Personalized Healthcare

"Fitting the treatment to the patients"

Forum για τα Οικονομικά & τις Πολιτικές Υγείας

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Personalised Healthcare (PHC) – What does it stand for?

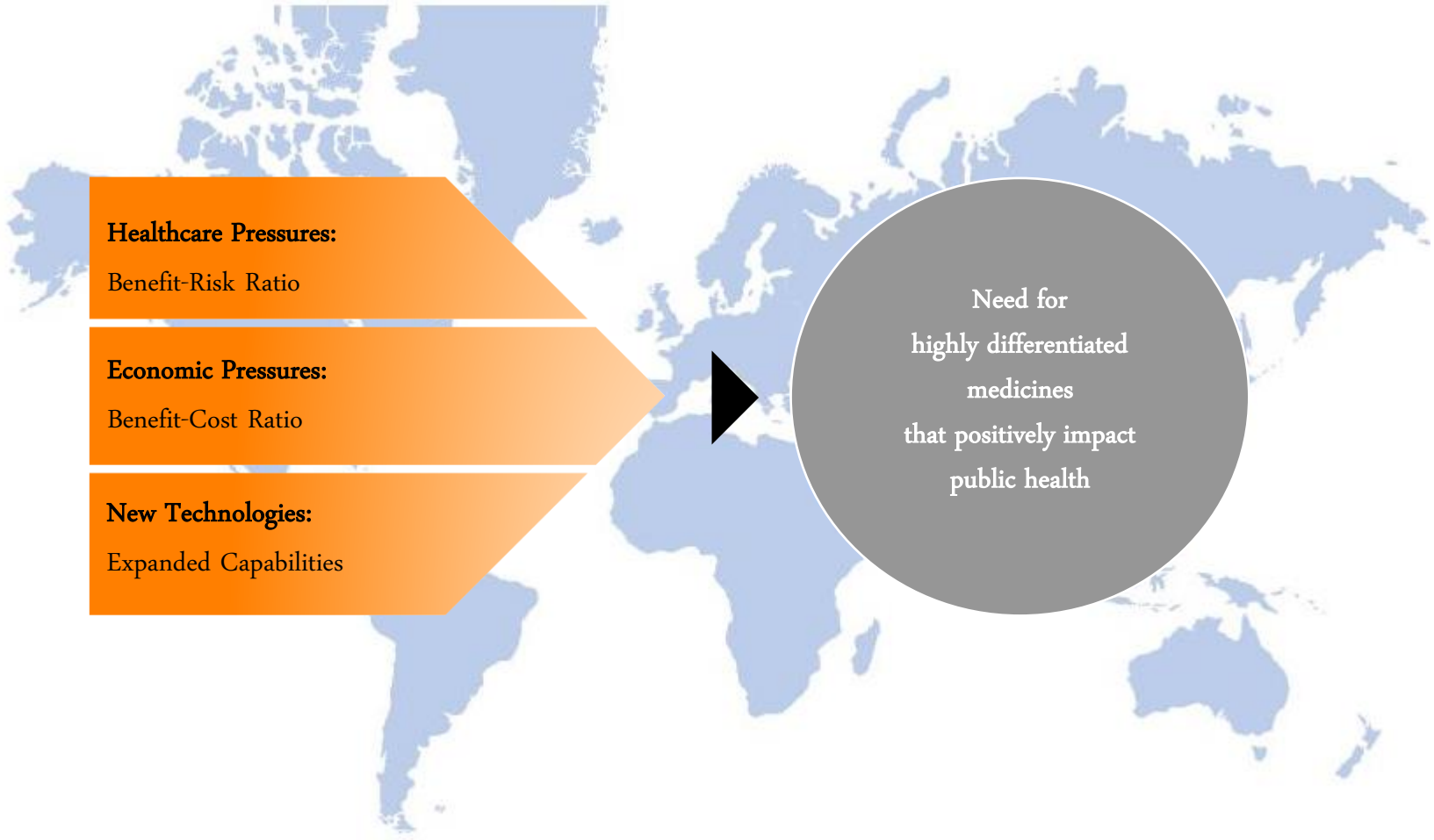
- Use of genetic or other biomarker information to improve safety, effectiveness and health outcomes of patients via:
  - Efficiency-targeted patient stratification
  - Prevention
  - Tailored medication
  - Tailored treatment-management approaches

Sources:

1. Faulkner E. et al - Value in Health 15 (2012): Challenges in the Development and Reimbursement of Personalized Medicine – Payer and Manufacturer Perspectives and Implications for Health Economics and Outcomes Research: A Report of the ISPOR Personalized Medicine Special Interest Group
2. Willard H et al – Academic Press 2010: Essentials of Genomic and Personalized Medicine.

Personalised Healthcare (PHC) - driver of change

Key to enabling highly differentiated medicines



PHC - Fitting the treatment to the patients

Delivering better, safer and more efficacious treatments

Personalised Healthcare approach enables experts:

- To better understand disease diversity or subtypes
- To identify the differences between patients
- To identify the best drug targets
- To improve the quality and efficiency of R&D results
- To provide biomarkers and diagnostic tests

Optimising patient care

Making development of new tests and drugs more efficient

PHC - Fitting the treatment to the patients

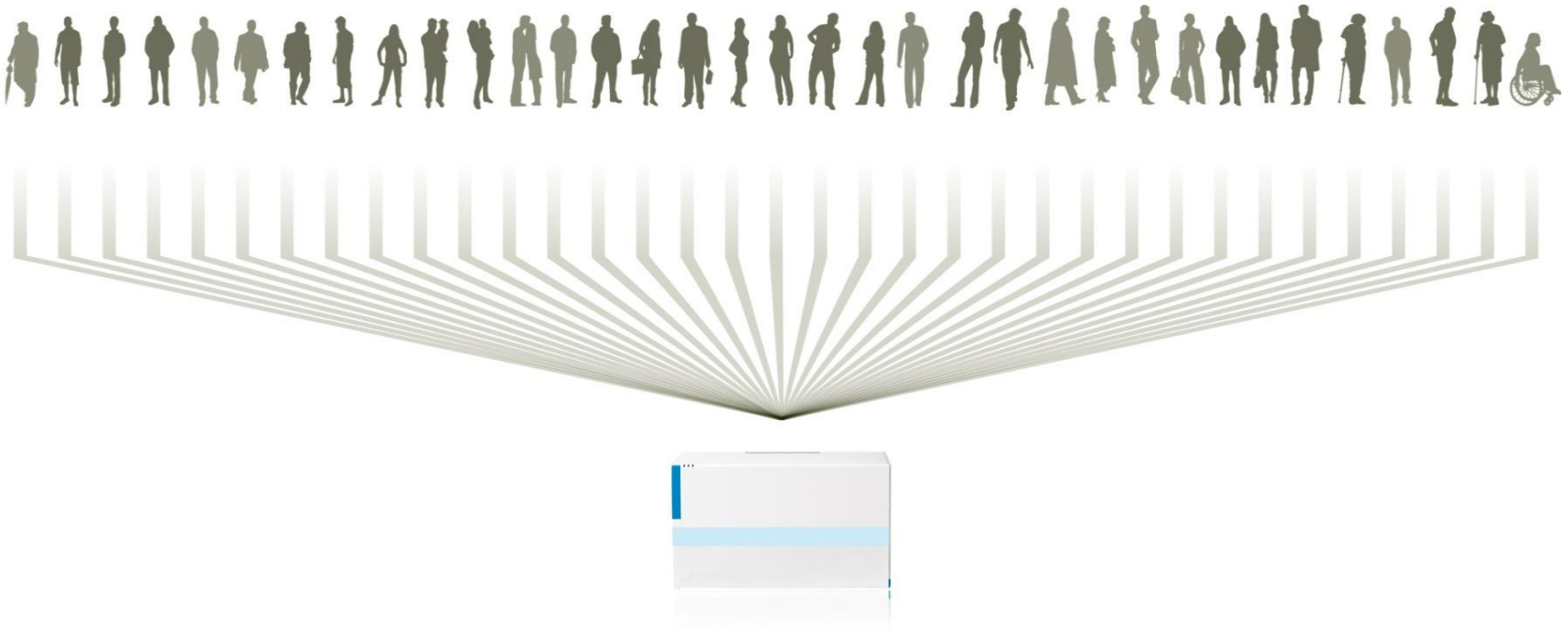
Delivering better, safer and more efficacious treatments

Personalised Healthcare enables physicians today:

- ✓ to stratify patients with specific characteristics, such as mutation-carrying tumors (e.g. HER2, BRAF, EGFR)
- ✓ to increase quality of prognosis (e.g. HBV, HCV)
- ✓ to define treatment duration (e.g. HCV)
- ✓ to identify optimal treatment sequence
  (e.g. Erlotininb first-line in EGFR-positive lung cancer)
- ✓ to monitor treatment success (e.g. HIV, HBV, HCV)

Personalised Healthcare versus standard treatment

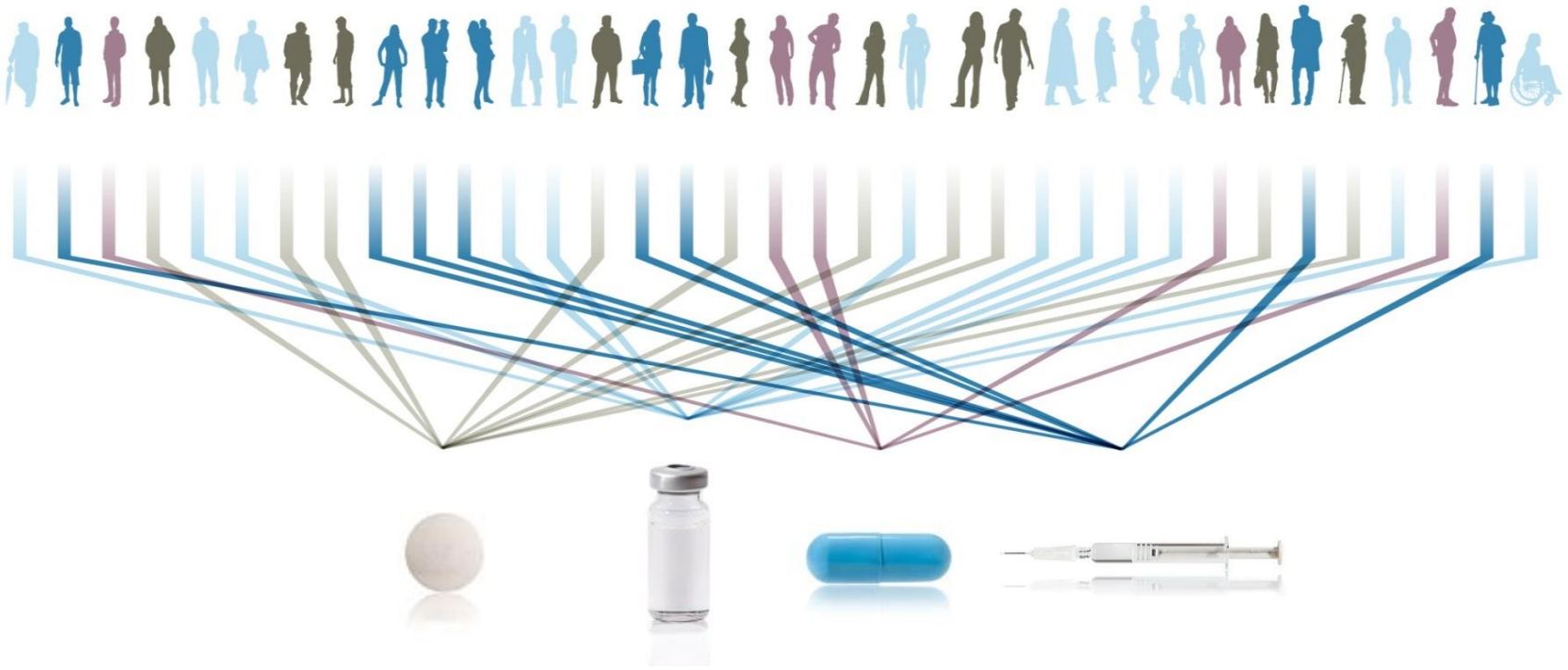
Patients with same syndrome



One-size-fits-all approach

Personalized Healthcare versus standard treatment

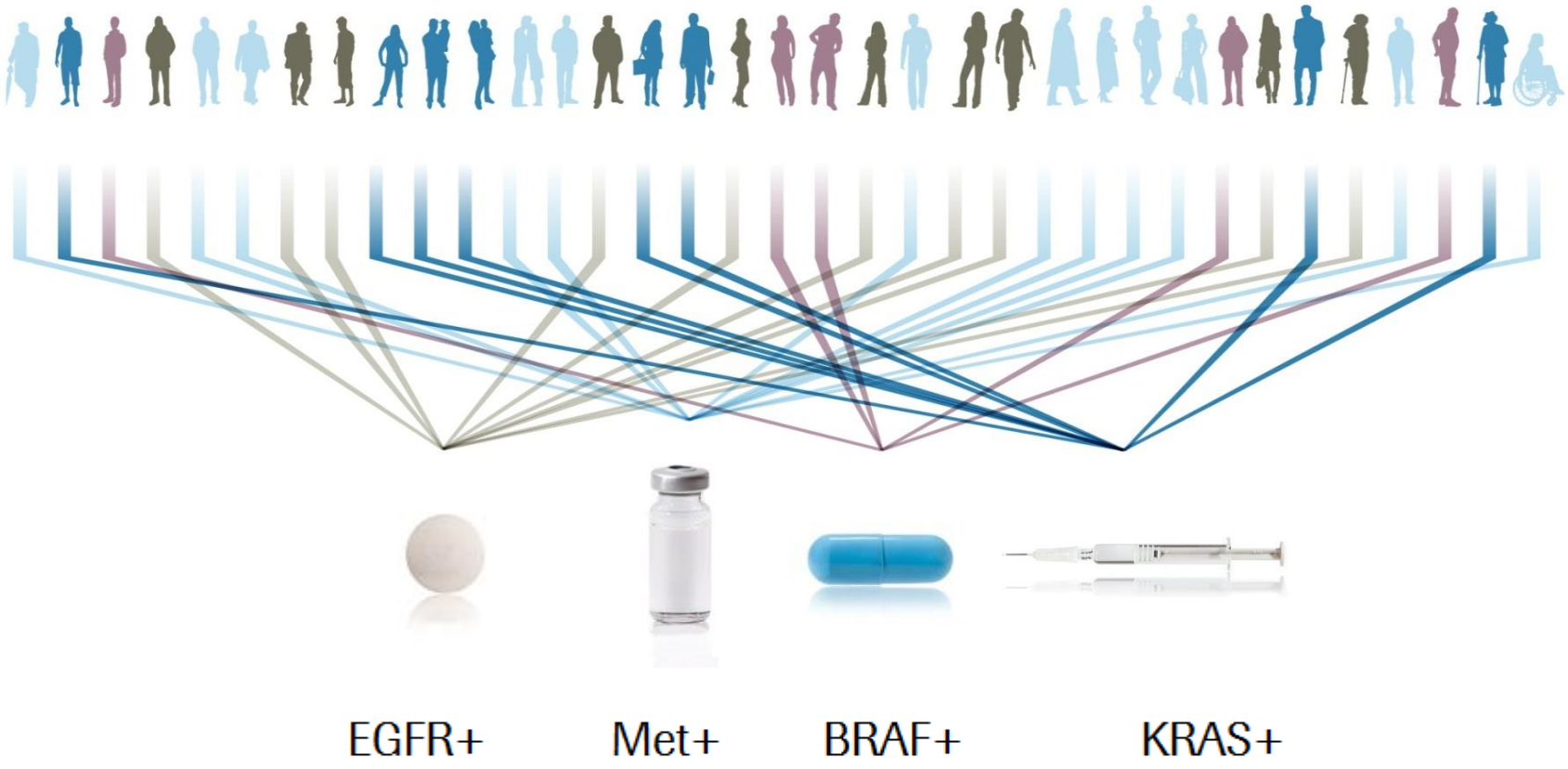
Groups of patients with the same syndrome



Targeted therapy

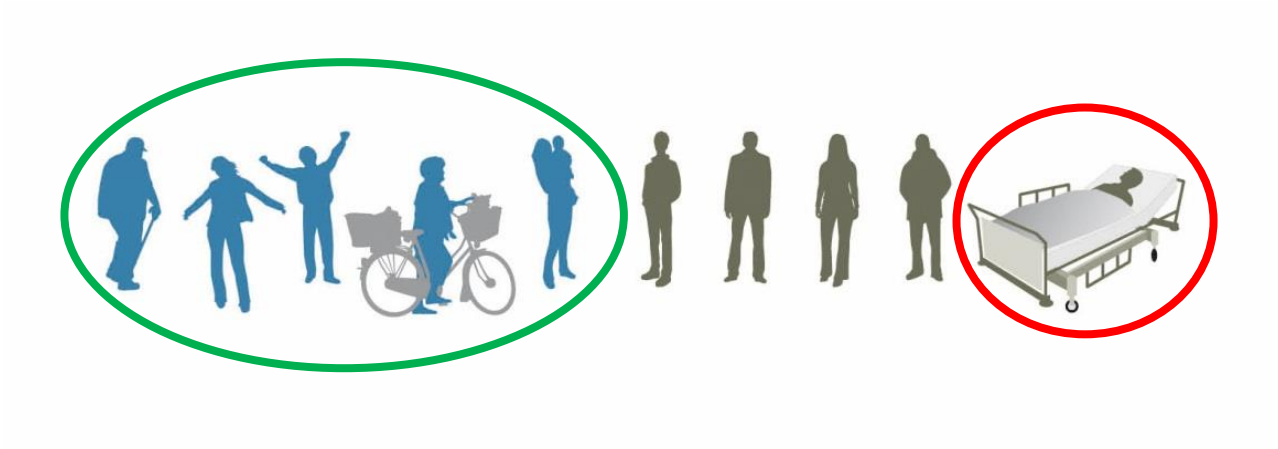
Personalized Healthcare vs. standard treatment

Groups of patients with the same syndrome: non-small lung cancer



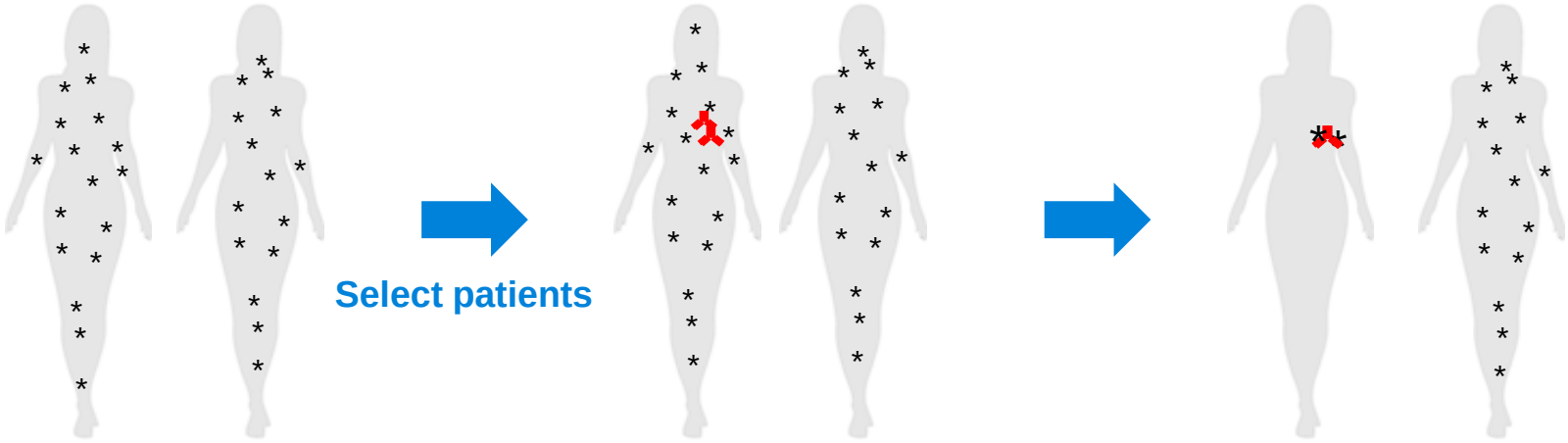
One size doesn't fit all – not tolerable any more

More efficient, targeted approach required by stakeholders



Traditional approach: out of 10 patients treated on average about half of them benefit. For some the treatment won't have any effect, and some may even suffer from side effects.

Evolution of Personalized Healthcare – The future is exciting…



Chemotherapy

e.g. paclitaxel

Targeted therapy + chemotherapy

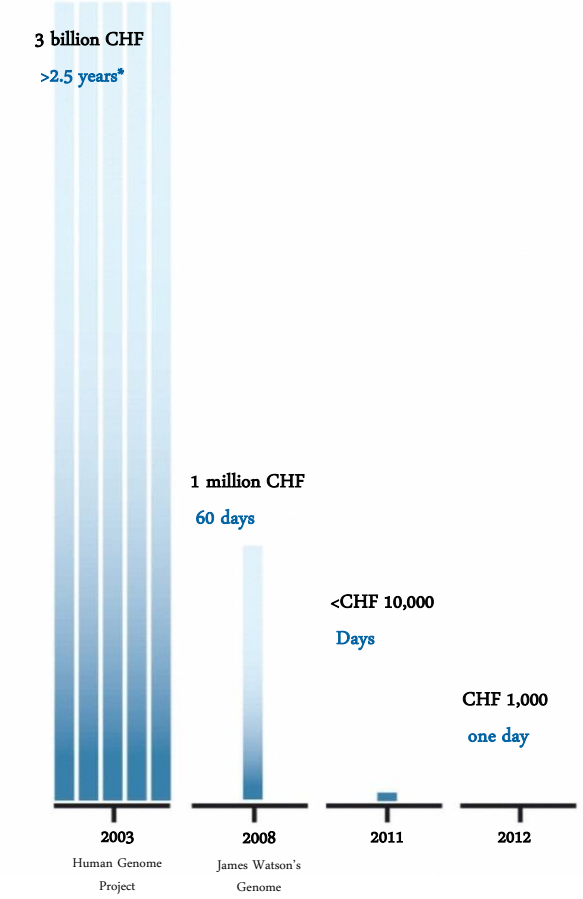
e.g. trastuzumab

Highly targeted Antibody-Drug Conjugates

e.g. T-DM1

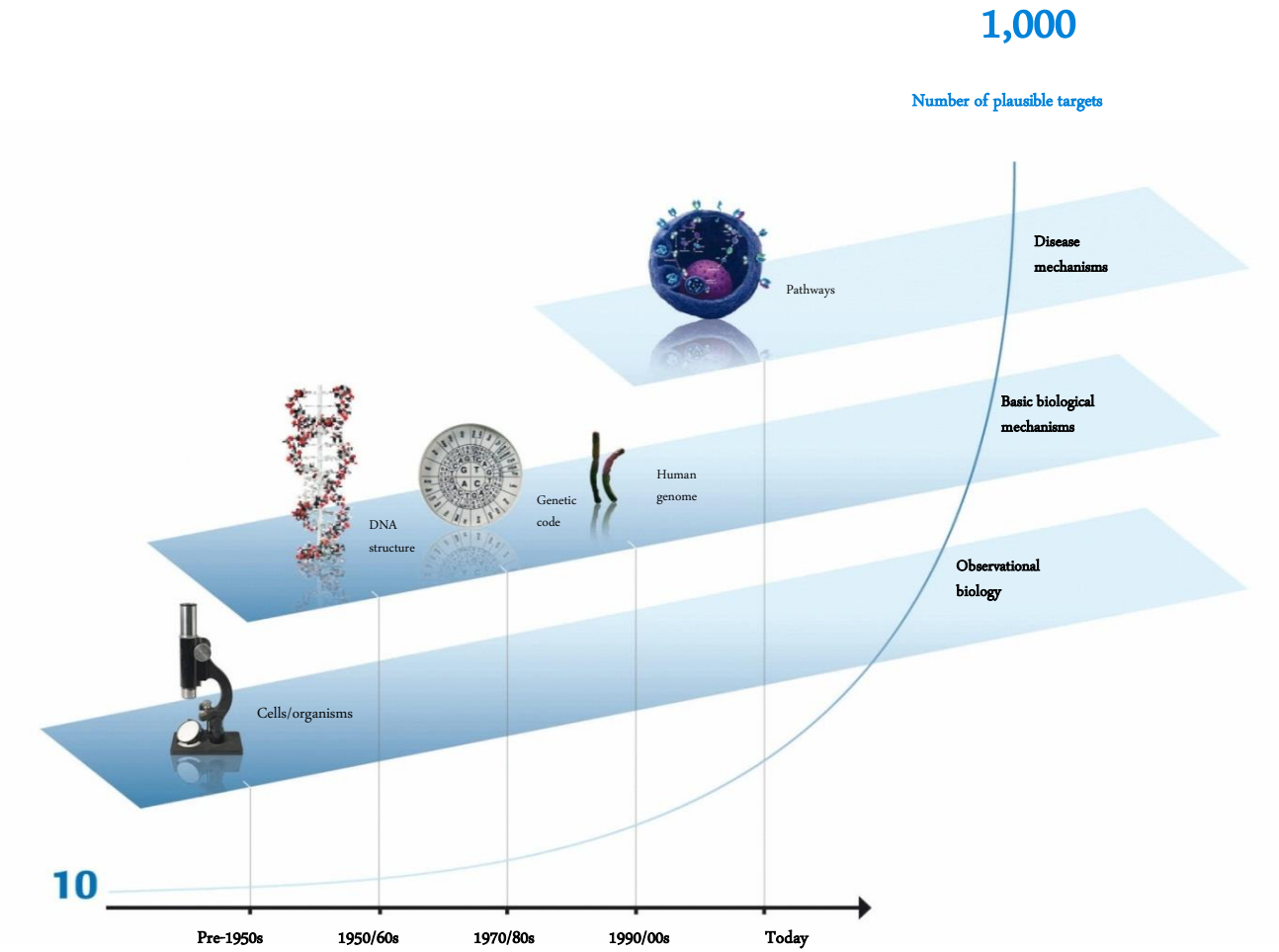
Example: gene-sequencing – much faster at significantly lower costs

Cost and time involved in gene-sequencing



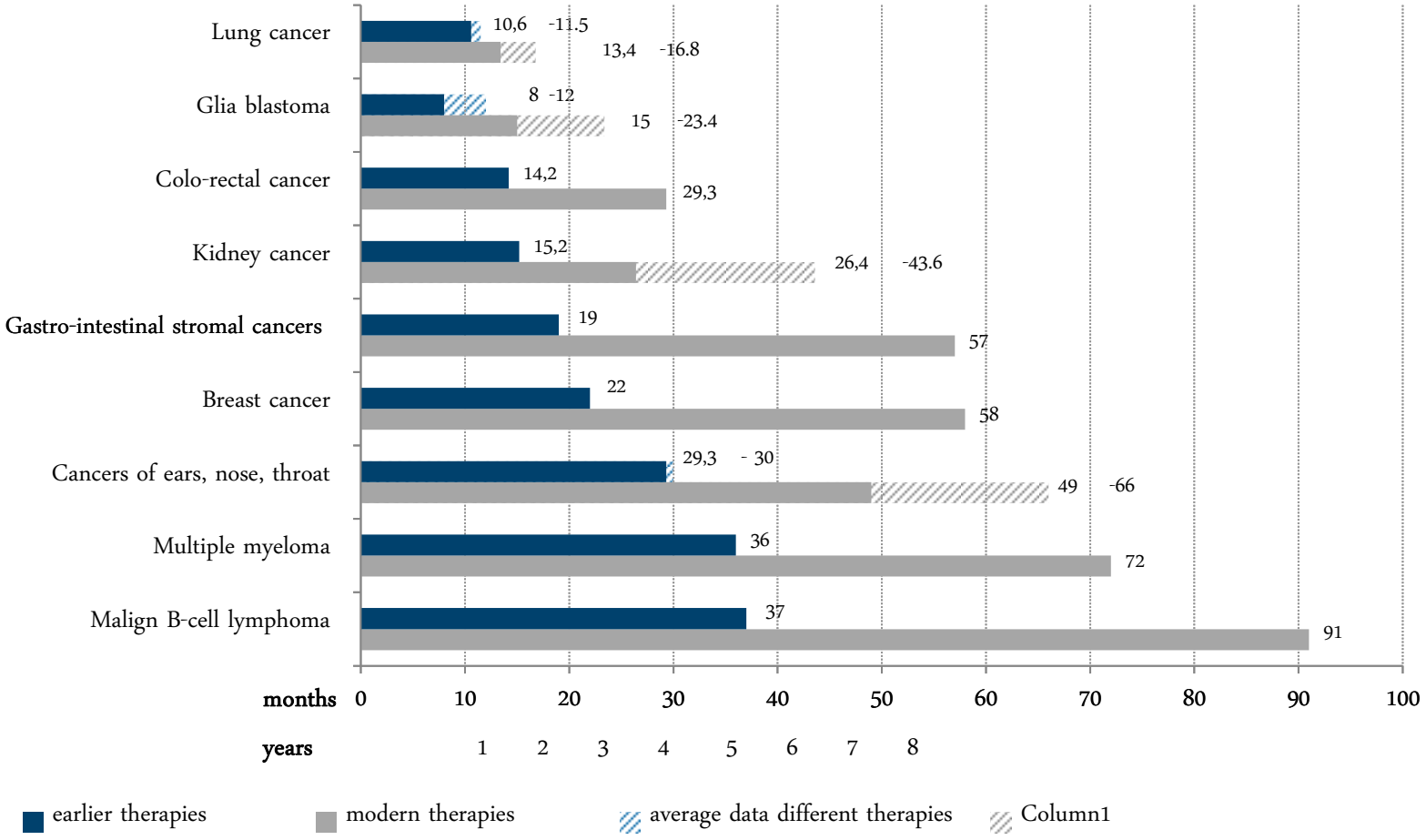
* including preparations almost 13 years

New technologies allow better insights and deeper understanding of diseases



Development of, Overall Survival‘ *

Advanced cancer – progress seen in 2000 - 2010



Source: Prof. Ch. Zielinski, University Vienna, Austria, March 2010, „Ergebnisoptimierung in der Therapie maligner Erkrankungen durch moderne Behandlungsstrategien: Einfluss auf die Überlebensdauer von PatientInnen mit Krebserkrankungen, http://www.onkologie-wien.at/forschung-und-lehre/positionspapier/;

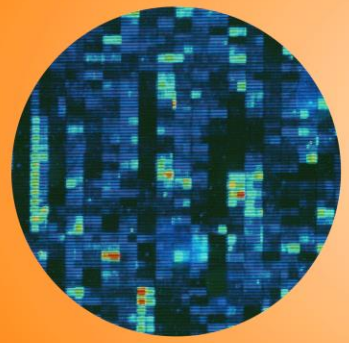
* average data, in months

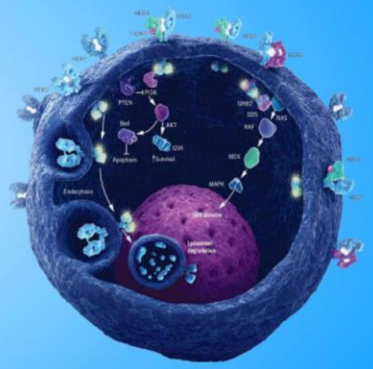
Combination of Diagnostic tools & targeted therapies

Translating excellence in science into effective treatments for patients

Companion Diagnostics

Pharmaceuticals

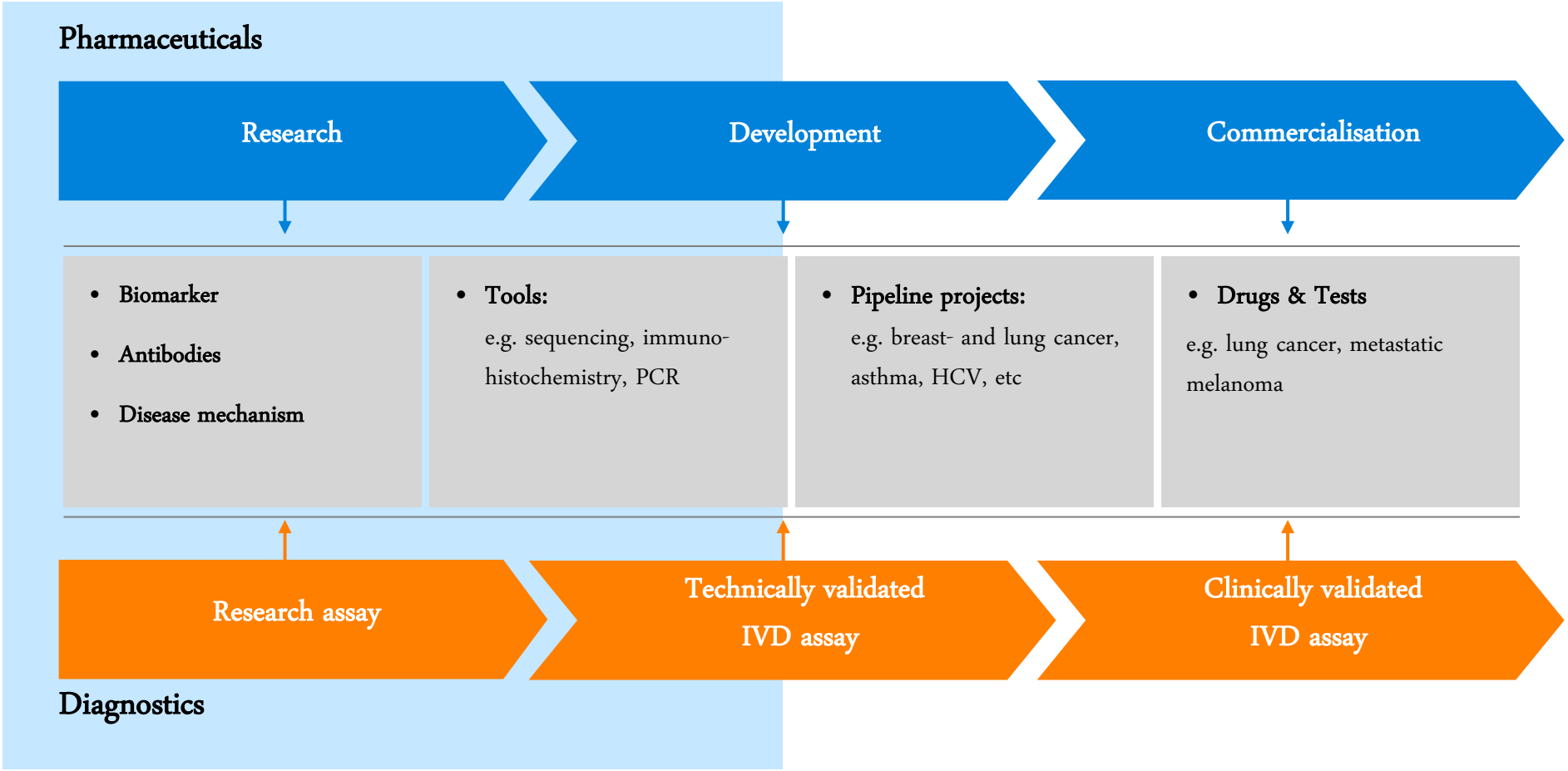




→ Combine molecular biology and technologies to benefit patient

Personalized Healthcare at the center of Research & Development

Diagnostics input – from discovery to market



Biomarkers – What are they?

- Measurable characteristics (indicators) that reflect the presence or severity of some disease state
  - Can be chemical, physical or biological
- A biomarker indicates a change in expression or state of a protein that correlates with the risk or progression of a disease, or with the susceptibility of the disease to a given treatment
  - Characteristic biological properties that can be detected and measured in parts of the body like blood or tissue
- May indicate either normal or diseased processes in the body
  - Help in early diagnosis, disease prevention, drug target identification, drug response
- Can be specific cells, molecules, genes, gene products, enzymes, hormones
  - Several biomarkers have been identified for many diseases such as serum LDL for cholesterol, gene mutations for cancer etc.

Source:

1. The Biomarkers Consortium". Foundation for the National Institutes of Health.

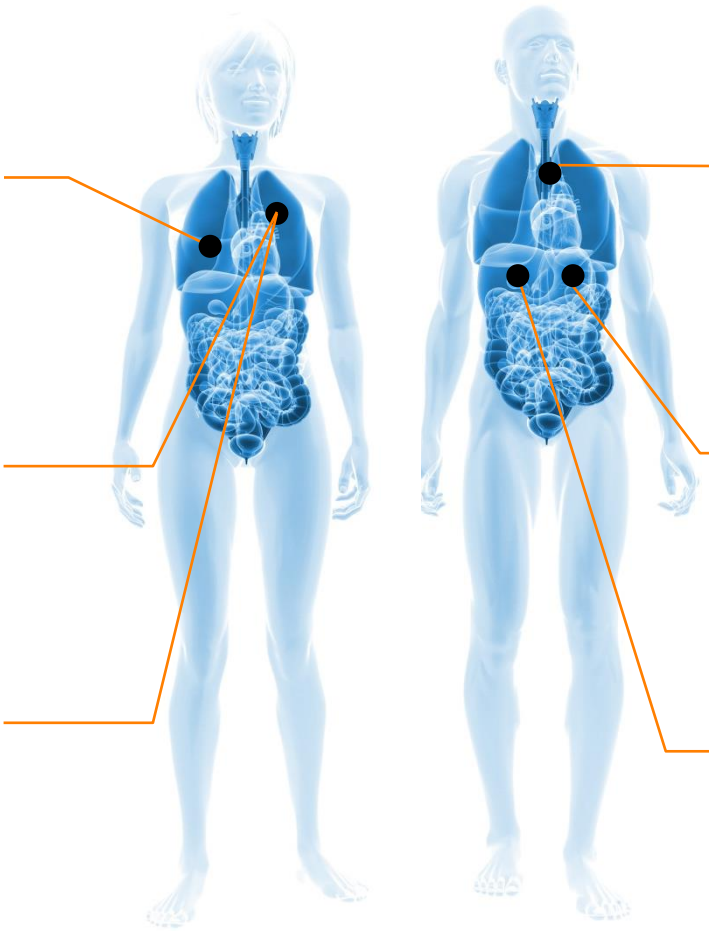
Biomarkers to guide treatment decisions

Diseases requiring sub-group identification

Metastatic breast cancer
15 – 20% HER2 positive

Non-small cell lung cancer I
10 – 30% EGFR positive

Non-small cell lung cancer II
~ 50% cMET positive



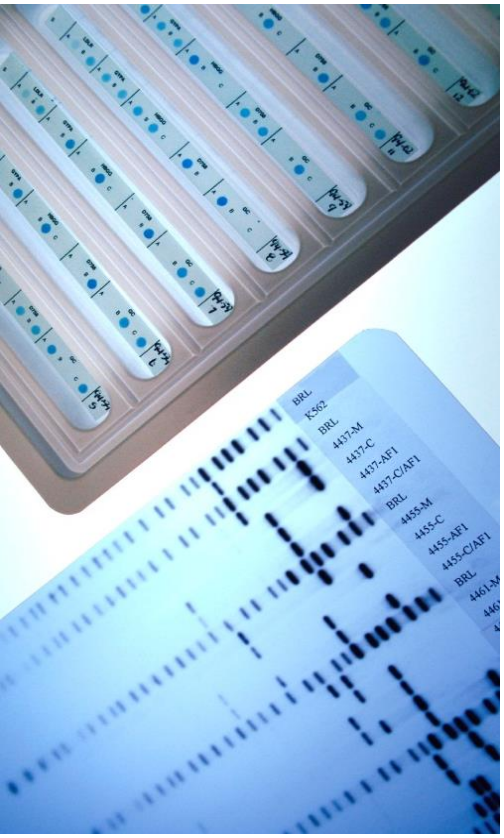
Asthma
~ 50% Periostin positive

Stomach cancer
16 – 22% HER2 positive

Metastatic melanoma
~ 50% BRAF positive

Importance of biomarkers

The 'Navigator' in highly complex systems



Biomarkers are critical for

- Identifying subgroups of patients
  (e.g. optimised patient stratification)
- Improved decision making in R&D
  (e.g. tools for profiling targets or compounds)
- Understanding pathways and mechanisms
- Drivers for pharmaco-diagnostic development
  (e.g. increased benefit/ risk ratio, companion diagnostics)

Biomarkers support navigation throughout the entire lifecycle of a medicine - from target identification to market

Use of biomarkers

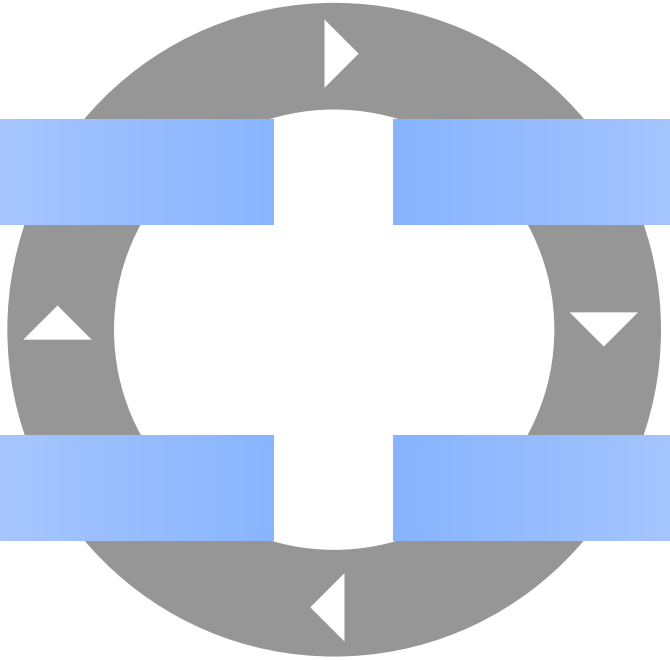
Increase patient safety

Toxicity Biomarkers

Patient Biomarkers

Verify the impact of
novel drugs on targets/pathways

Achieve the goal of
Personalized Healthcare



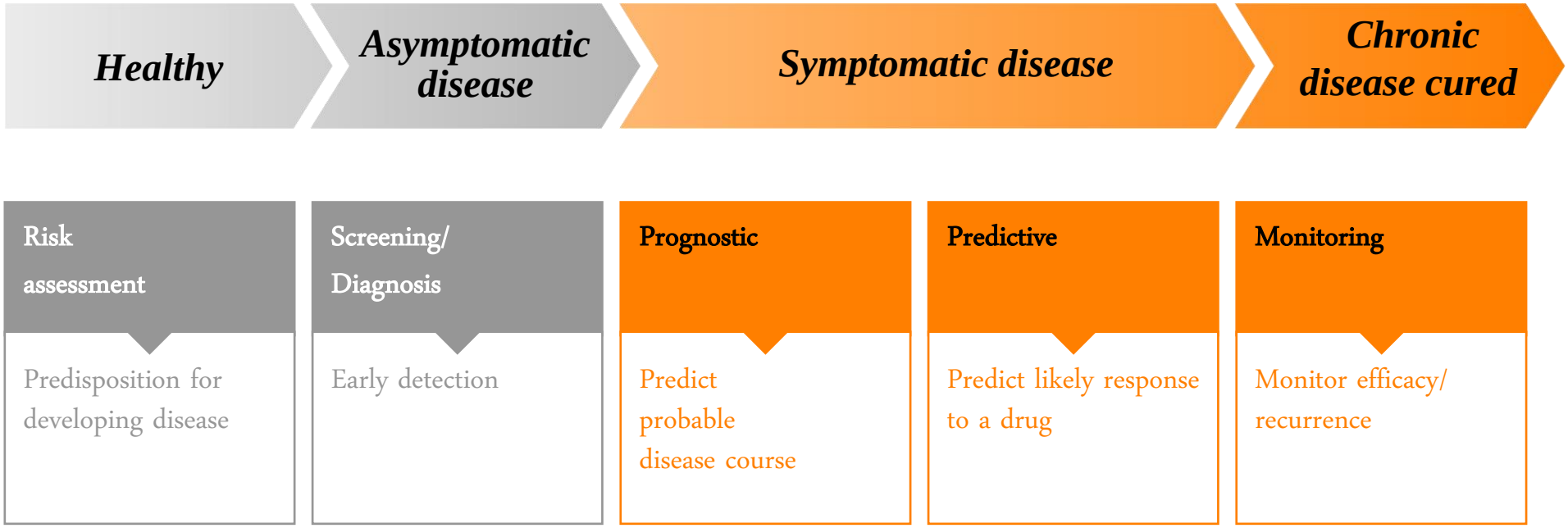
Efficacy Biomarkers

Disease Biomarkers

Treat diseases more effectively

Biomarker development – why ?

Most likely to benefit / most effective treatment



Biomarker development – why ?

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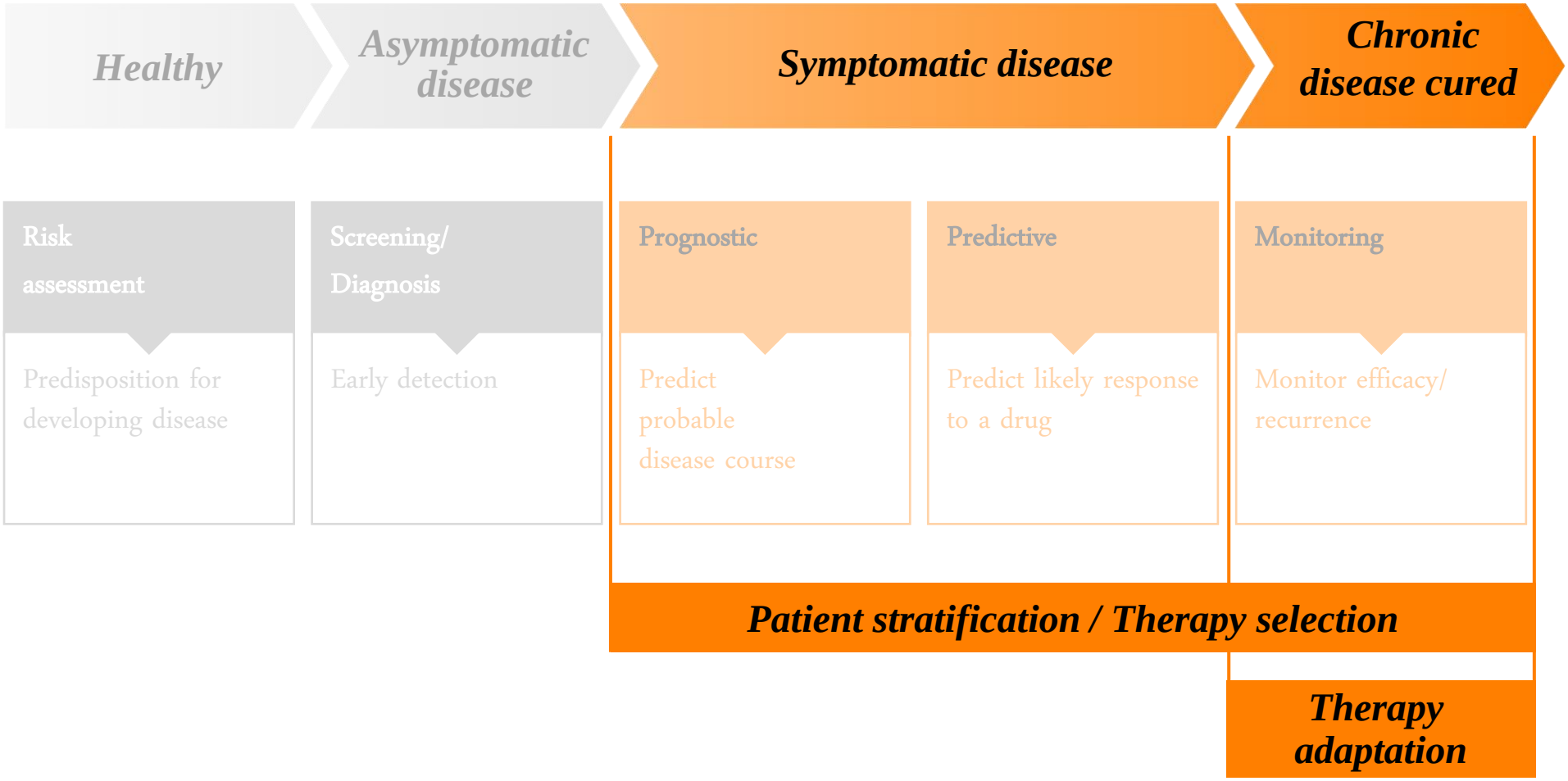
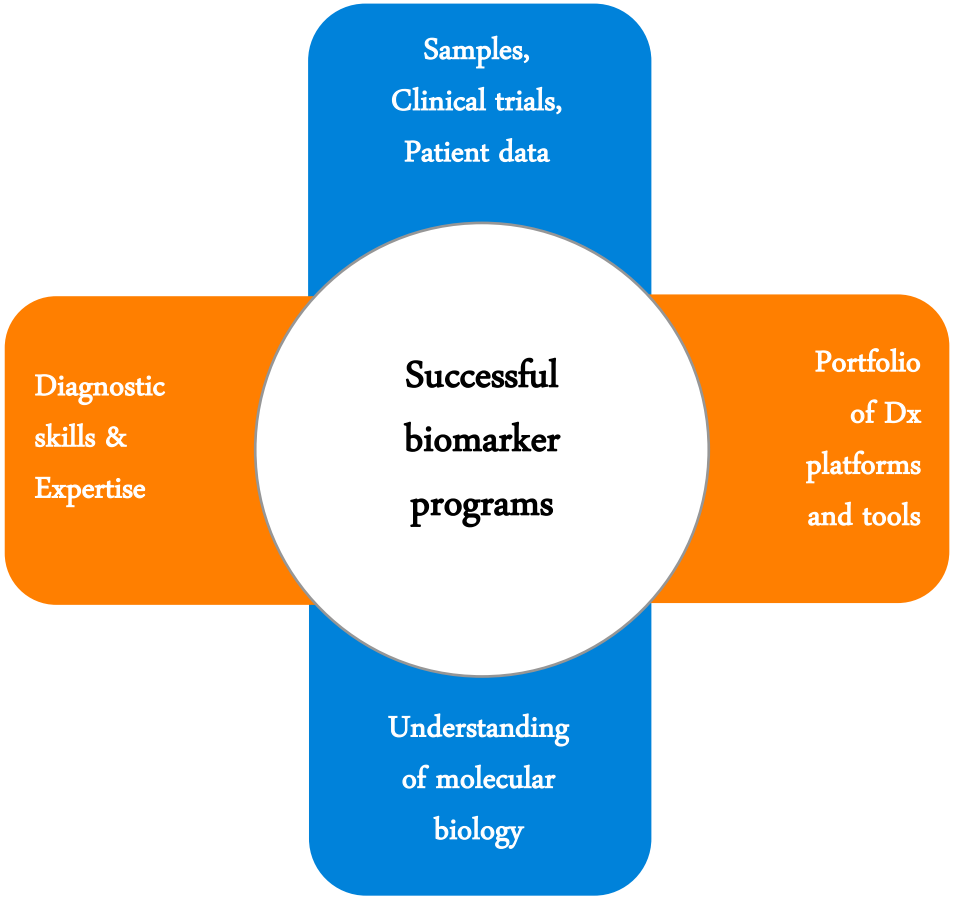


Biomarker development – what it takes

A plethora of tools, skills and capabilities needed



Pharmaceuticals

+

Diagnostics

=

Highly linked information network

Challenges with PHC

- PHC technologies do not neatly fit into existing HTA and reimbursement process

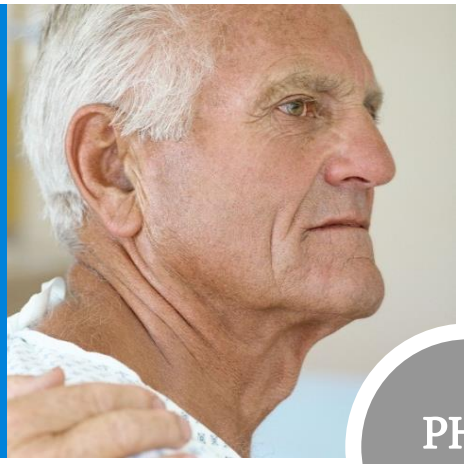
- Revision of the current regulatory framework is necessary with respect to:
  - Clinical evidence development and outcomes research requirements
  - Health economic assessment
  - Value assessment & decision standards
  - New incentives and reimbursement approaches for PHC

Source: Faulkner E. et al, Value in Health 15 (2012): Challenges in the Development and Reimbursement of Personalized Medicine – Payer and Manufacturer Perspectives and Implications for Health Economics and Outcomes Research: A Report of the ISPOR Personalized Medicine Special Interest Group

PHC benefits all stakeholders in healthcare

Patients

→ Best treatment at the right time



Physicians & Providers

→ Maximum benefit

→ Minimum toxicity

PHC

Payers & Reimbursing bodies

→ Efficient use of healthcare budgets

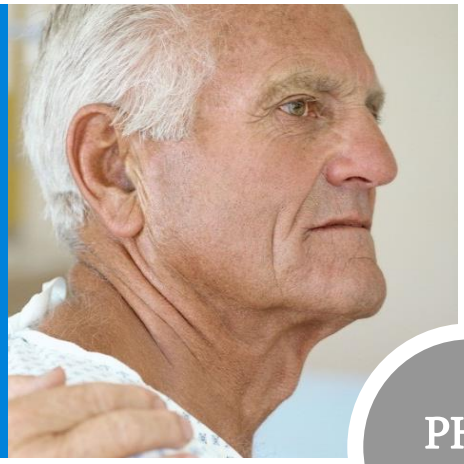
Regulators & Policy Makers

→ Increased efficacy & safety

Focus on patients and payers

Patients

→ Best treatment at the right time



PHC

Payers & Reimbursers

→ Efficient use of healthcare budgets

Benefits can be:

- Better and more predictable clinical outcomes
- Improved quality of life and lifetime gained
- Reduced morbidity
- Fewer unnecessary treatments/ side effects and associated costs
- Better compliance due to better results
- Optimized use of resources in healthcare

Conclusions

- Allocation of scarce healthcare resources should be done is such a way that:
  - Combination of “old” traditional treatment approaches and innovative PHC alternatives is optimized through a predictable and sustainable system
  - Access of stratified eligible patients to PHC treatment approaches should not be hindered or delayed

- Updated regulatory requirements framework and HTA systems to best fit the thorough evaluation of constantly evolving PHC

- PHC therapeutic options of demonstrated value:
    - Maximize benefits & minimize untoward effects for patients, society and healthcare system

Thank you