EUPATI
European Patients' Academy on Therapeutic Innovation

The basics of medicines development, and how to educate yourself about medicines research

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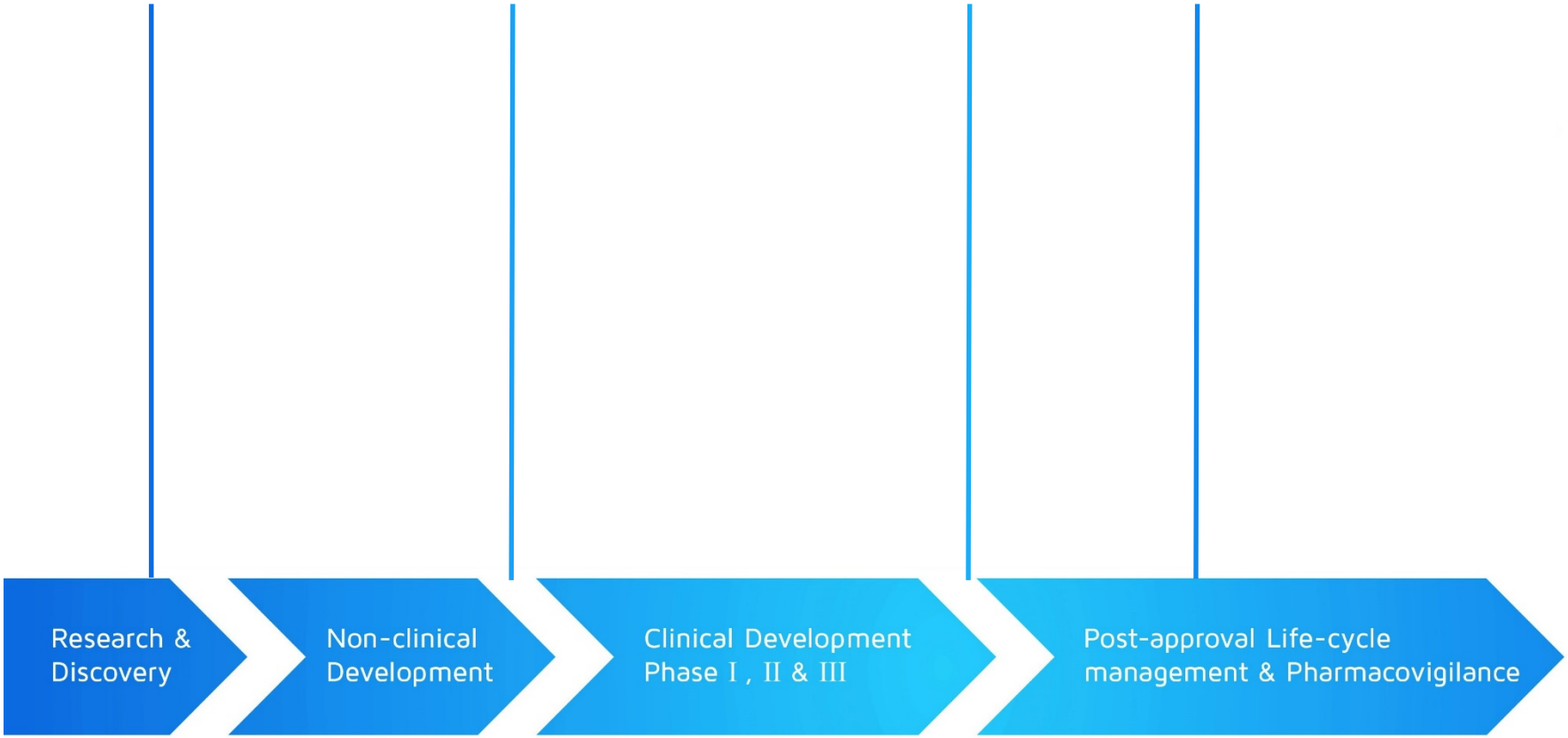
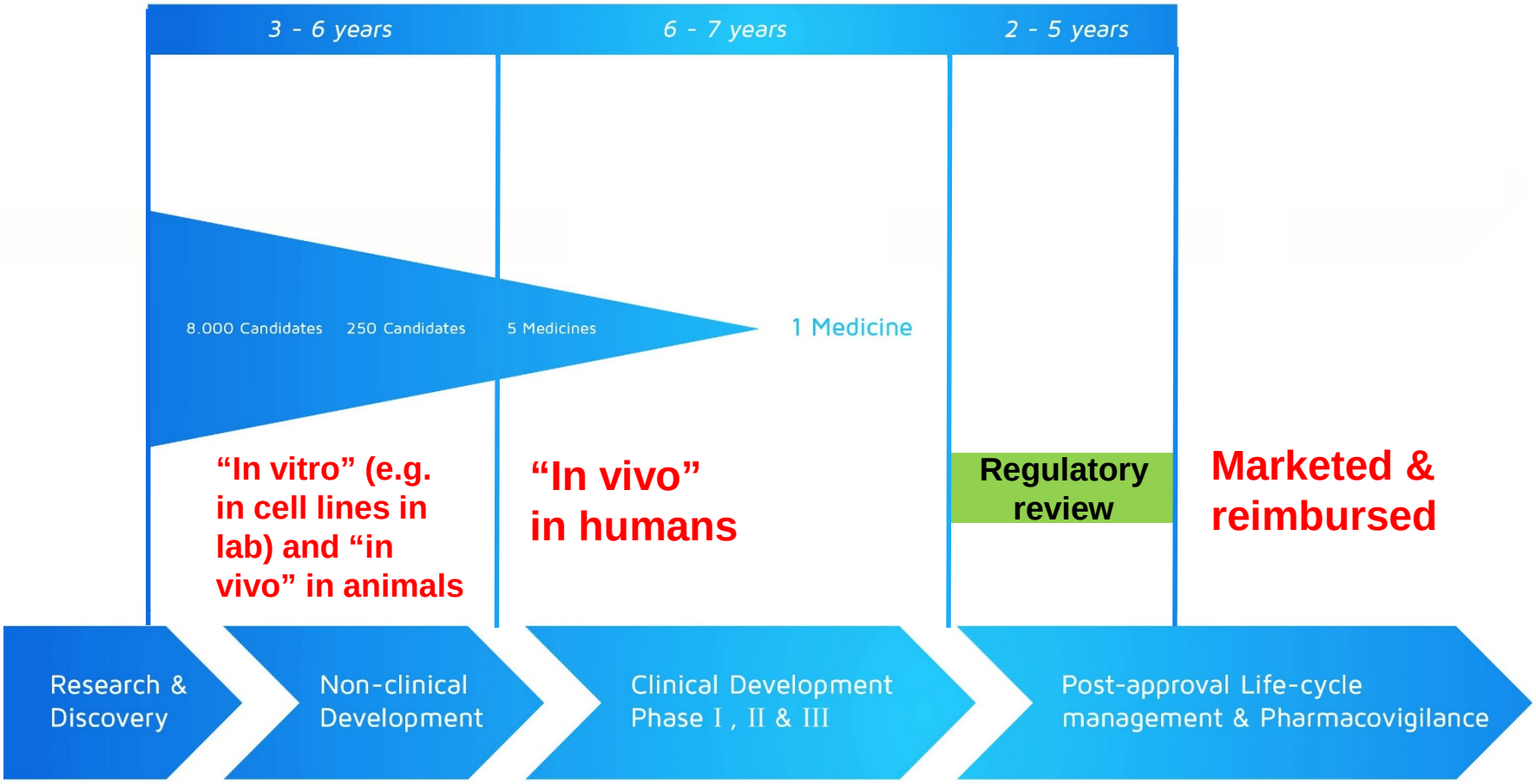
Hope and Hype

- It takes over 12 years and on average costs over €1 billion to perform all research and development before a new medicine can be made available to patients.
- Only about 2% of substances evaluated in early research make it to the market as new medicines.
- “Hope and hype”: CML patient advocates need to understand the development stages of new medicines to interpret their potential.

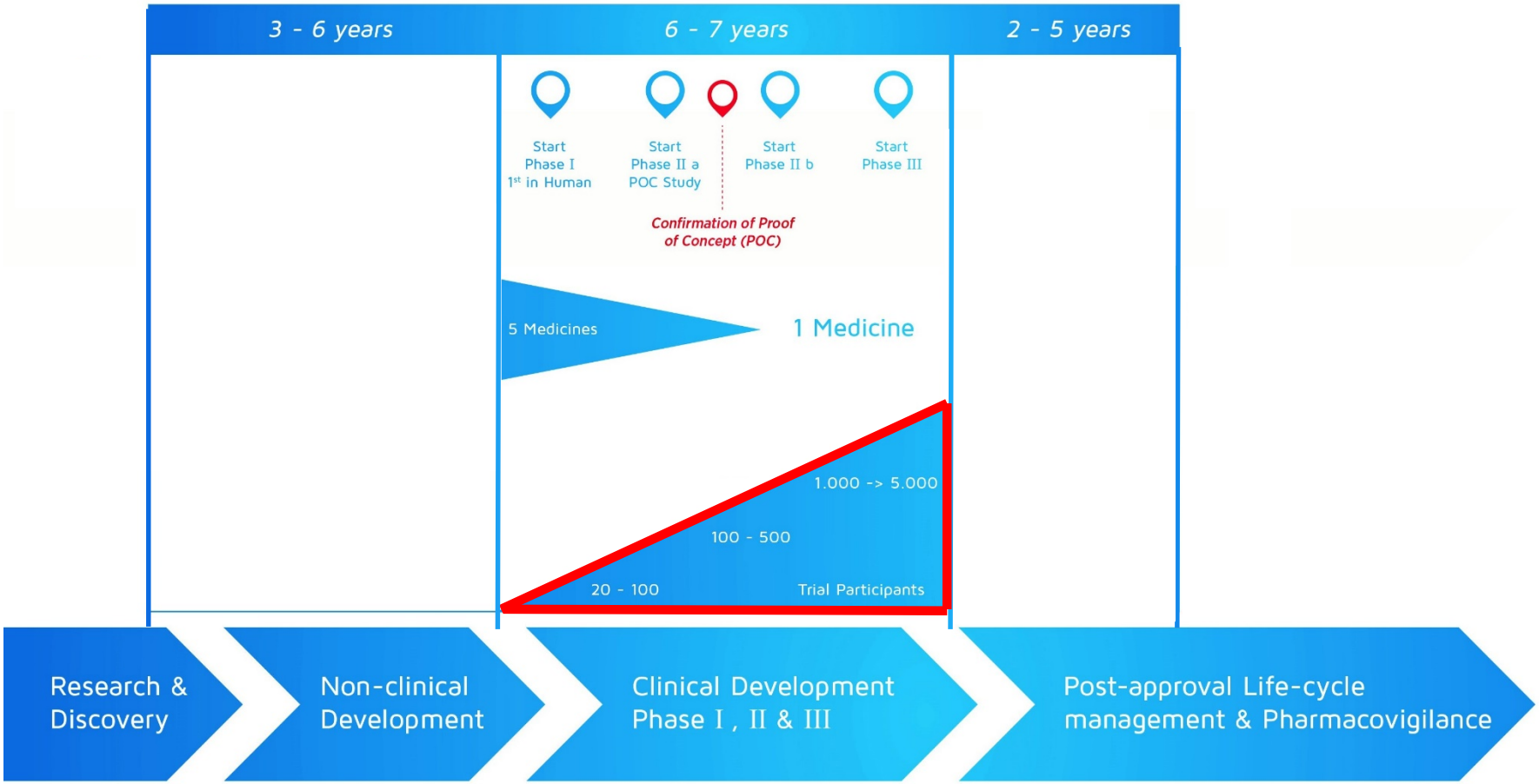
Phases of Medicines Research & Development



Of 8.000 molecule candidates, only 5 ever get into human clinical trials, and only 1 makes it to the market



In different phases of clinical trials, dosing, safety, efficacy is tested in an increasing numbers of patients



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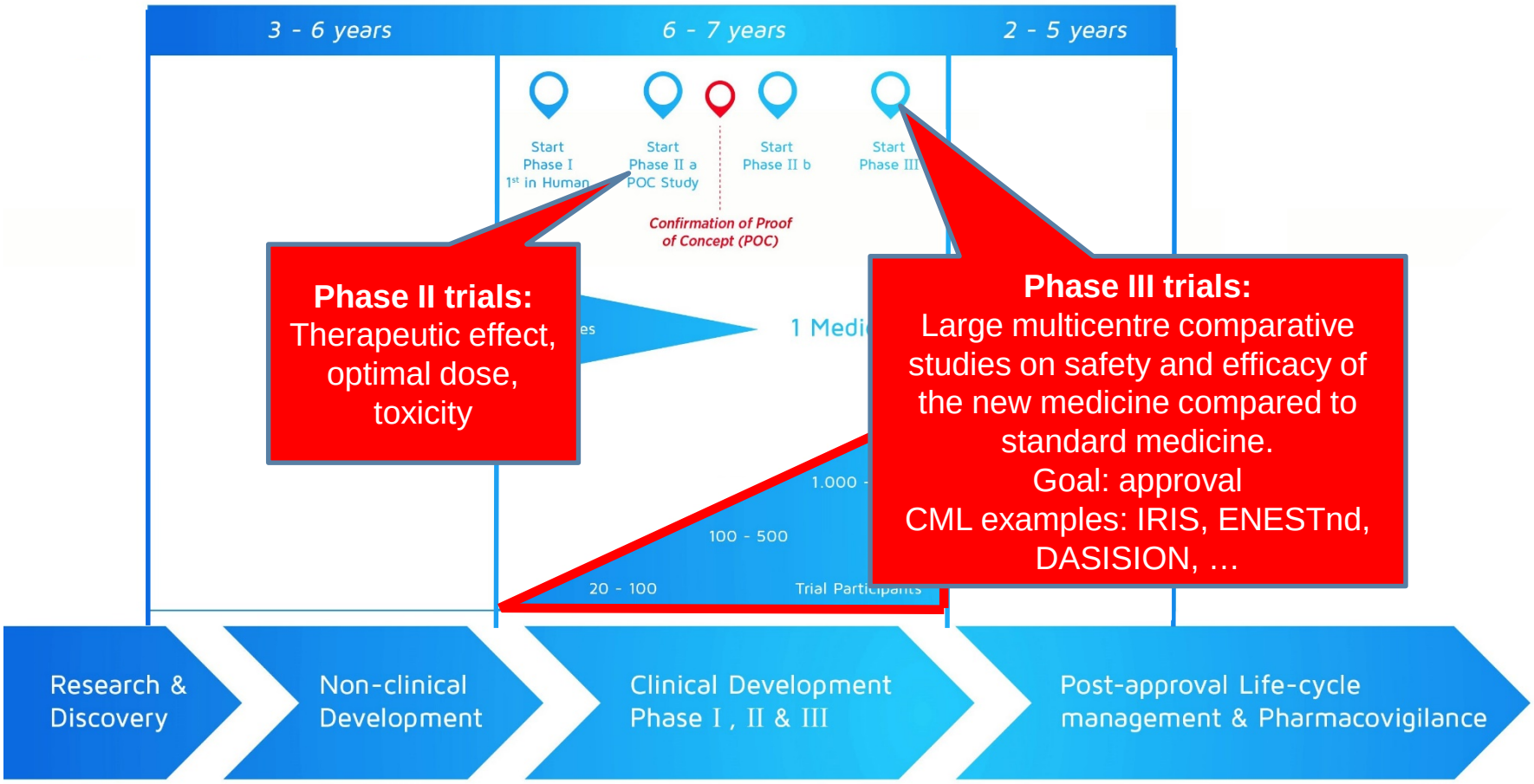
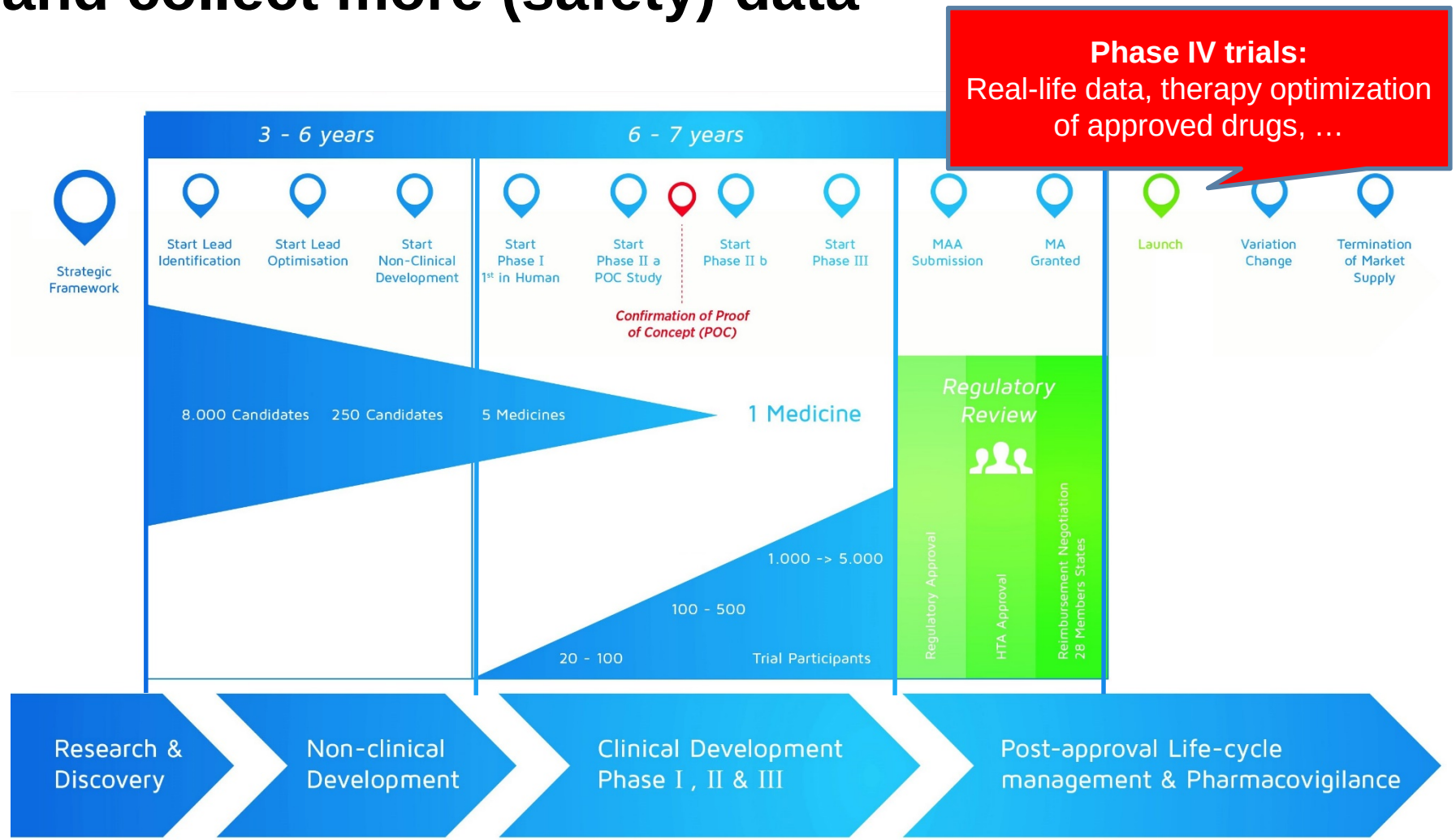
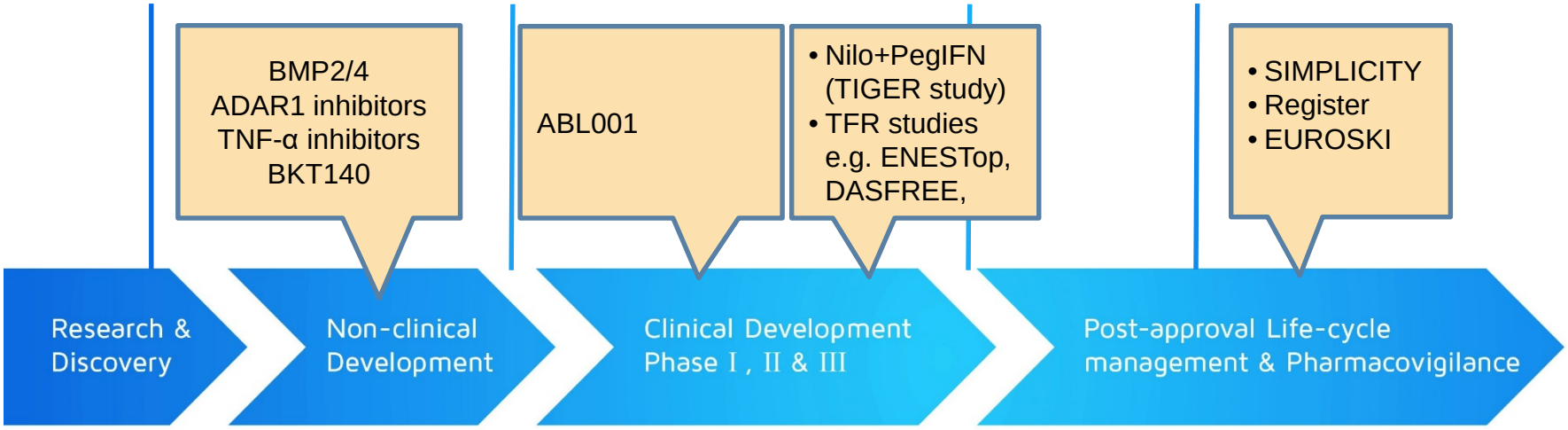


After regulatory approval and reimbursement decision, late-phase trials optimize therapy and collect more (safety) data

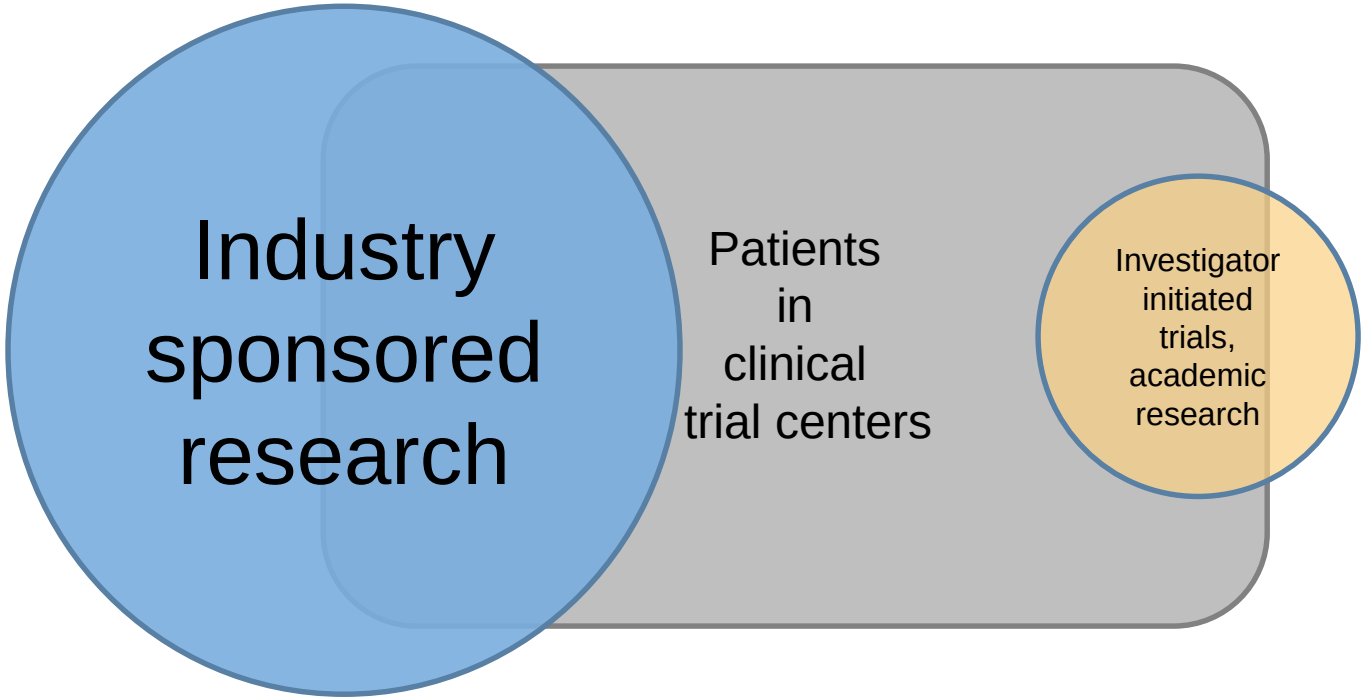


So what does this mean in CML?

- CML is still a dangerous chronic disease without a cure for every patient.
- Future CML therapies are in different stages of development.
- Many Phase I candidates will not work, some might bring the cure.
- To interpret the “hope and hype”, we need to look carefully where they are in the development cycle.



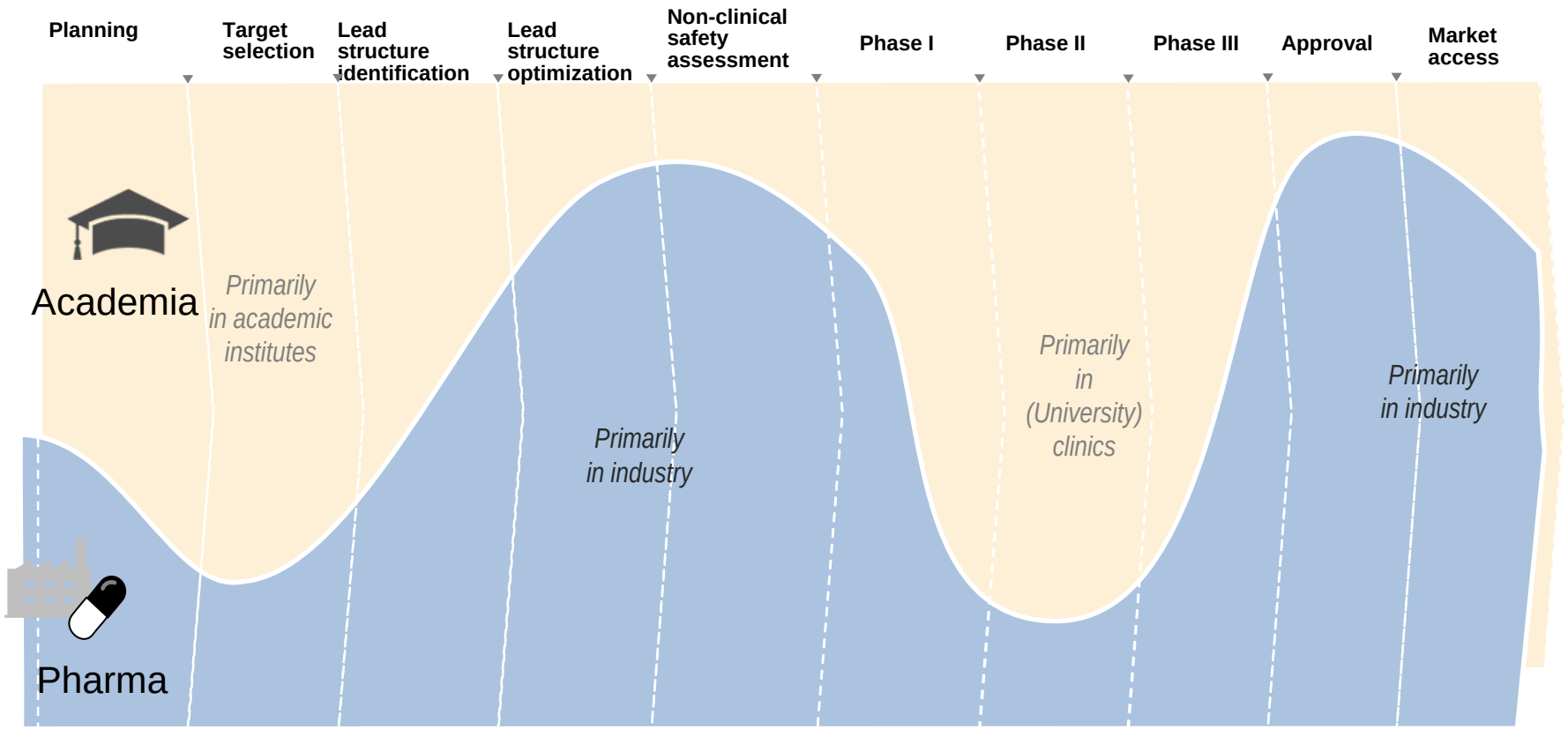
Most CML trials are initiated/sponsored by industry and then run at academic and community trial centers. Not so many CML trials are purely academic.



More targeted towards on assessment and regulatory approval of new drugs, new indications, new regimens

More focused on therapy optimisation, long-term outcomes, understanding disease biology, real-life care

Academia *and* industry are both involved -- not either/or



Why patient involvement in R&D?

Addressing public scrutiny and distrust in research

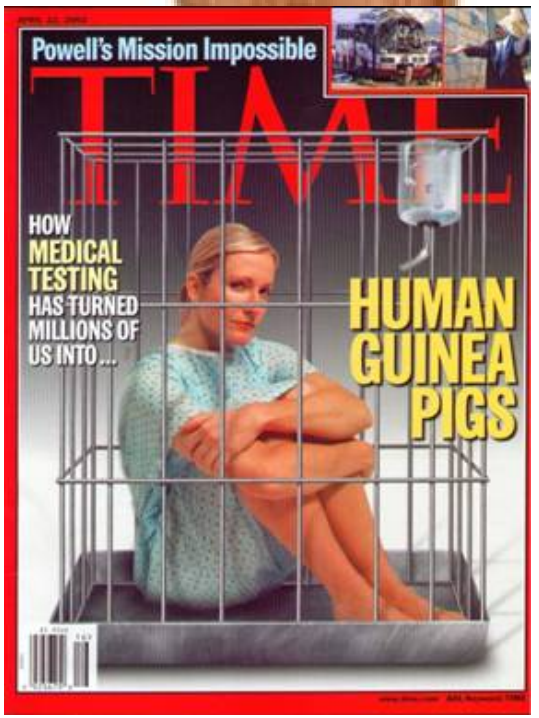
- **Only 6-12% of cancer patients participate in clinical studies**
- **75% of Phase II-IV studies delayed** due to slow patient recruitment
- Bad image one reason for delayed generation of meaningful clinical data

MEDICINE AND THE MEDIA

"We saw human guinea pigs explode"

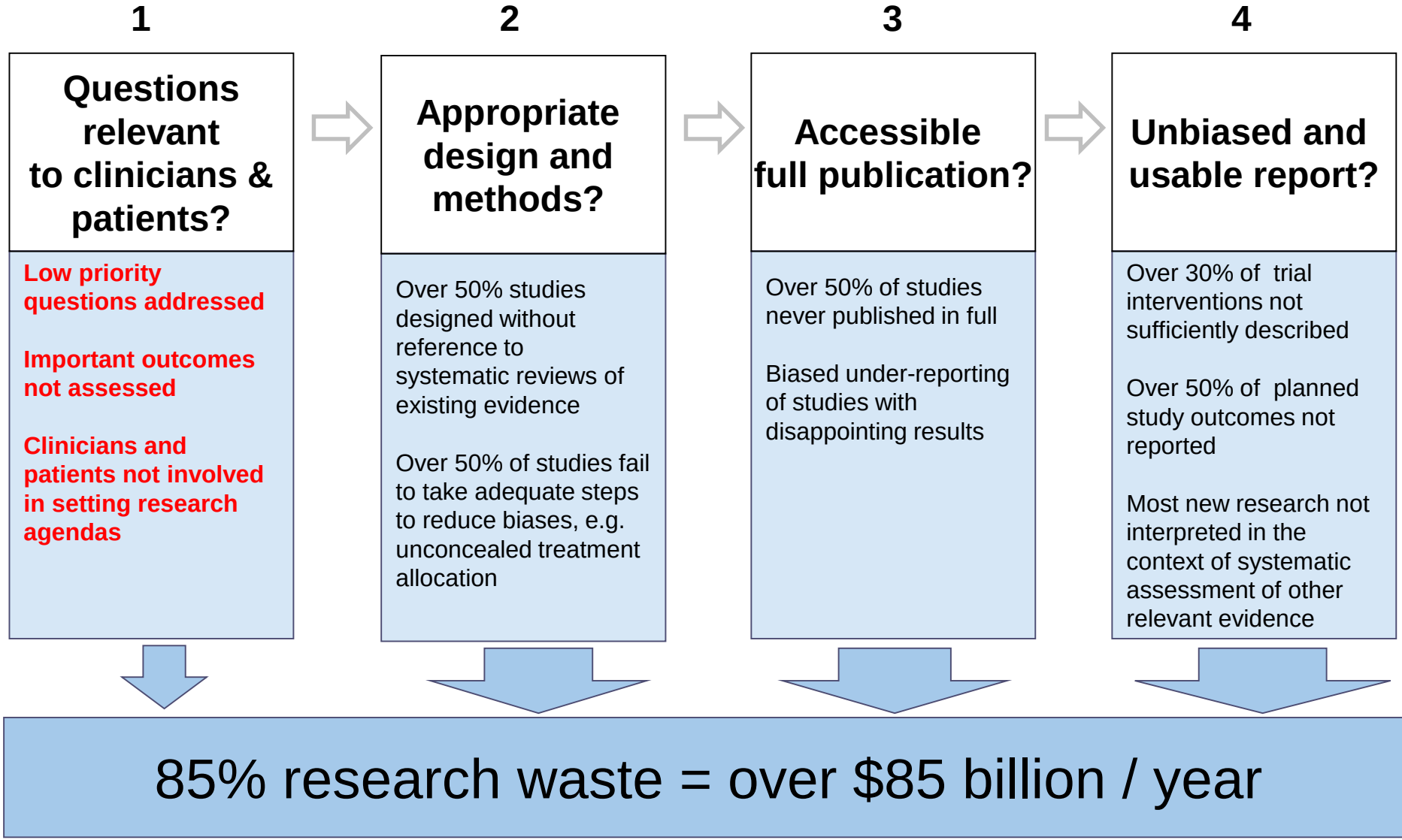
L Stobbart and colleagues examine newspaper coverage of adverse events in the TGN1412 trial

with death and disfigurement. Science fiction or cinematic imagery is often used to add potency to detailed and gruesome descriptions—although no pictures were printed of the victims' deformities, references such as "his face now resembled that of the Elephant Man" (*Daily Star*, 16 March) were used with effect.

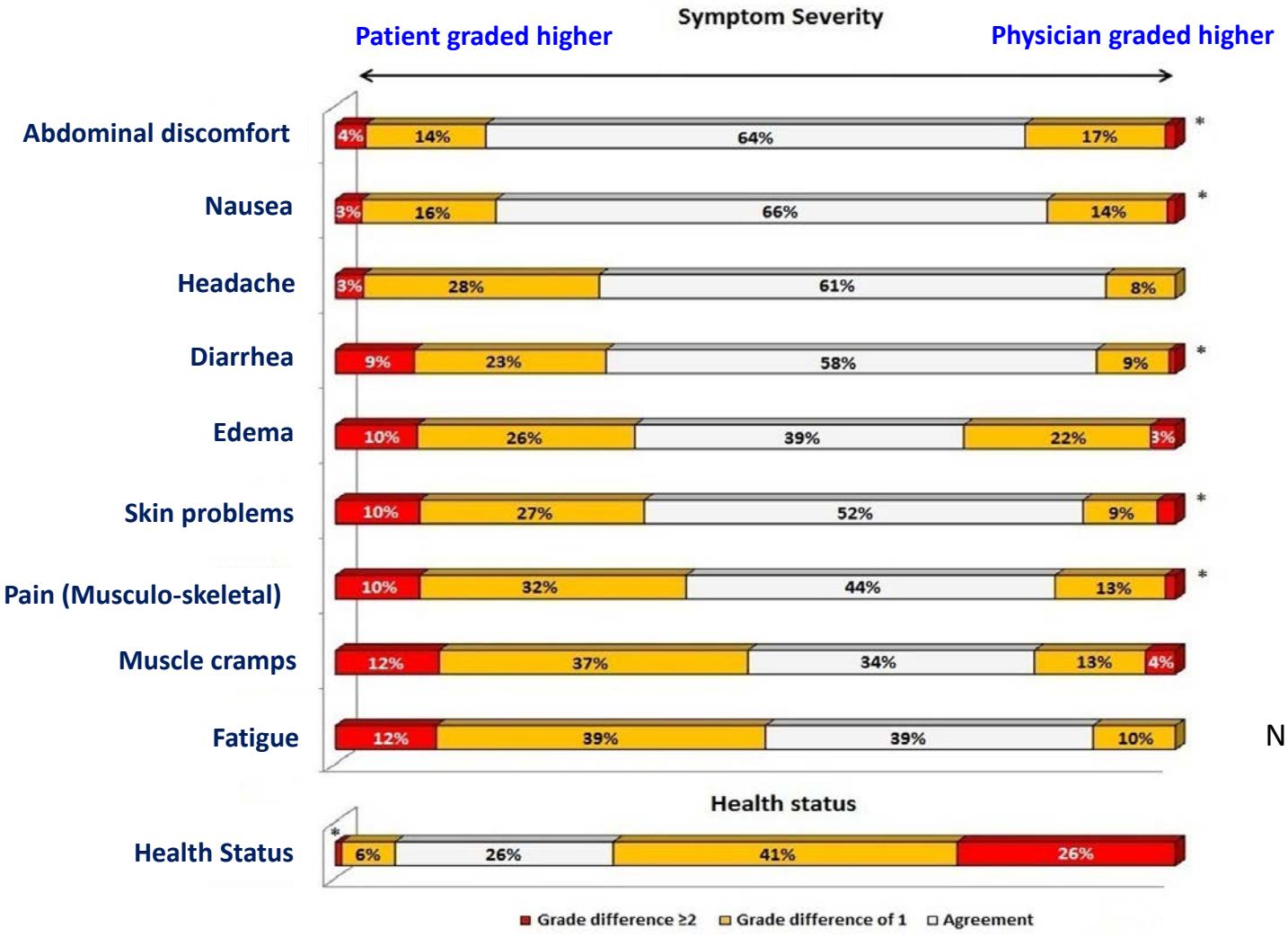


“Avoidable waste in the production and reporting of research evidence”

Iain Chalmers, Paul Glasziou, The Lancet, 15 June 2009, doi:10.1016/S0140-6736(09)60329-9



Do doctors know what is most important to patients? Here‘s the data.



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The CML Quality of Life Study. Efficace F, Rosti G, Aaronson , et al, Haematologica, 2013

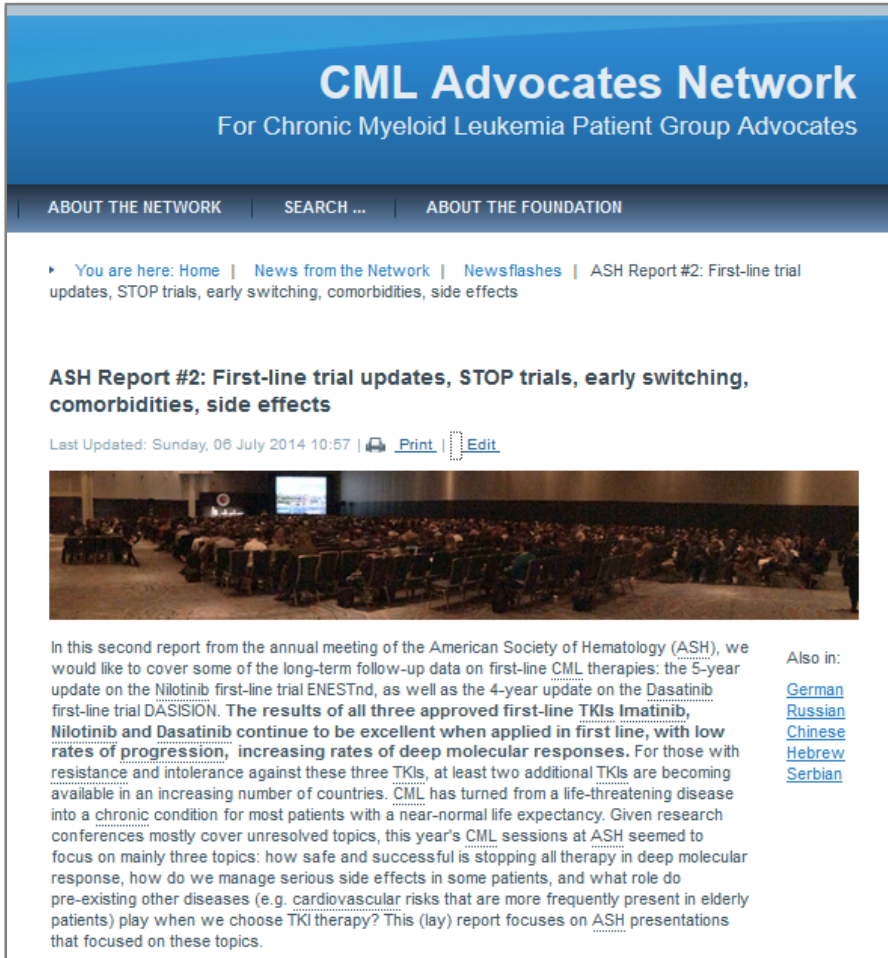
Patient involvement in R&D: How can I find out how this works?

CML Advocates Network Trial Database
http://www.cmladvocates.net/cmltrials

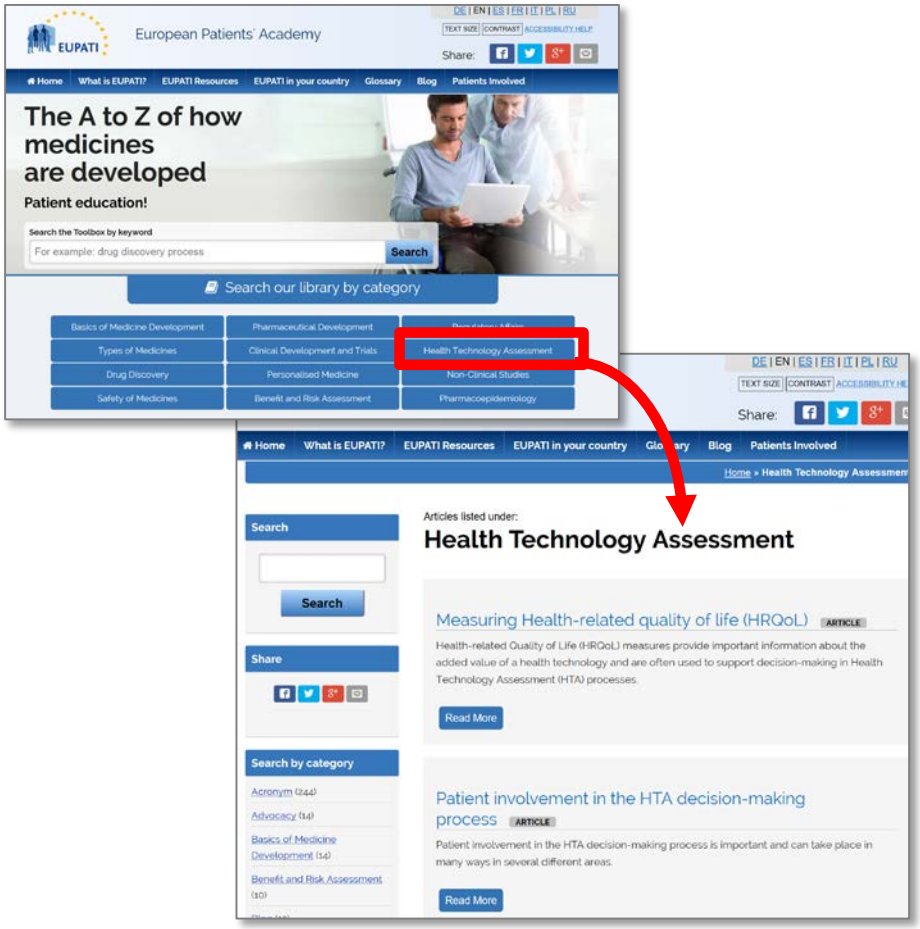
- Currently 29 clinical trials listed
- Continuously updated

Get information from medical conferences, e.g. ASH and EHA

- Read the ASH Report of Giora and Jan
- Read the ASH Abstracts
- Go to EHA – free tickets available via Fellowship
- Get medical publications via doctors



Use the EUPATI Toolbox on Medicines R&D at www.eupati.eu



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4. Clinical Trials
5. Regulatory Affairs, Drug Safety, Pharmaco-vigilance
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Evidence-based medicine ARTICLE

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Evidence-based medicine combines clinical evidence and experience so that doctors and patients can make the best decisions on an individual basis.

Critical reading of clinical study results ARTICLE

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When performing a critical reading of clinical study results, the reader should take relevant information into account from the best available sources and should consider questions about the reliability, methodology, results, discussion, significance, and conclusions of the study.

Measuring Health-related quality of life (HRQoL) ARTICLE

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Health-related Quality of Life (HRQoL) measures provide important information about the added value of a health technology and are often used to support decision-making in Health Technology Assessment (HTA) processes.

Pharmacovigilance: Monitoring the safety of medicines ARTICLE

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Pharmacovigilance is the practice of detecting, understanding, and preventing adverse events or any other medicine-related problem.

Patient involvement in the HTA decision-making process ARTICLE

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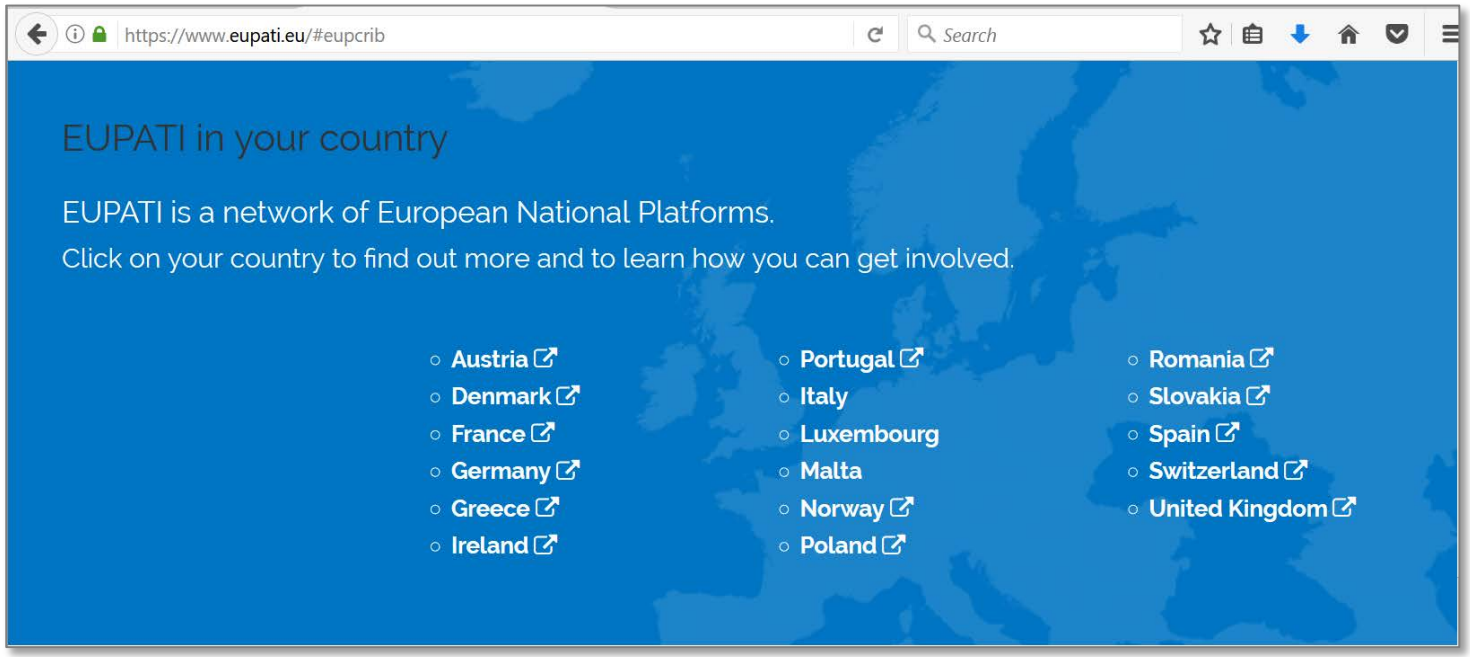
Patient involvement in the HTA decision-making process is important and can take place in many ways in several different areas.

Join one of the 18+ EUPATI National Platforms

EUPATI National Platforms...

- bring all stakeholders together in countries
- address educational needs in R&D
- disseminate EUPATI's training material to patient organisations



…or join the annual EUPATI Patient Expert Training Course … but it‘s hard!

Our CML graduates:

- Sarunas Narbutas
- Conny Borowczak
- Aimo Stromberg

CML Trainees on the 2017-2018 course:

- Toni Montserrat
- Giora Sharf
- Celia Marin

Educate and Engage!

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