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European Organisation for Research and Treatment of Cancer

Cancer Medicines Forum (EMA-EORTC)

Advancing Cancer Treatment Optimization Across Europe

Denis Lacombe May, 2024

EORTC by numbers (2023)

World-class network

2489 patients screened
- 2021-2023: **8960** patients

1910 patients enrolled in the CTs
- **229** institutions
- **480** principal investigators
- **26** countries
- 2021-2023: **7666** patients

34 intergroup collaborations
- **17** active groups & taskforces
- **60** peer reviewed papers

Total EORTC Network
> 3800 Members
> 1000 Institutions

Unique output

21 studies open on 1/01/2024
- **5** opened studies in 2023

≈ 100 studies closed/LTFU
- **10** closed in 2023

15 studies in protocol development
8 studies in regulatory activation

Working on ≈ 150 studies

Centre of expertise

220 + employees

> 215,000 patients in database

± 22,700 patients in follow-up

5 EORTC HQ peer reviewed papers

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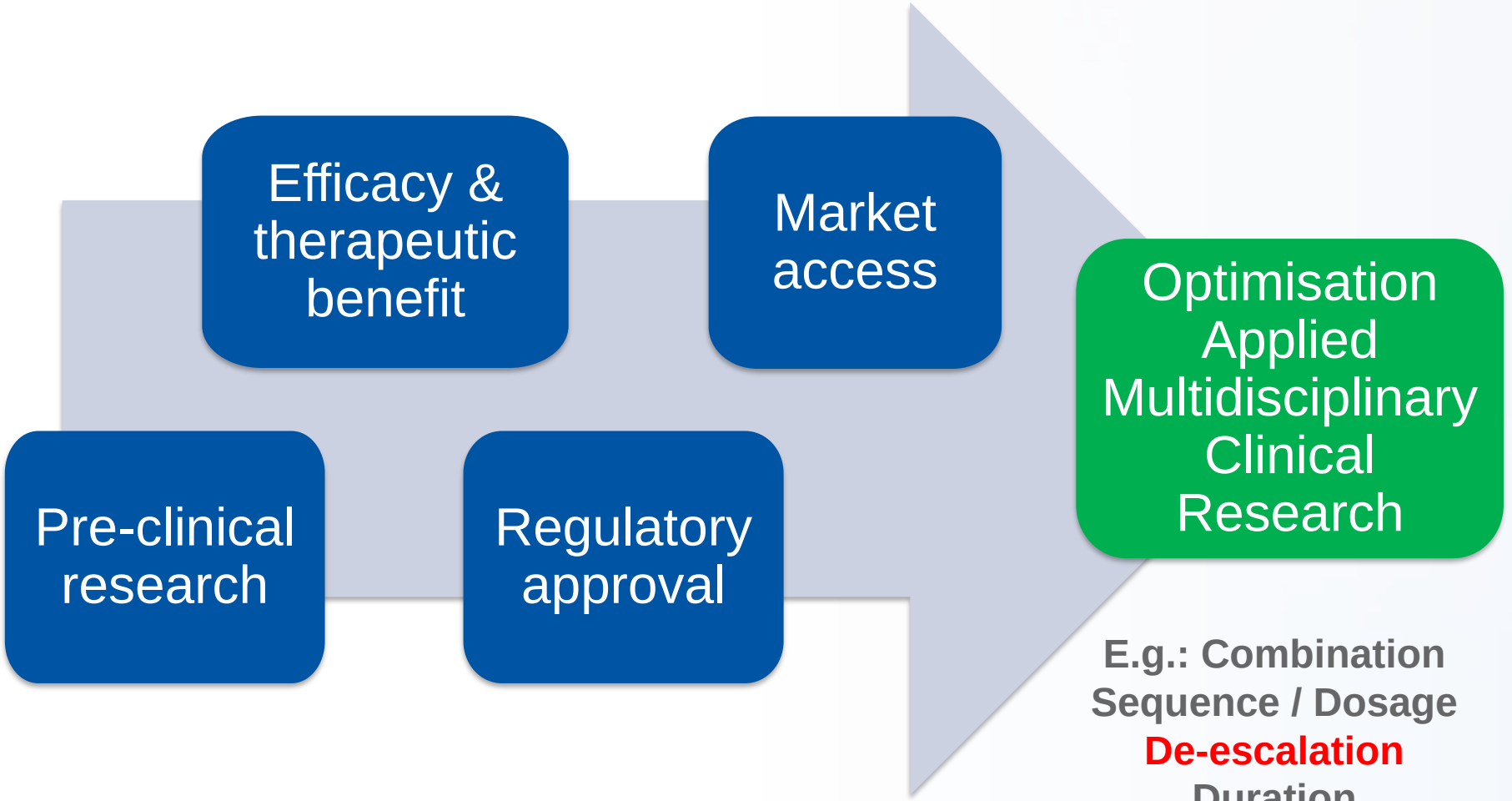
Why did the CMF get started?

Once upon a time in the late years 2000s….

When we needed to re-think why and how!



Optimisation Applied Multidisciplinary Clinical Research

E.g.: Combination
Sequence / Dosage
De-escalation
Duration
Benchmarking
Specific populations

Health System Optimisation

Health Services & Implementation Research

Access / costs
Guidelines
Cancer control plans

Clinically relevant endpoints for patients

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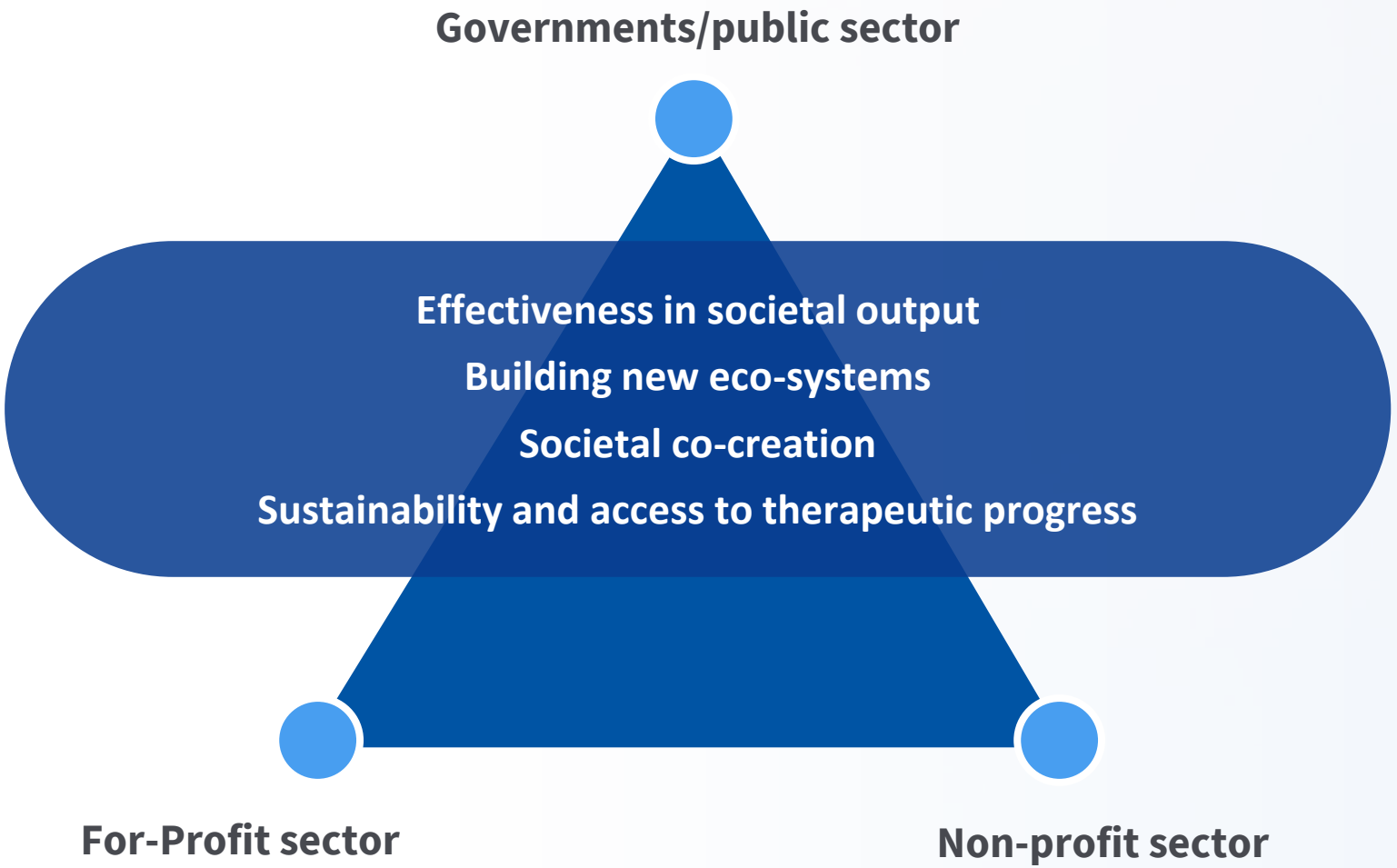
Why EORTC stimulated the CMF?



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It has been a journey… today is an important milestone

The Porto Declaration on Cancer Research

2021 PORTUGAL.EU

2. **Infrastructures for clinical and prevention trials:**

'Proof-of-concept' studies may serve as a starting point for further clinical and prevention research, with a practice-changing aim, including the assessment of its utility in healthcare or prevention, patients´/individuals 'at risk, cure/survival and health-related quality of life. Well-developed clinical trial structures, and advanced diagnostic methods such as state-of-the-art molecular pathology, omics technologies, and pharmacology to stratify patients as well as innovative imaging are crucial. CCCs can play a role in this together with clinical research networks. The European Organisation for Research and Treatment of Cancer (EORTC) can facilitate this.

For prevention, infrastructures must include strong epidemiology closely connected to basic research, data acquisition capacity, and advanced computational capabilities, and both the International Agency for Research on Cancer (IARC) and Cancer Prevention Europe can play a prominent role in this, along with many other stakeholders. Again, it will be critical to establish funding mechanisms that stimulate these activities and guarantee sustainability. Funding should include resources for proof-of-concept trials initiated by academic investigators.

European Parliament
2019 - 2024

Directorate-General for Internal Policies
Special Committee on Beating Cancer

SUMMARY

BECA Hearing
"Mind the gap: For equal access to cancer medicines and treatments"
Thursday 28 January 2021, 13:45 to 16:15 & 16:45 to 18:45

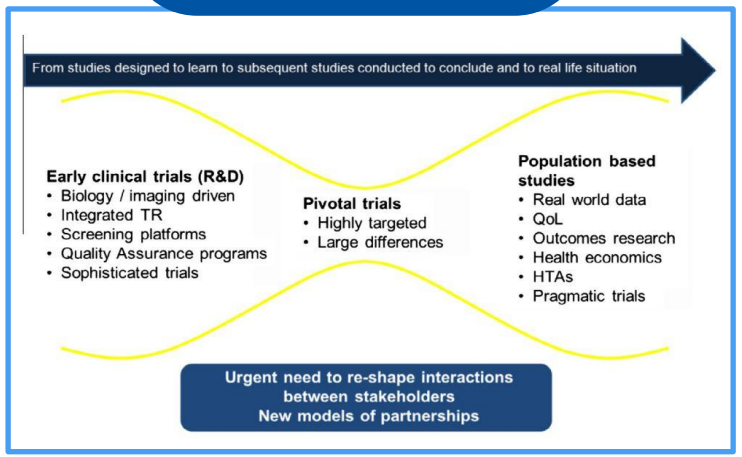
(Jósef Antall 4Q2 and with remote participation)

In the Chair: Bartosz ARŁUKOWICZ, Chair

…….2013

2013-2019

2019-2024



Manifesto

for a new approach for better medicine in Europe
Establishing Treatment Optimization
as part of personalized medicine development
(version 29 May 2020)

Available online at www.sciencedirect.com
ScienceDirect
journal homepage: www.ejcancer.com

to the Editor

Advancing academia-driven treatment optimisation in oncology: Launch of the EMA Cancer Medicines Forum

Robbe Saesen [a,*], Claire Espinasse [b], Francesco Pignatti [b], Denis Lacombe [a]

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Received 23 March 2022; accepted 26 March 2022

Burock S, Meunier F, Lacombe D. **How can innovative forms of clinical research contribute to deliver affordable cancer care in an evolving health care environment?** Eur J Cancer 2013; 49:2777-2783.

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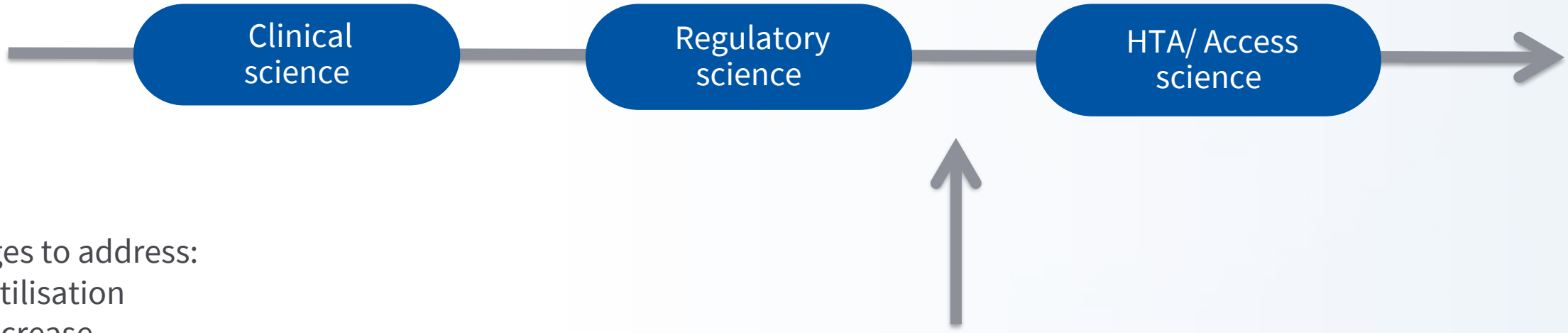
Key questions to the policy makers

- How to address the gap between supra-national versus national competences?
- If treatment optimisation is to be structured in the process: when, how and who?
- How do we re-engineer the sequence of questions from development into access?
- How do we prioritise questions and select the most appropriate methodology?
- Can Pragmatic clinical trials help structuring and addressing some of the issues?
- How do we structure independent multidisciplinary clinical research in the EU?

A new continuum to be set up ….Re-engineer….



Challenges to address:

- Over-utilisation
- Cost increase
- No association between magnitude of benefit and drug price
- Less informative clinical trials
- Establish a sustainable economic model for cancer treatment research, development and access.

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Need for strategic intelligence approaches

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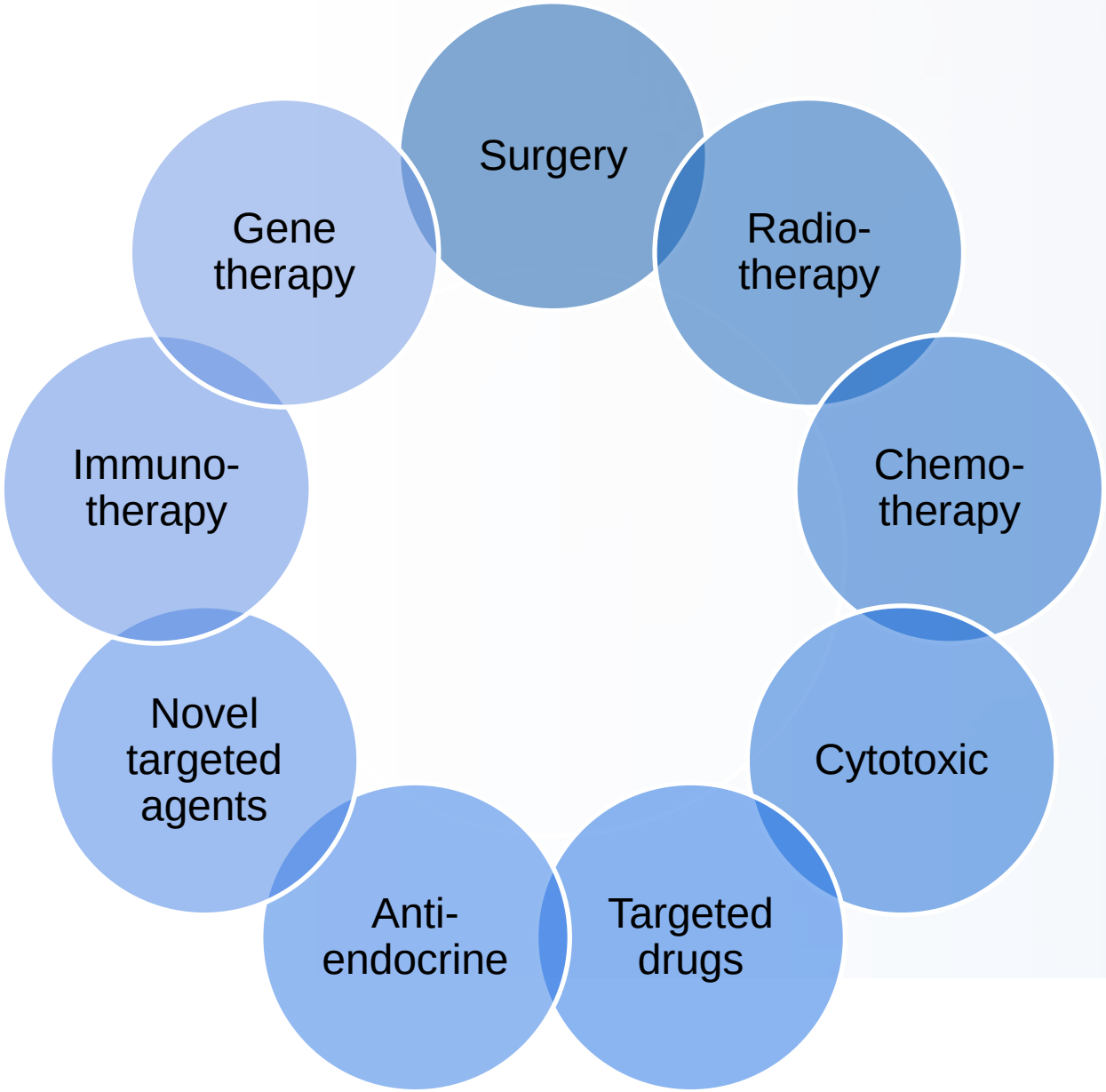
The Future of Cancer Treatment is Combinatorial

Multidimensional data

Authorisation



Optimal access

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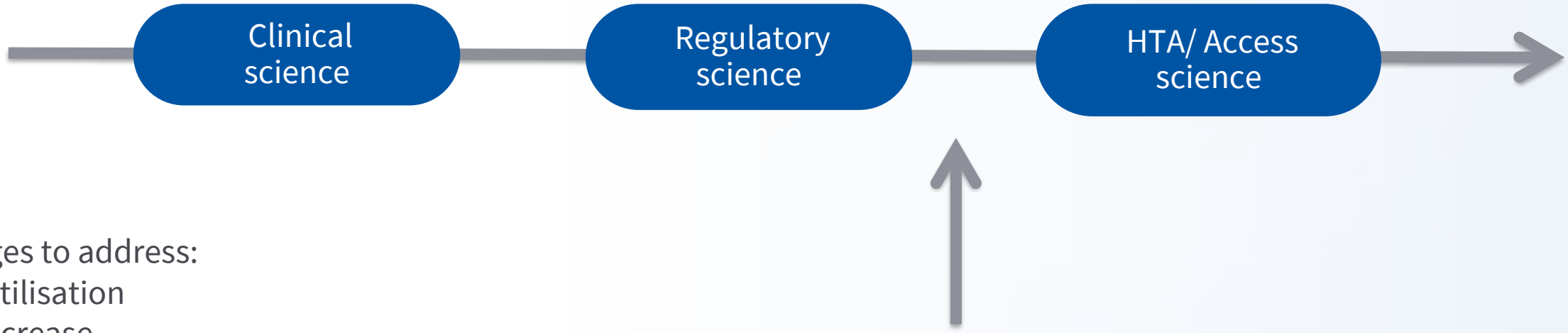

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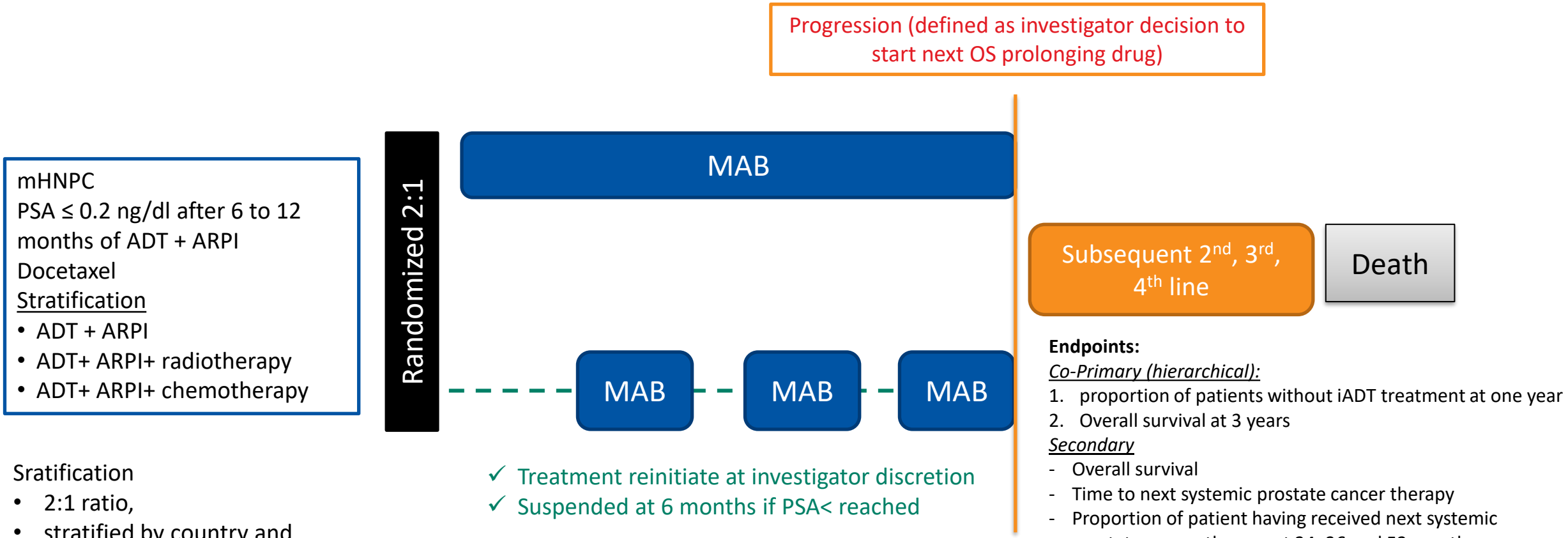
DE-ESCALATE Intermittent ADT in the era of AR pathway inhibitors; a phase 3 **pragmatic** randomized trial (EORTC 2238)

Funded by the European Union



✓ Treatment reinitiate at investigator discretion
✓ Suspended at 6 months if PSA< reached

Sratification
- 2:1 ratio,
- stratified by country and
- ARPI alone, ARPI + docetaxel, ARPI + radiotherapy)
- PSA ≤0.1 vs >0.1 - ≤ 0.2 ng/dl

Endpoints:

Co-Primary (hierarchical):

1. proportion of patients without iADT treatment at one year
2. Overall survival at 3 years

Secondary

- Overall survival
- Time to next systemic prostate cancer therapy
- Proportion of patient having received next systemic prostate cancer therapy at 24, 36 and 52 months.
- Toxicity with CTCAE v5
- Quality of life with QLQ-C30/PR-25
- Health economics parameters (e.g. Incremental cost effectiveness ratio)

mHNPC: metastatic hormone naïve prostate cancer patients;

 This project has received funding from the European Union's HORIZON-MISS-CANCER-2022-01 under grant agreement Nº (101104574).

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The future of cancer therapy

Objectives of the Cancer Medicines Forum

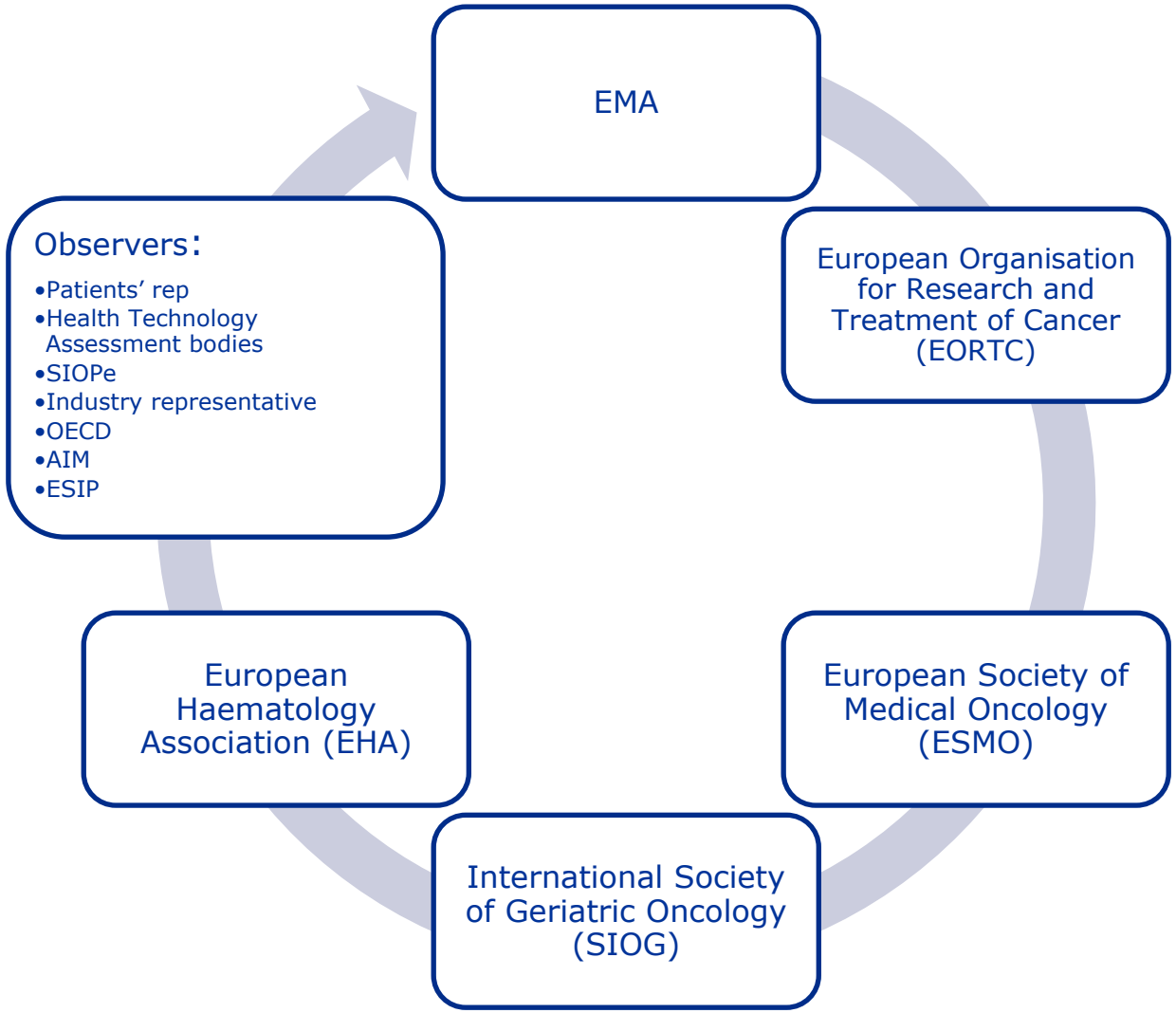
To serve as a direct and official communication channel with the academic community in oncology

To identify key research questions and best methodological approach to improve the clinical use of cancer medicines

To discuss the uptake of academic work in the wider context of regulatory decision-making in oncology

Treatment optimisation

Focus on academia with other stakeholders



Structuring Treatment Optimisation

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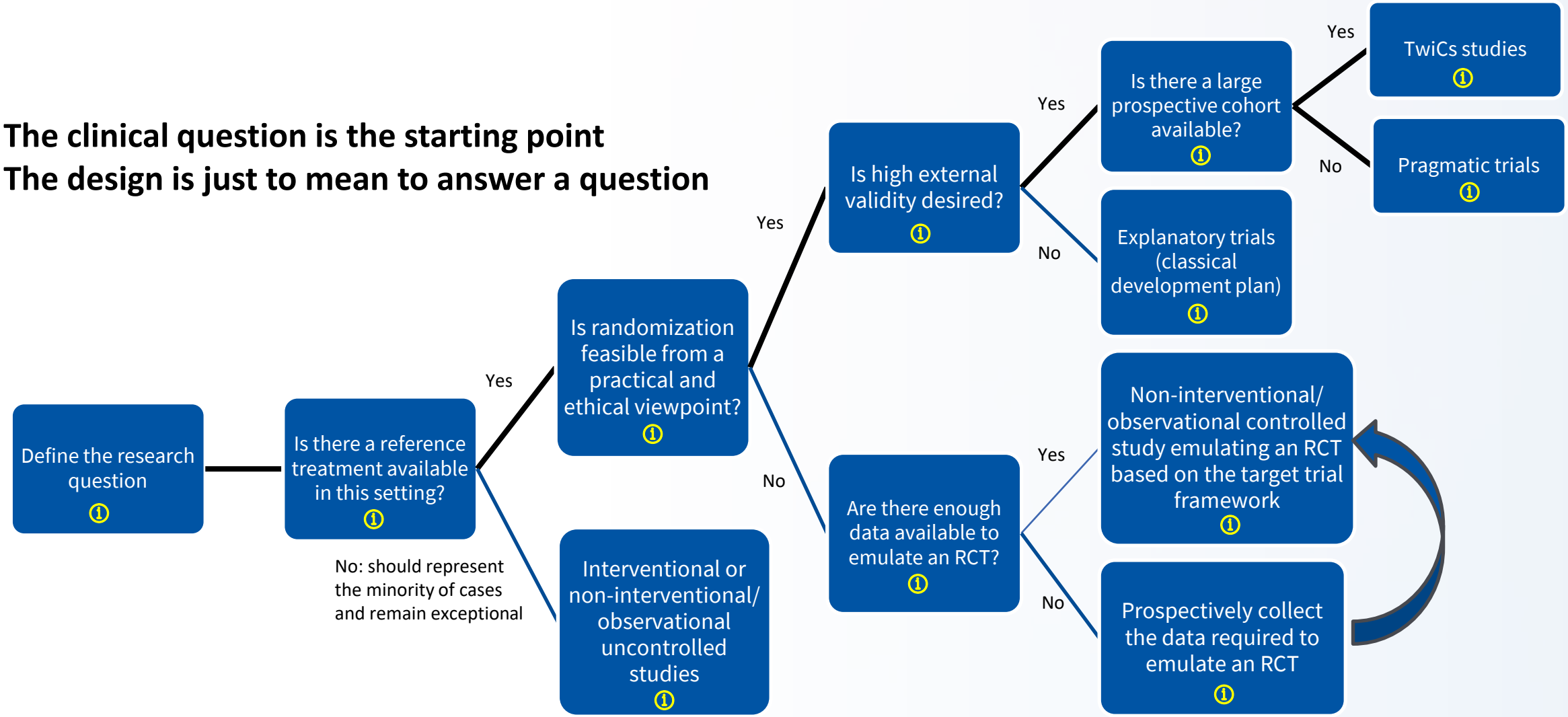
The clinical question is the starting point
The design is just to mean to answer a question



Thick lines represent the default route to deliver evidence for trial design selection

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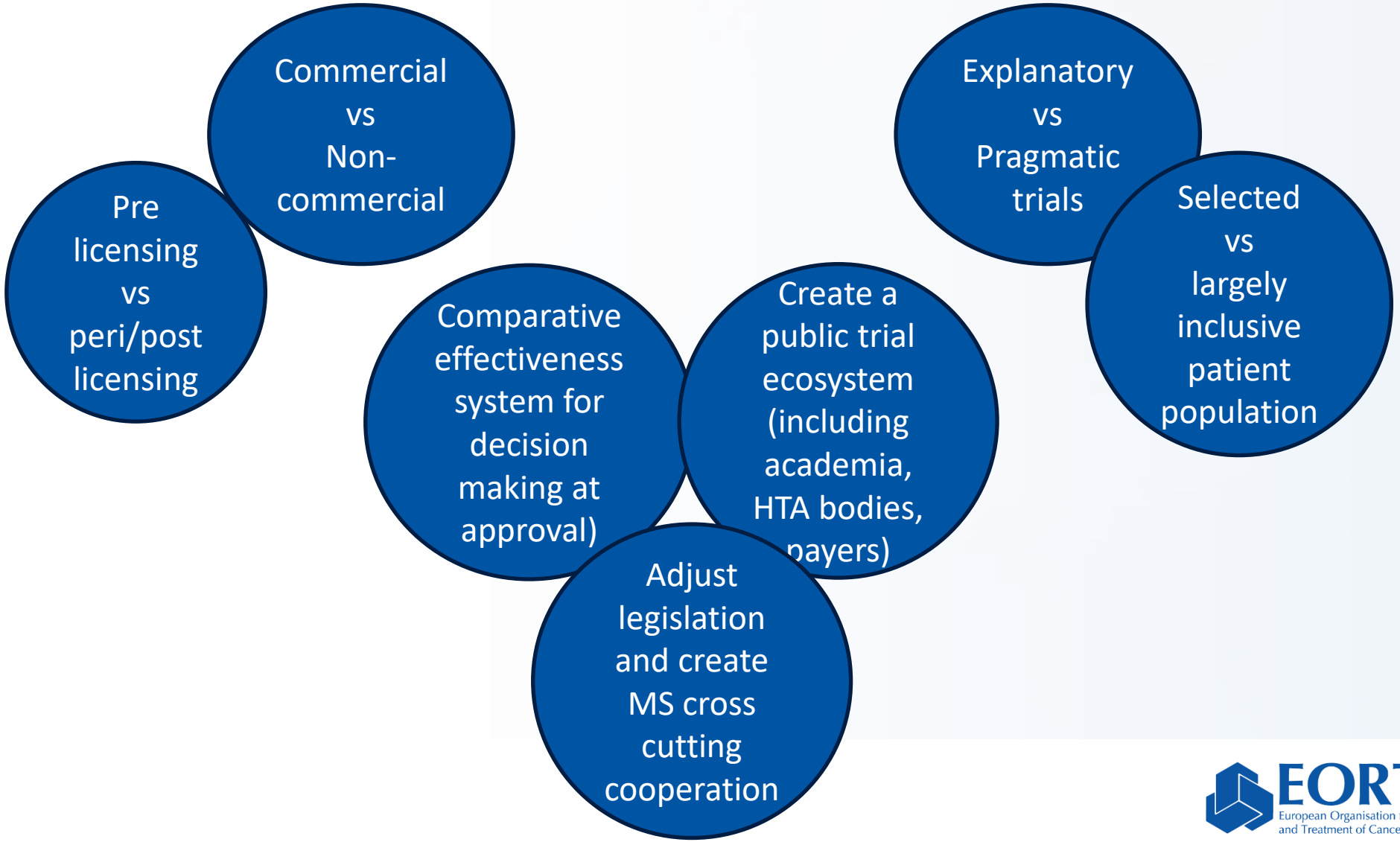
Addressing What-When-How-Who



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Treatment Optimisation is a spectrum

	What	Who	How	feasibility
Early stage Pre-licensing	Dose/safety R/B	Manufacturer	Regulatory process (i.e Optimus)	Explanatory trials
Late stage Post licensing	Combo, de-escalation, population, duration, schedule	Manufacturer academia	Public trial eco-system (adjust legislation)	Pragmatic trials Registry based trials Platform trials

Operational challenges to implementation

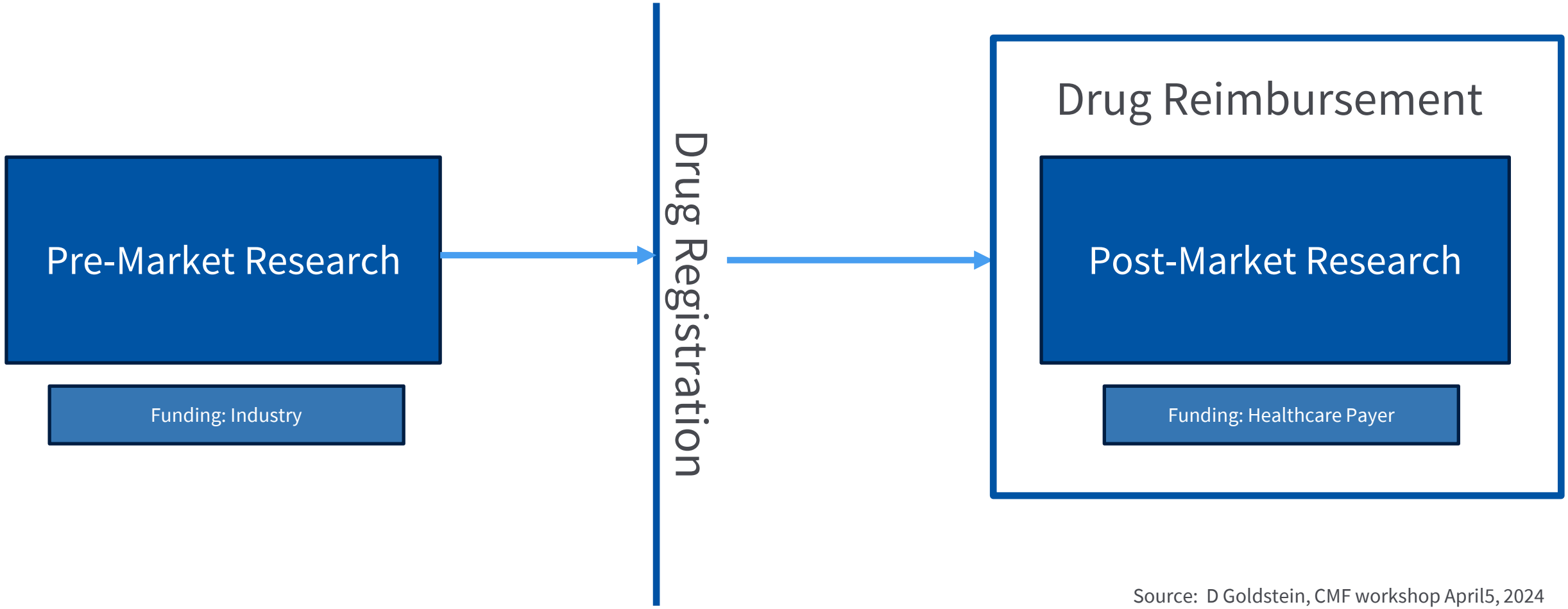
Methodological research:
Pragmatic trials, TwiCs…
Recruitment and ethics

Adapt the clinical research environment
Education of stakeholders

Reporting and access to datasets

Health economic dimension and funding

Drug Development & Approval Process

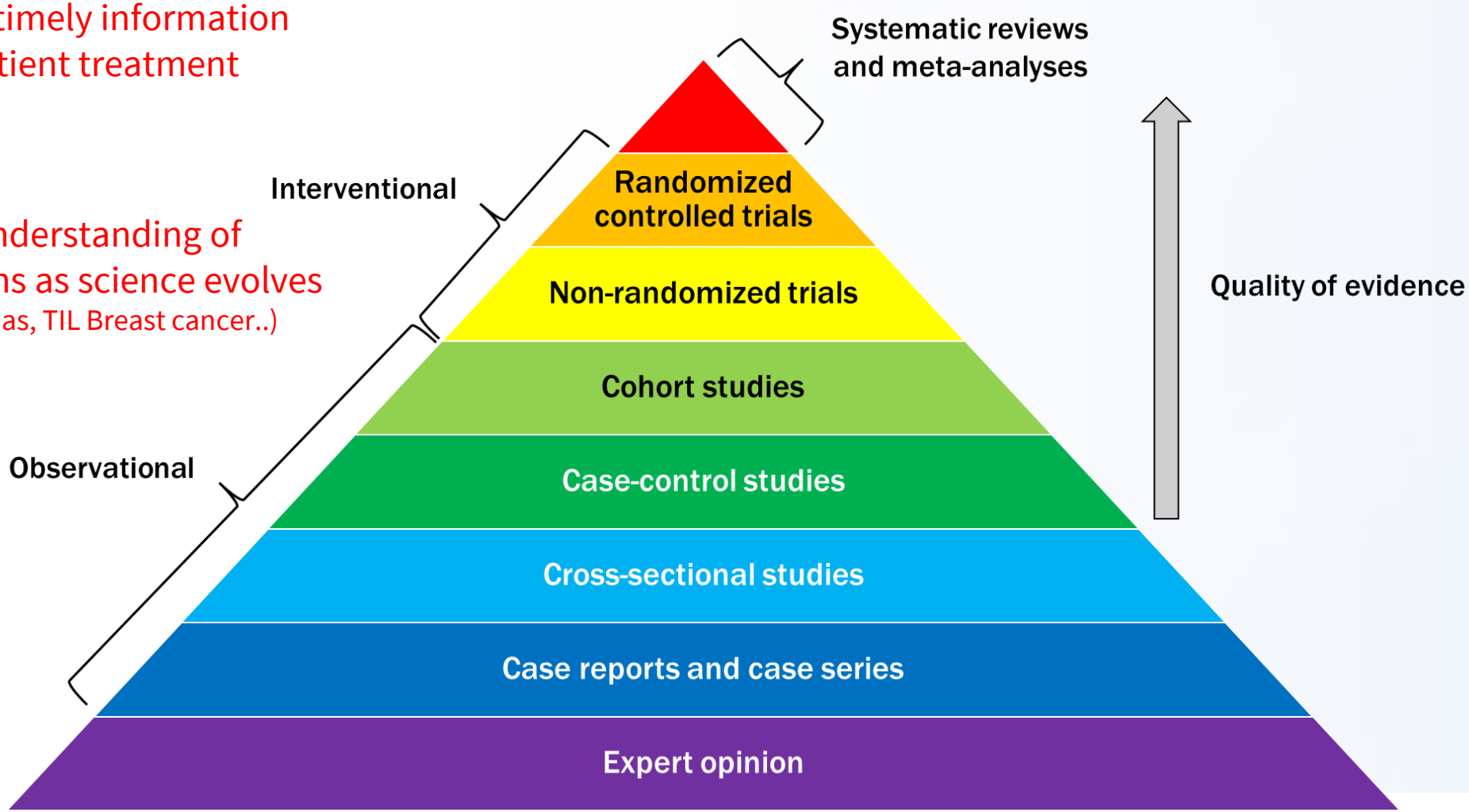


Source: D Goldstein, CMF workshop April5, 2024

Continued refinement of the pyramid of evidence-based medicine

Thorough and timely information to guide patient treatment

Continued understanding of patient populations as science evolves
(LOH 1p/19q gliomas, TIL Breast cancer..)



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Key directions for treatment optimisation

- Become more systematic about optimisation questions, when to address them, pre vs post licensing, the role of stakeholders, including independent research
- Foster pragmatic trials focusing on clinically relevant questions and informing on patient centric end-points
- Ensuring a visible (regulatory/policy) link between post-authorisation optimisation and regulatory frameworks
- Develop international mechanisms at the level of healthcare systems to facilitate and finance independent clinical trials
- Re-engineer the methods of development –access with new forms of collaboration defining the roles of stakeholders alongside the continuum to deliver rationale therapeutic progress

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