



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

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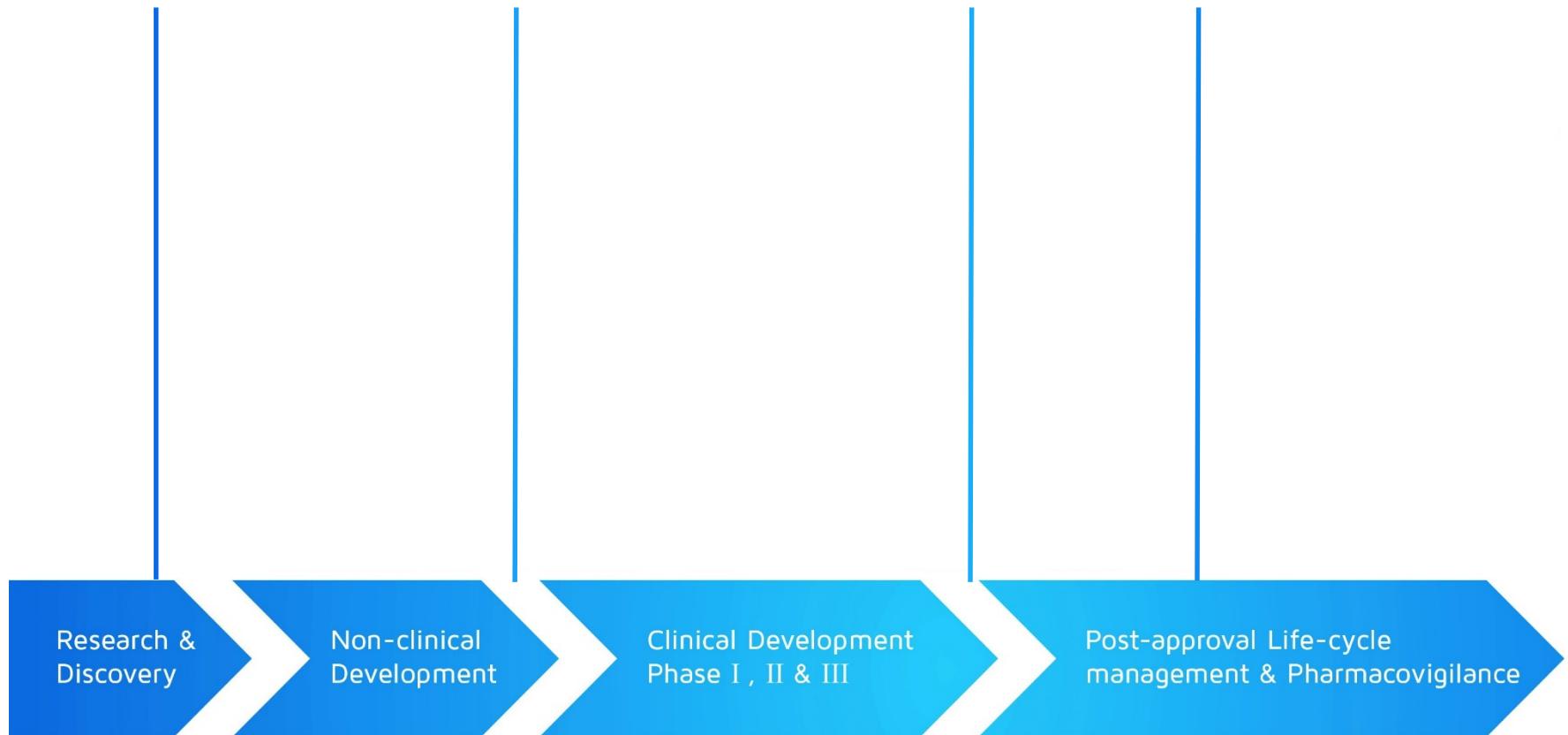


Hope and Hype

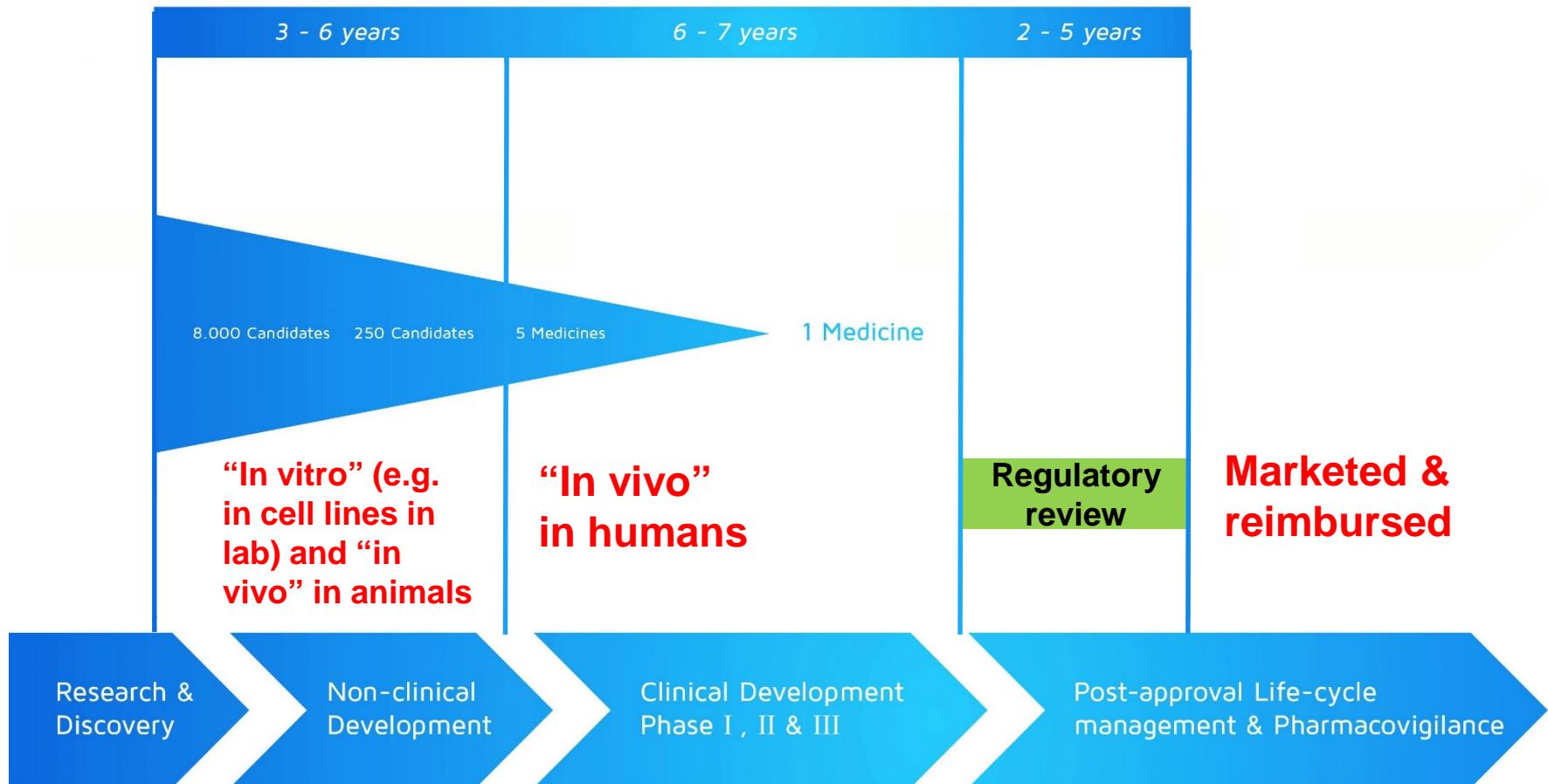
- It takes over 12 years and on average costs over €1 billion to perform all research and development before a new medicine can be made available to patients.
- Only about 2% of substances evaluated in early research make it to the market as new medicines.
- “Hope and hype”: CML patient advocates need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



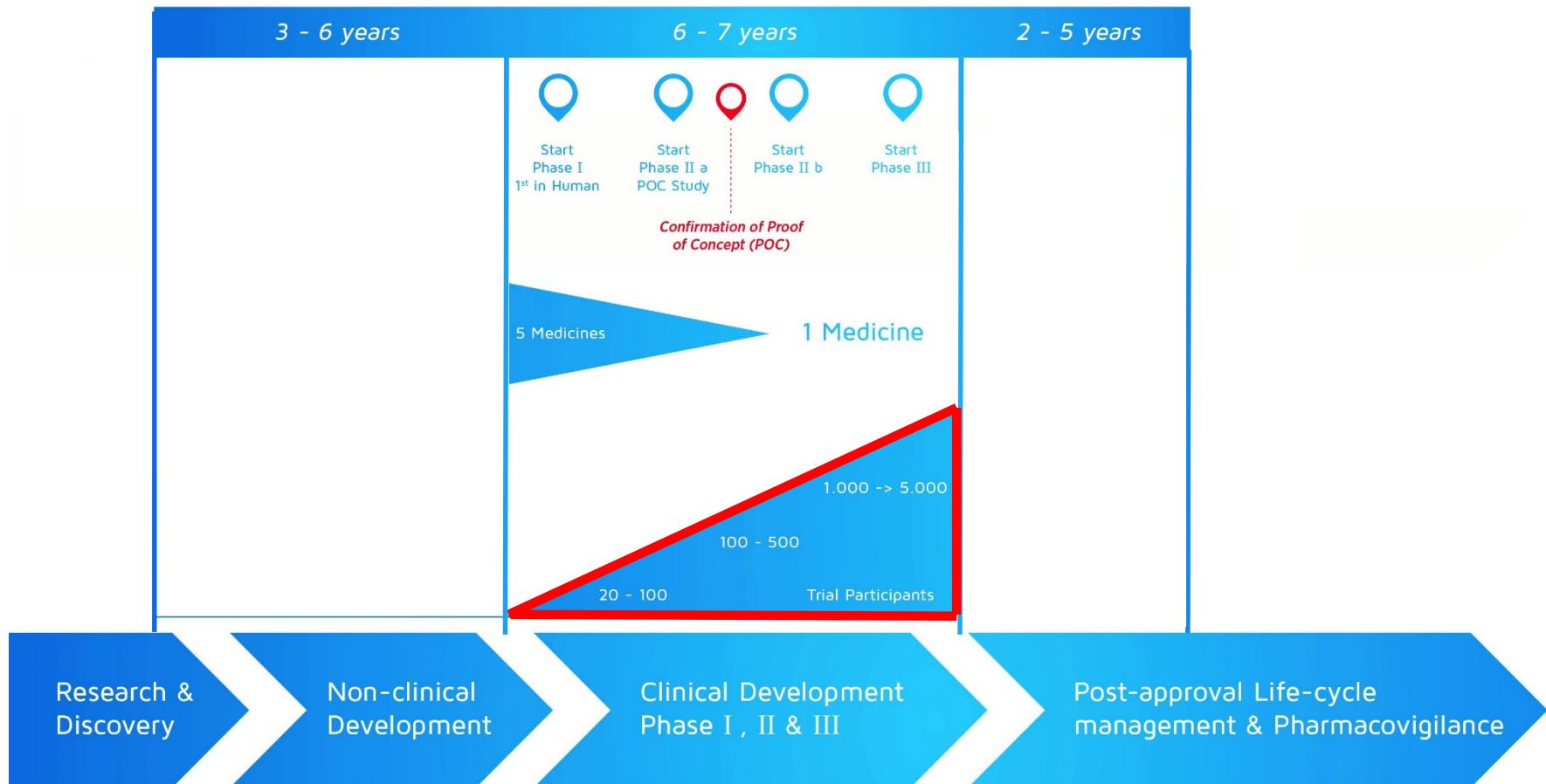
Phases of Medicines Research & Development



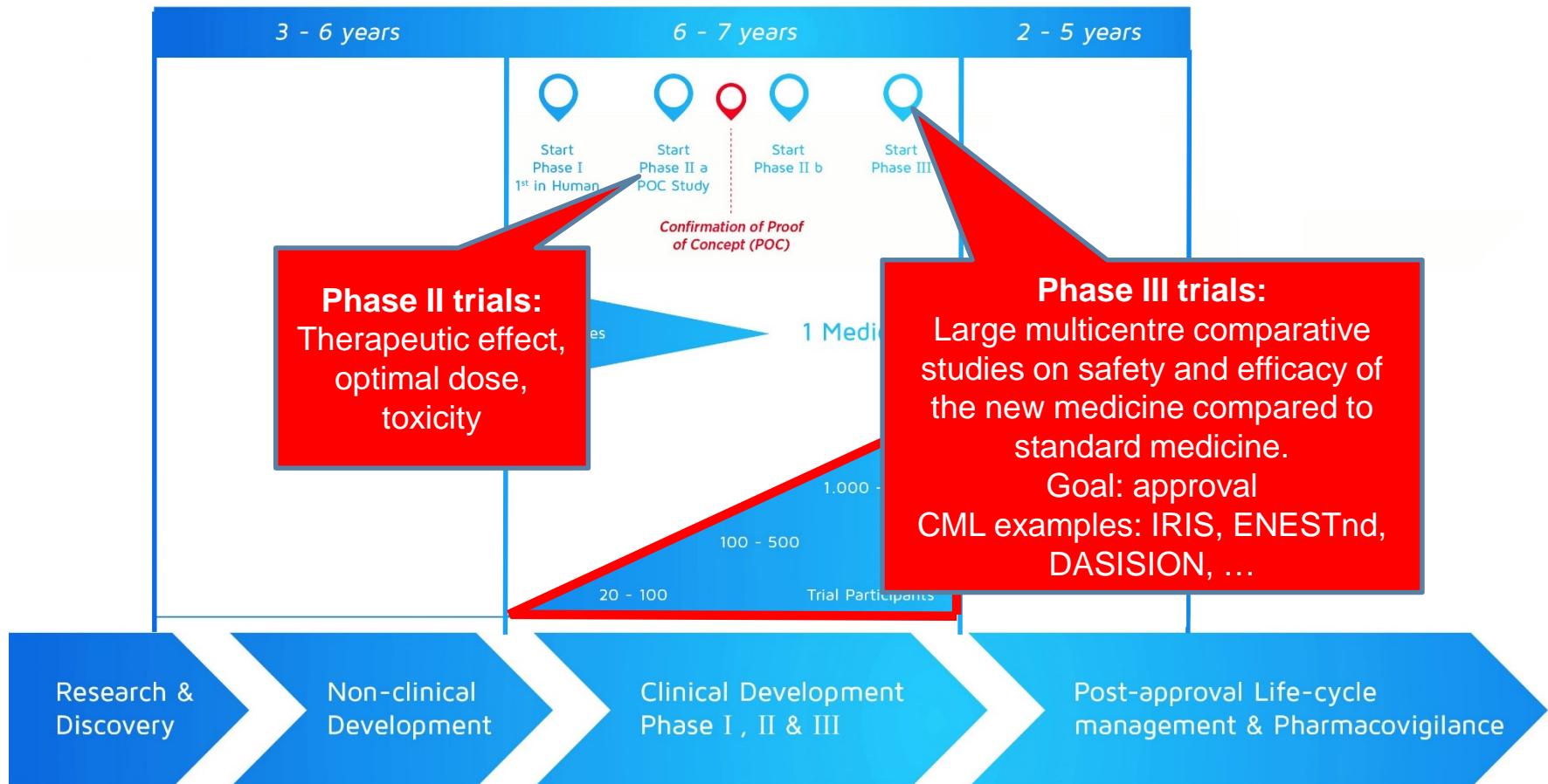
Of 8.000 molecule candidates,
only 5 ever get into human clinical trials,
and only 1 makes it to the market



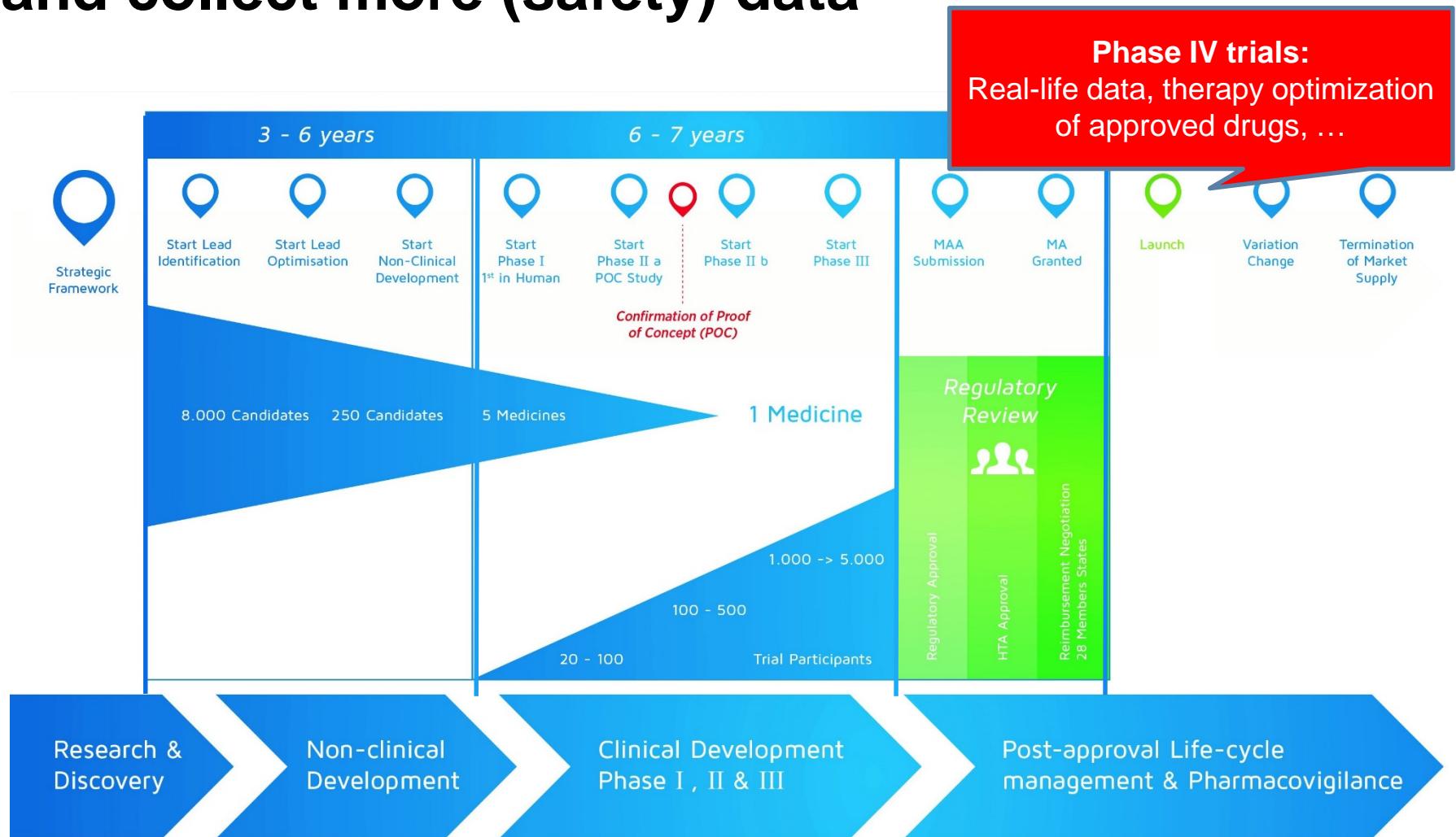
In different phases of clinical trials, dosing, safety, efficacy is tested in an increasing numbers of patients



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

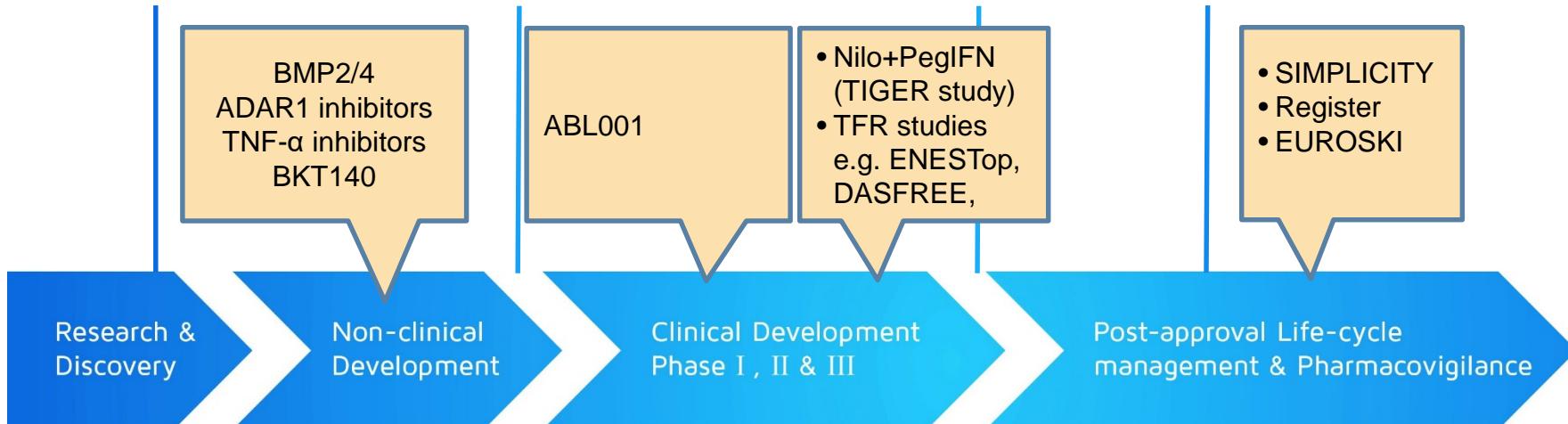


After regulatory approval and reimbursement decision, late-phase trials optimize therapy and collect more (safety) data

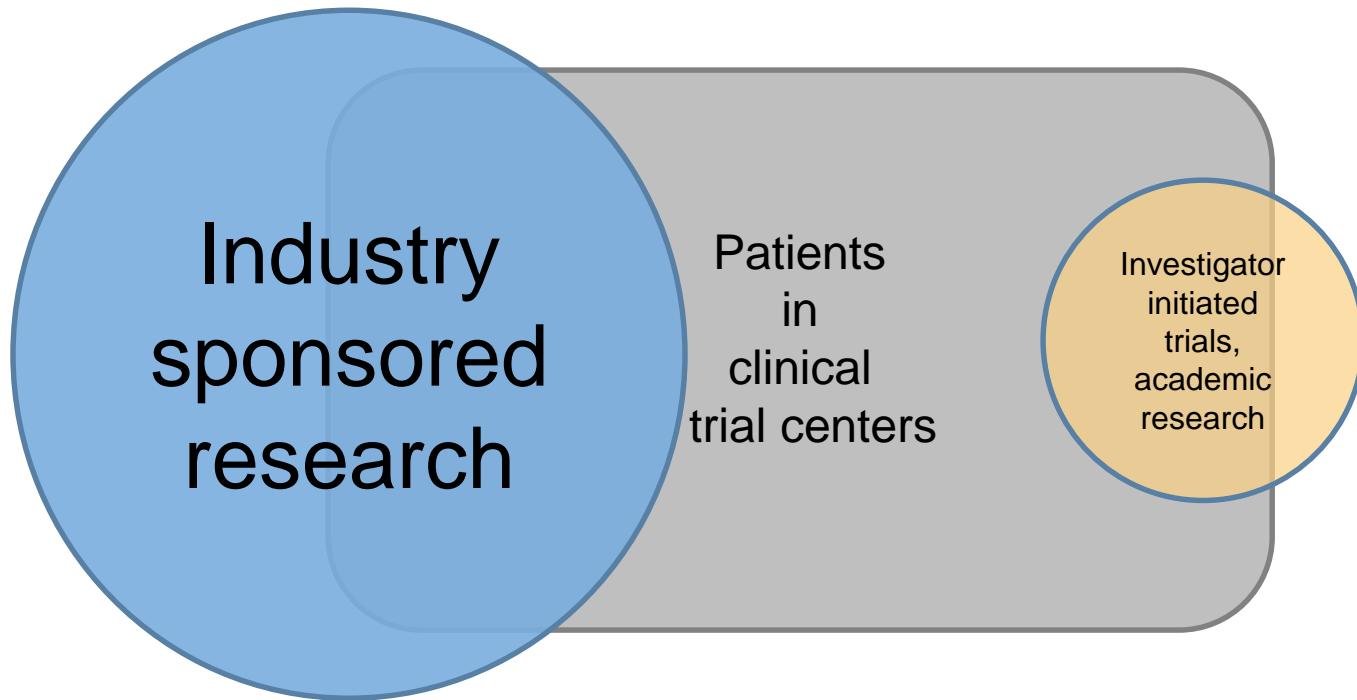


So what does this mean in CML?

- 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.



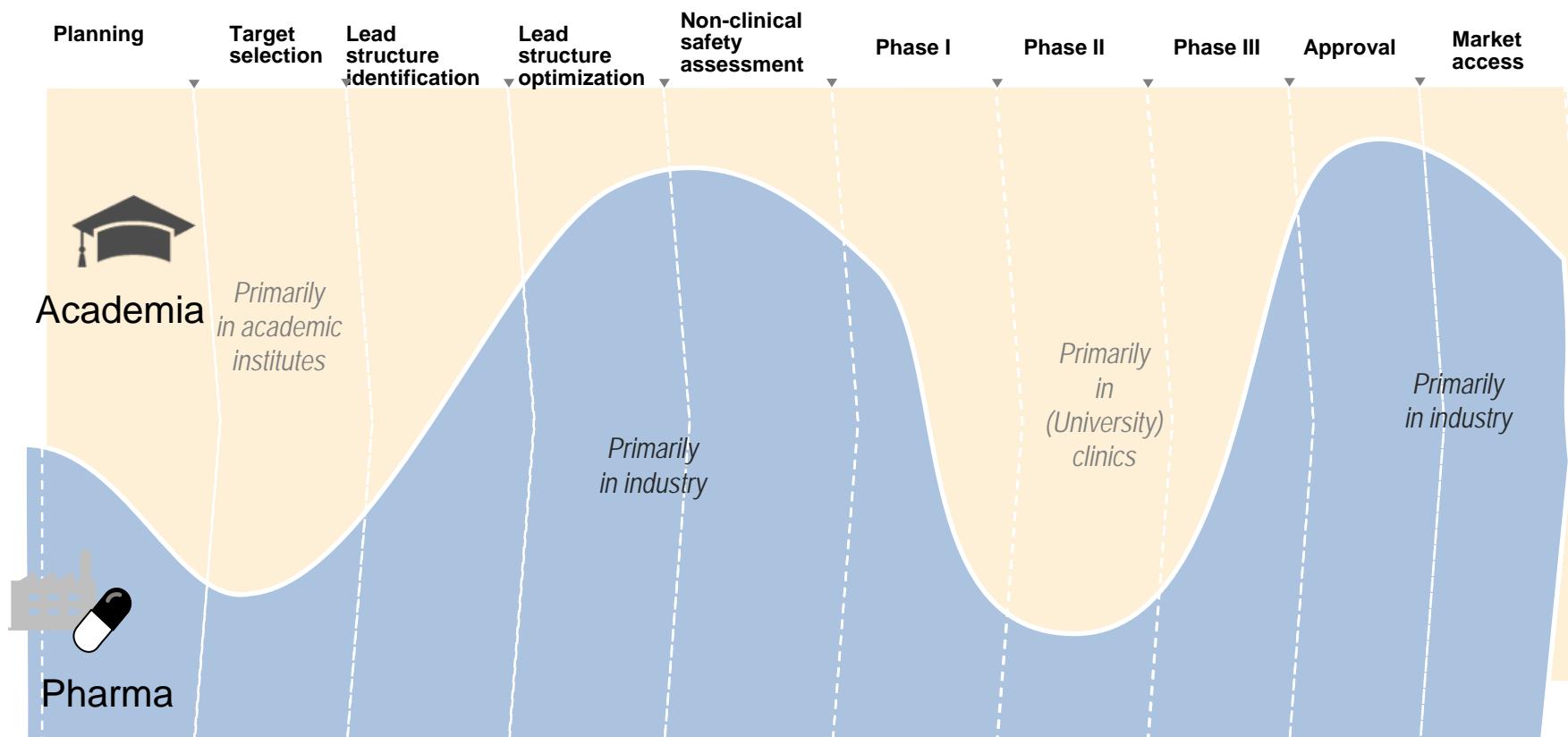
**Most CML trials are initiated/sponsored by industry and then run at academic and community trial centers.
Not so many CML trials are purely academic.**



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 Stobart 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

1

Questions relevant to clinicians & patients?

Low priority questions addressed

Important outcomes not assessed

Clinicians and patients not involved in setting research agendas

2

Appropriate design and methods?

Over 50% studies designed without reference to systematic reviews of existing evidence

Over 50% of studies fail to take adequate steps to reduce biases, e.g. unconcealed treatment allocation

3

Accessible full publication?

Over 50% of studies never published in full

Biased under-reporting of studies with disappointing results

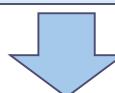
4

Unbiased and usable report?

Over 30% of trial interventions not sufficiently described

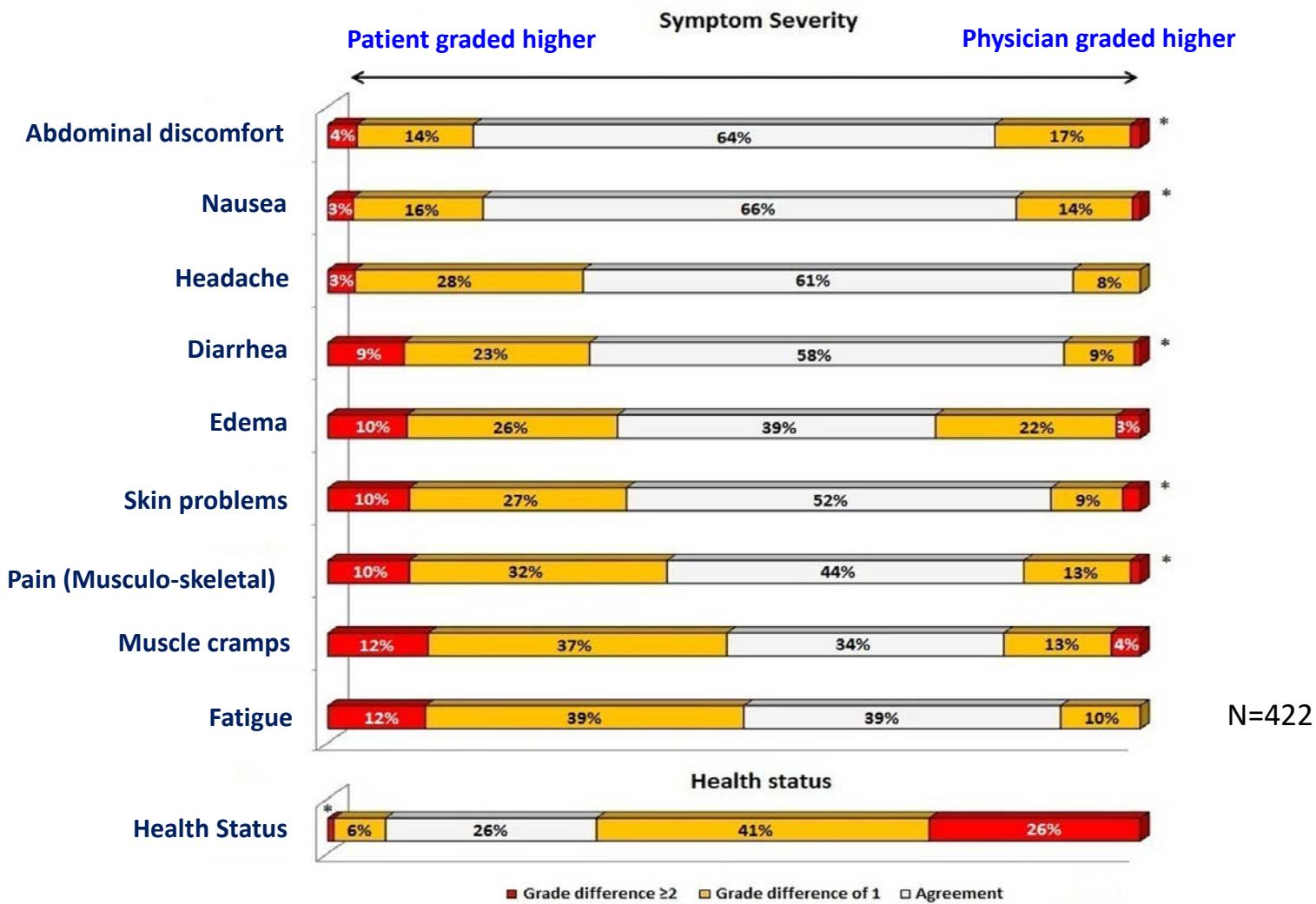
Over 50% of planned study outcomes not reported

Most new research not interpreted in the context of systematic assessment of other relevant evidence



85% research waste = over \$85 billion / year

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





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

CML Advocates Network
For Chronic Myeloid Leukemia Patient Group Advocates



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CML Clinical Trials

CML Clinical Trials Database -

Cancer research is an area where new treatments are being explored for the prevention, diagnosis and treatment of different types of cancer. Researchers conduct so called "clinical studies" or "clinical trials" to improve treatment options for the patients of today and tomorrow. These studies are performed to investigate the biological mechanisms of the disease, to optimize the use of existing therapies or to test new forms of treatment or new drugs to find out whether they are more effective or better tolerated. For more information please read [our manual "what are clinical trials?"](#)

Patients wishing to participate in clinical studies often look for a central source of information where they can find easy-to-understand facts on ongoing trials. CML Advocates Network is now hosting an unofficial database of current CML trials which have been initiated by academia (universities) or by pharmaceutical companies. This database is run by patients for patients and is set up as a dynamic register in which data are updated on an ongoing basis. CML Advocates Network has taken utmost care to verify the data entered, however, cannot assume any liability for the accuracy or completeness of the information. Patients should consult their doctor for personal advice and/or their scientific contact/study staff for the most up-to-date study information.

Do you know of any study that is not yet listed here? [Please let us know by filling out this form](#). Or is there an entry which should be corrected? If so, please [send us an email](#). Please also visit our forum if you have any questions or wish to share your experience on these studies.

CML Clinical Trials Database

1. First line trials

- 1 [TIGER \(CML_V\) = Nilotinib or nilotinib + peginterferon with the aim to discontinue treatment](#)
Updated: 22 January 2015
- 2 [Ponatinib for CML in accelerated phase](#)
Updated: 14 February 2015
- 3 [BFORE \(AV001\) = Bosutinib versus Imatinib in Adult Patients with Newly Diagnosed Chronic Phase Chronic Myelogenous Leukemia](#)
Updated: 22 January 2015

2. Trials after therapy failure or intolerance

- 1 [DASCERN \(CA180-399\) = Phase IIb Study of Dasatinib versus Imatinib \(Early Switch\)](#)
Updated: 13 March 2015
- 2 [CABL001X2101 = A Phase I, Multicenter, Open-label Study of Oral ABL001 in Patients With CML or Ph+ ALL](#)
Updated: 8 March 2015
- 3 [B1871039 = Safety and Efficacy of Bosutinib in Ph+ CML Previously Treated with TKI](#)
Updated: 16 April 2015

3. Therapy optimization trials

- 1 [SIPRIT 3 = imatinib, nilotinib and ponatinib in patients with newly-diagnosed chronic phase chronic myeloid leukaemia](#)
Updated: 8 March 2015
- 2 [NordDutchCML009](#)
Updated: 8 March 2015
- 3 [MSIT - Malvevia Sten Tyrosine Kinase Inhibitor Trial](#)

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

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Free EHA congress registrations for representatives of haematology patient organizations: Deadline 29 April 2017!

Last Updated: Sunday, 09 April 2017 11:31 | [Print](#)

 **MADRID**
22ND CONGRESS
JUNE 22-25 | 2017
European Hematology Association

This year EHA Congress will be held on the 22-25 June 2017 in Madrid, Spain. Thanks to very patient-centric thinking, EHA continues to work closely with the patient community and is once again offering a "Patient



American Society *of* Hematology
Helping hematologists conquer blood diseases worldwide

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ASH Report #2: First-line trial updates, STOP trials, early switching, comorbidities, side effects

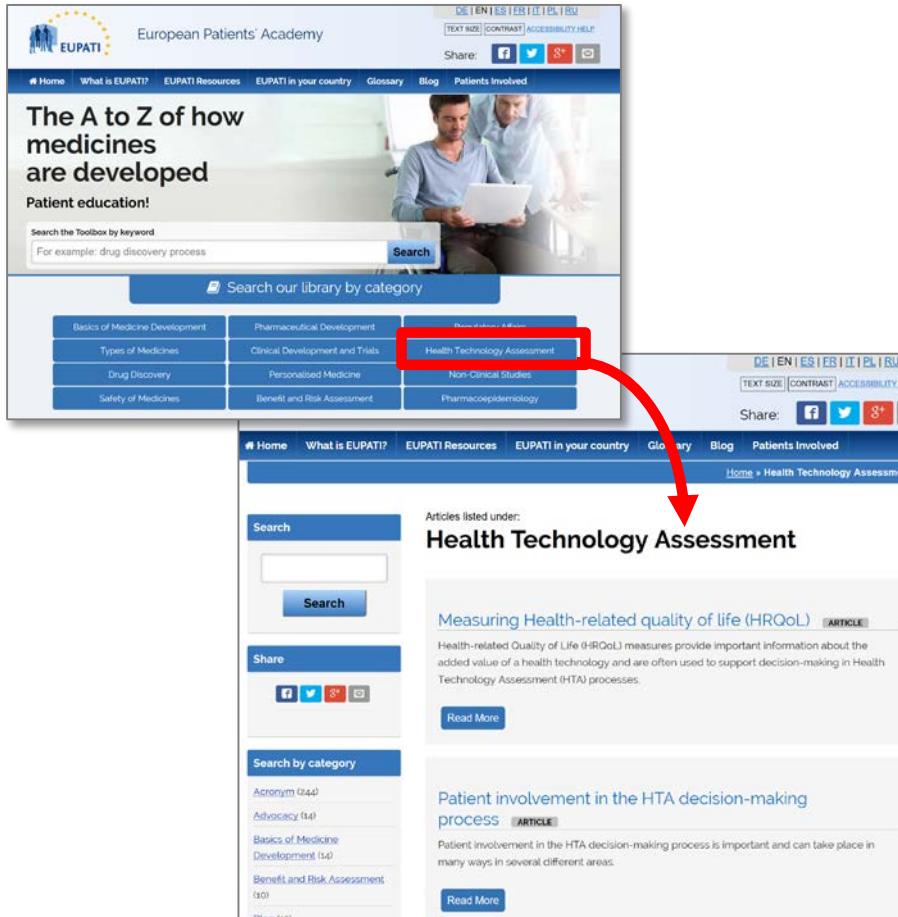
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In this second report from the annual meeting of the American Society of Hematology (ASH), we would like to cover some of the long-term follow-up data on first-line CML therapies: the 5-year update on the Nilotinib first-line trial ENESTnd, as well as the 4-year update on the Dasatinib first-line trial DASISION. The results of all three approved first-line TKIs Imatinib, Nilotinib and Dasatinib continue to be excellent when applied in first line, with low rates of progression, increasing rates of deep molecular responses. For those with resistance and intolerance against these three TKIs, at least two additional TKIs are becoming available in an increasing number of countries. CML has turned from a life-threatening disease into a chronic condition for most patients with a near-normal life expectancy. Given research conferences mostly cover unresolved topics, this year's CML sessions at ASH seemed to focus on mainly three topics: how safe and successful is stopping all therapy in deep molecular response, how do we manage serious side effects in some patients, and what role do pre-existing other diseases (e.g. cardiovascular risks that are more frequently present in elderly patients) play when we choose TKI therapy? This (lay) report focuses on ASH presentations that focused on these topics.

Also in:
[German](#)
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Use the EUPATI Toolbox on Medicines R&D at www.eupati.eu



The screenshot shows two pages of the EUPATI website. The top page displays a grid of categories under 'The A to Z of how medicines are developed'. One category, 'Health Technology Assessment', is highlighted with a red box and a red arrow points from it to the second page. The second page shows a search result for 'Health Technology Assessment', listing articles such as 'Measuring Health-related quality of life (HQoL)' and 'Patient involvement in the HTA decision-making process'.

1. Discovery of Medicines
2. Pre-clinical Development
3. Clinical Development
4. Clinical Trials
5. Regulatory Affairs, Drug Safety, Pharmaco-vigilance
6. Health Technology Assessment

English
French
German
Spanish
Polish
Italian
Russian

(Danish,
Romanian,
Dutch,
Portuguese)

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

The screenshot shows a web browser window with the URL <https://www.eupati.eu/#eupcrib>. The page title is "EUPATI in your country". The main content features a blue-toned map of Europe. Overlaid on the map are lists of national platforms, grouped by continent:

- Europe (Northern Europe):**
 - Austria ↗
 - Denmark ↗
 - France ↗
 - Germany ↗
 - Greece ↗
 - Ireland ↗
- Europe (Central Europe):**
 - Portugal ↗
 - Italy
 - Luxembourg
 - Malta
 - Norway ↗
 - Poland ↗
- Europe (Southern Europe):**
 - Romania ↗
 - Slovakia ↗
 - Spain ↗
 - Switzerland ↗
 - United Kingdom ↗

Below the map, there is a search bar and a menu icon.

...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!

Jan Geissler

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Web: www.eupati.eu and www.cmladvocates.net