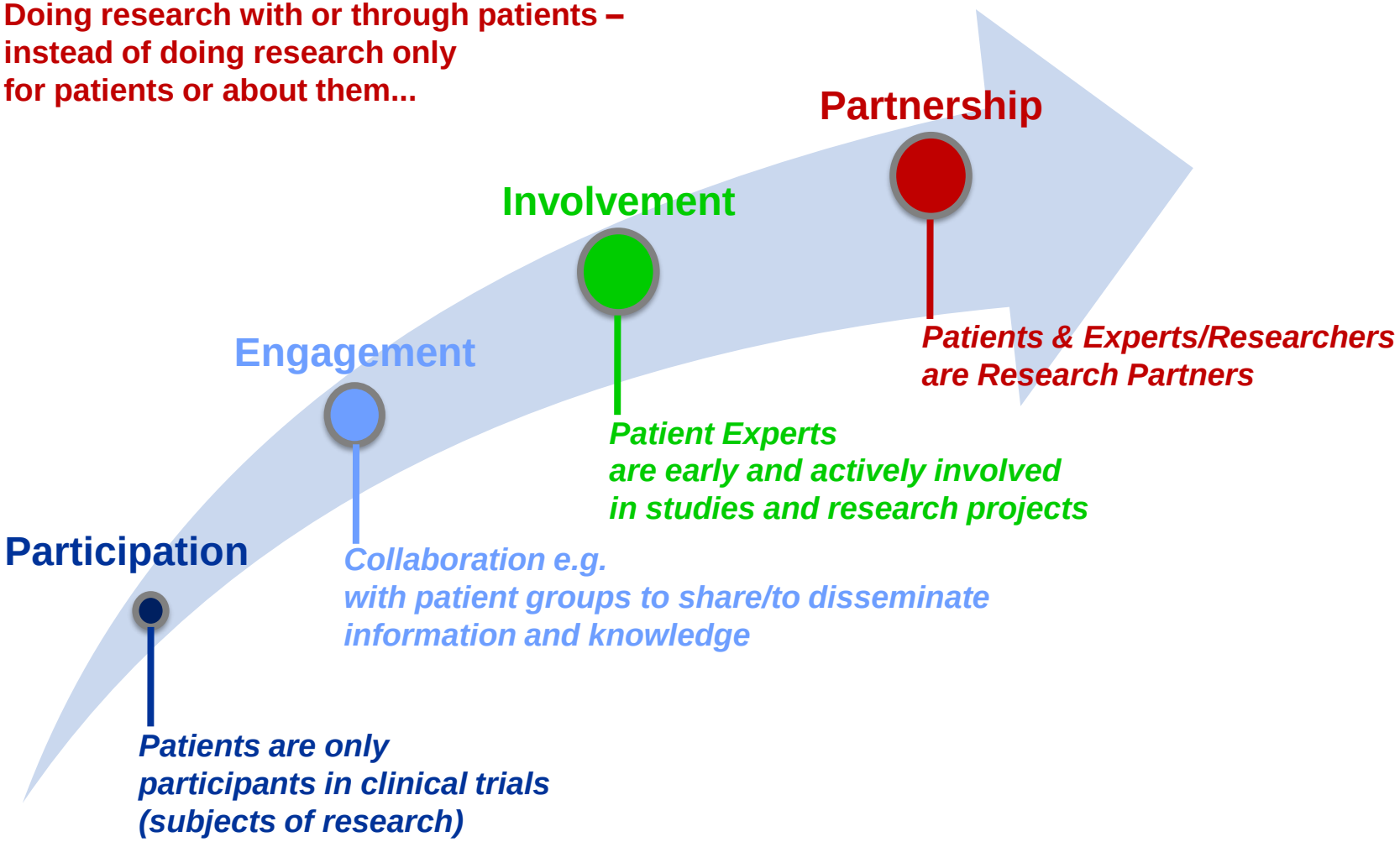
Clinical Research is not an end in itself! Ultimately, it

- **should benefit patients and the society,**
- **must lead to more effective > better (more affordable) therapies >**
- **thus to increased knowledge and benefits/values.**

"**Patient Involvement in Clinical Research**" is the permanent dialogue with the patient and her/his relatives (i.e. the real customer).
It is a partnership process between patients (s. t. expert-patients) and researchers / medical professionals >>> to bring questions, insights, experiences, wishes, preferences, real life information of/from patients early on in the R & D Process.
This is done on a peer to peer level and with the common goal –
to conduct research faster, more efficient, more target-/patient-oriented – closer to real life and of higher quality!

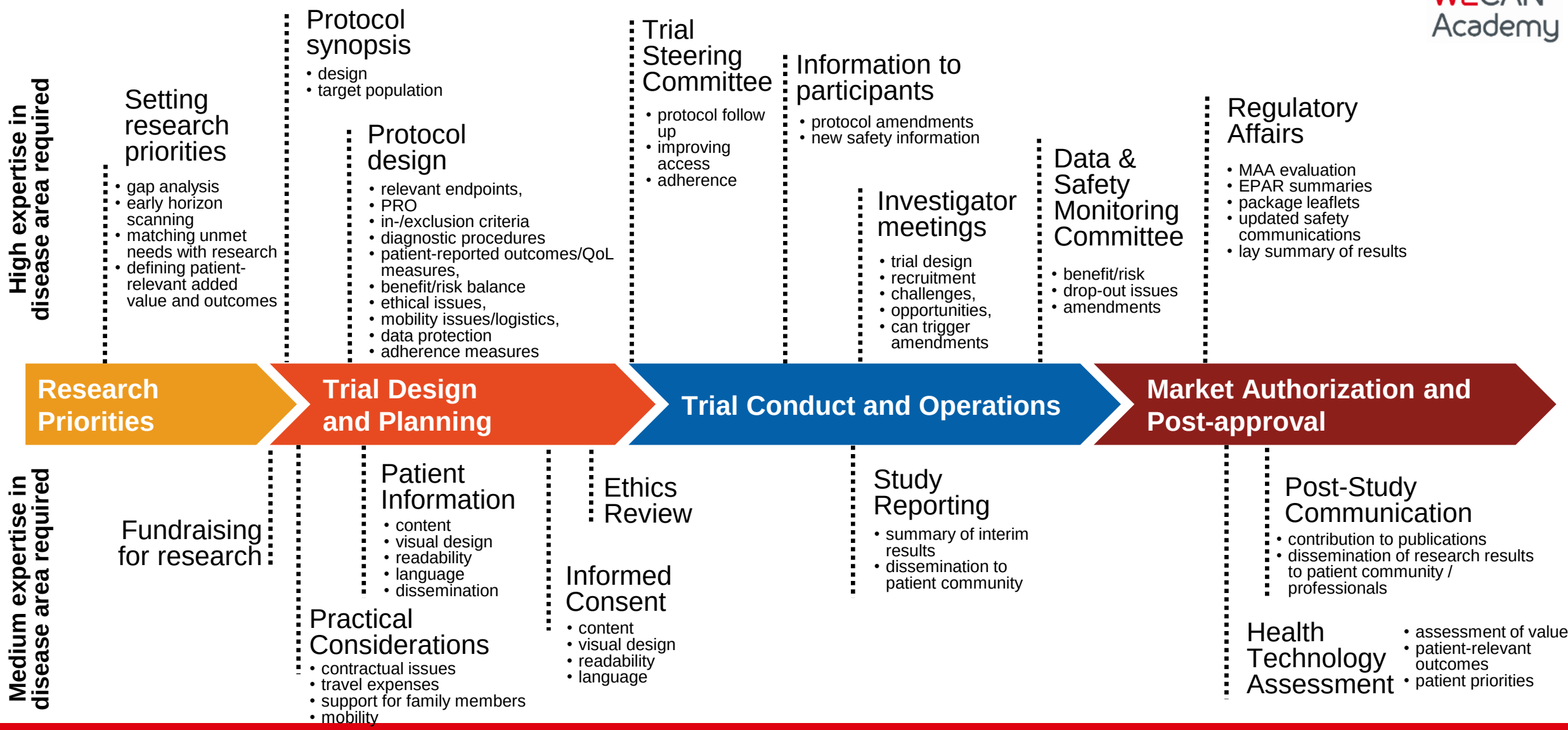
Early Patient Involvement in Clinical Research…

In the future:
Doing research with or through patients –
instead of doing research only
for patients or about them...



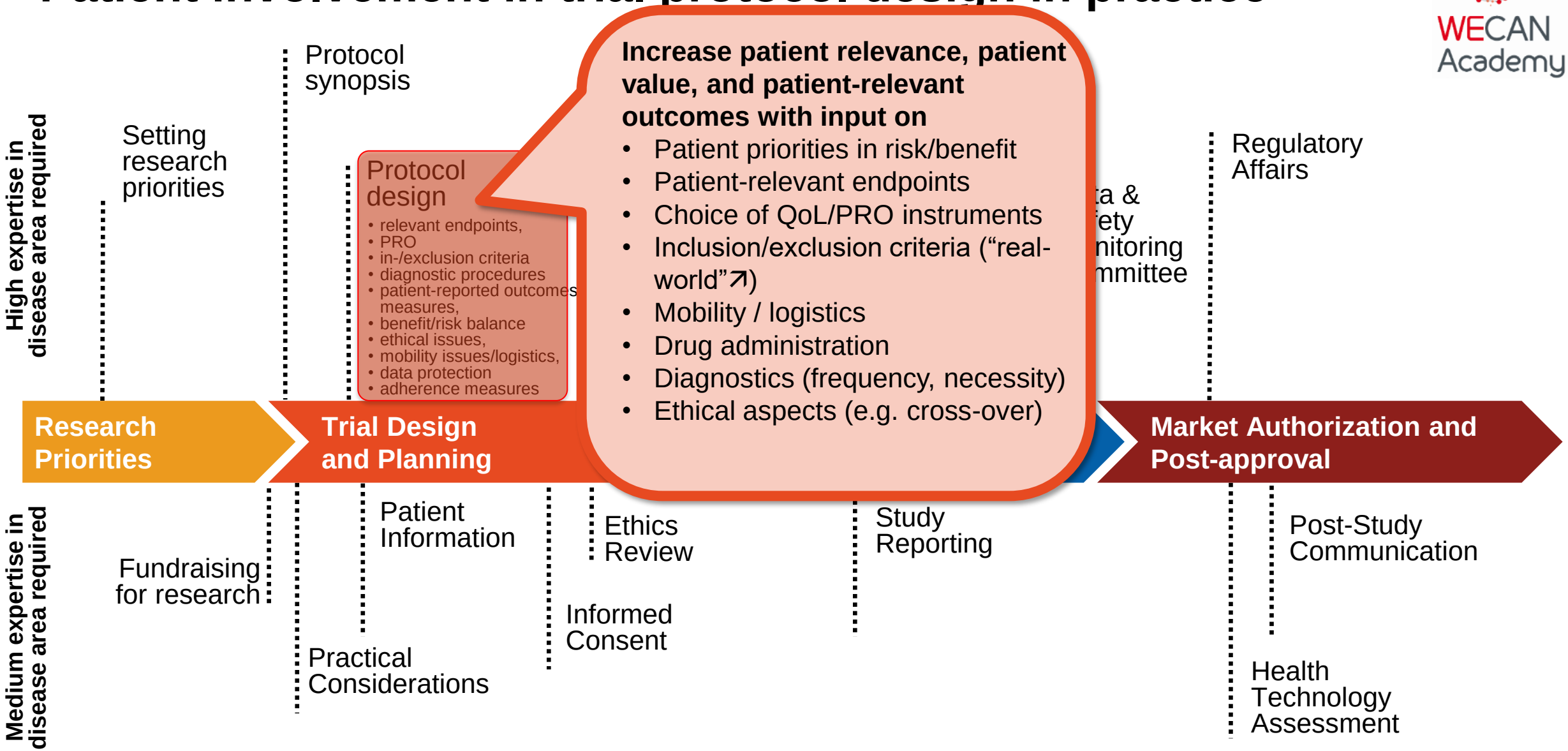
- *Research = better - faster – more efficient and more meaningful = closer to real life / to real needs*
- *studies = part of cancer treatment and part of the patient experience*
- *better recruitment*
- *change of scientific culture*
- *more public confidence in research*

Patient involvement in clinical development in practice



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

Patient involvement in trial protocol design in practice



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Some aspects for “rare cancer patient organisations”…

“Patient Involvement in Clinical Research”…means…

…enormous opportunities for change…

…to understand how research works and which language is spoken =
Basic Research Training!

…to understand how research in your country and your disease is organised…

…close peer to peer cooperation with experts, researching companies and other stakeholders…

…to prepare your organisation to be part of the research process...

…to analyse and define where in your disease the needs/gaps are and the research priorities should be…

…a general changing mindset, that this topic is a core success factor for research and not just a nice PR idea…

…to understand that countries are differently developed on this topic. E.g. UK has a history in cancer for more than 20 years…

…(maybe) to unite with other patient organizations to make general progress on the topic…

Discussion & questions

- In your country: Do you see a **changing mindset** of medical experts/researchers to involve patients/patient advocates in clinical trials / in clinical research?
- What are the **main challenges** you experienced as patient advocates in engaging in the design and conduct of clinical trials?
- What are the specific challenges of **rare cancers** when engaging in clinical trials?
- If you engaged in clinical trials design or conduct, where was **your engagement** as a patient advocate in clinical trials **most effective**?
- Do you feel **well prepared, well trained** to take part in such project?

Use Zoom “Q&A” and/or “Raise Hand”