Patient Access in Cancer Drugs

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The Patient's Perspective

"Is there anything ... anything ... that exists that might work for me?"

The Patient's Perspective

"*A cynic is a man who knows the price of everything and the value of nothing*"

(Oscar Wilde)

What is value?

- Price changes significantly over time
- The value of innovation should be fully appreciated during period of exclusivity

2008 - *"Folfox costs more than 20 times the traditional regimen, which is quite an enormous cost"*

(Gary Lyman MD, MPH, an oncologist and health outcomes researcher at the Duke University Comprehensive Cancer Center)

2017 - Folfox *"was the least expensive with 56€ per month of progression-free survival)"*

(Andrea Bonetti - study presented at the annual congress of the American Society of Clinical Oncology (ASCO)

Andrea Bonetti, Jcopo Giuliani - Cost-effectiveness of front-line trials in metastatic colorectal cancer: Integrating the European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS) with the costs of drugs. Journal of Clinical Oncology 35, 2017

What is value?

Example of breast cancer – analysis in Belgium: reduced morbidity and mortality off-set the increase in direct healthcare costs by 3,896 euro/patient

	2000	2017
Average health care cost per patient	18,859 €	32,355 €
Average productivity costs due to morbidity per patient	43,306 €	42,903 €
Average productivity costs due to mortality per patient	41,250 €	24,261 €
TOTAL COSTS PER PATIENT	103,414 €	99,518 €
Difference in cost per patient	-3,896 €	

The Value of Medicines in Belgium, Pharma.be, 2019

European Code of Cancer Practice

1. Equal Access

You have a right to:
Equal access to affordable and optimal cancer care, including the right to a second opinion

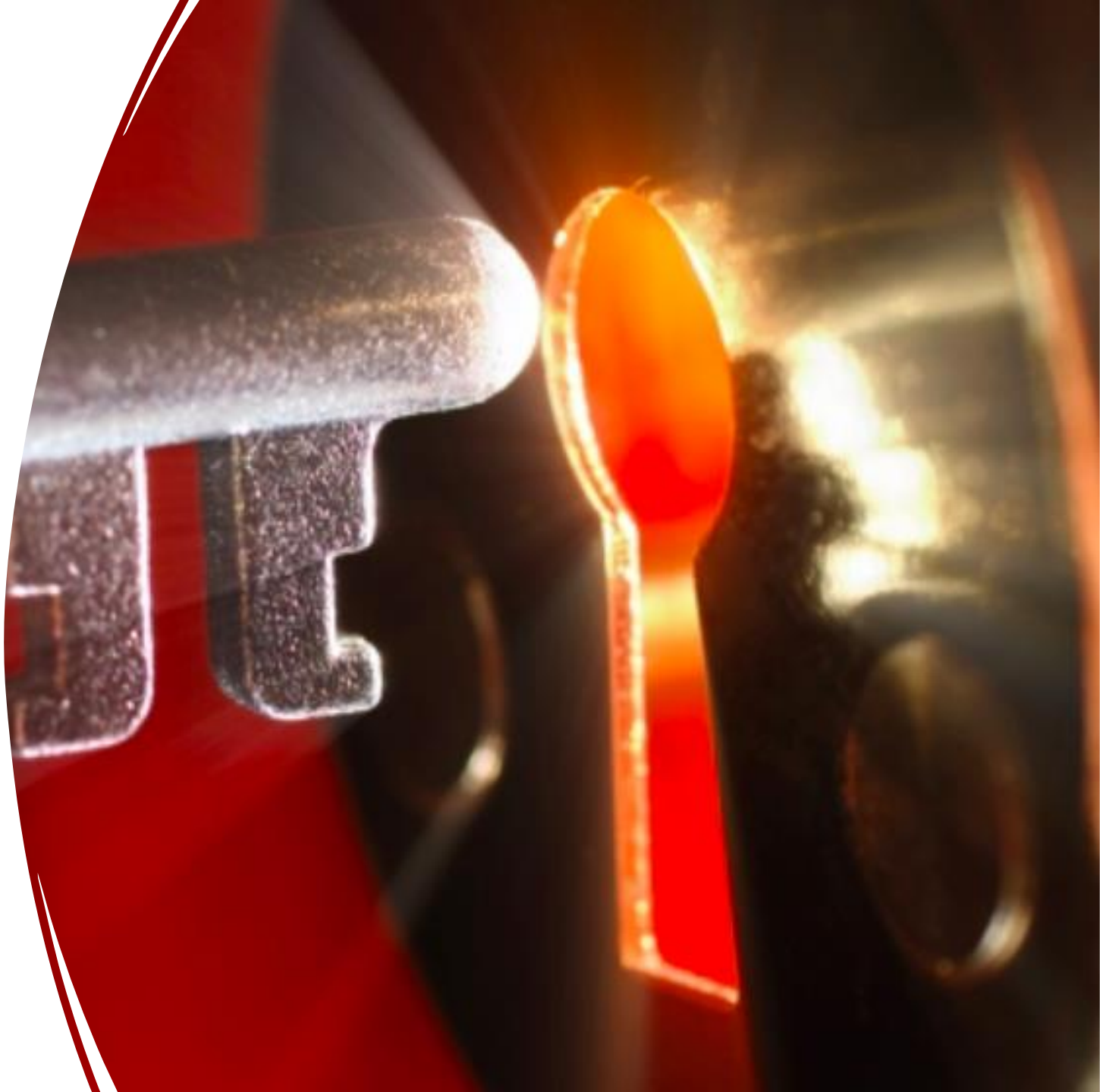
If patient X gets access to a new drug, so should patient Y
- early access
- access between countries

It is the patient's fundamental and ethical right to have access to innovative new treatments

Source: European Code of Cancer Practice, European Cancer Organisation, 2019

Early Access

- Unmet medical need: "*seriously-debilitating or life-threatening diseases that cannot be treated satisfactorily by available therapeutics*"
- FDA
  - Fast Track Approvals
  - Expanded Access
  - Compassionate use
- EU
  - Conditional Approvals
  - Early Access Programmes
  - Named-Patient Programmes
  - Compassionate use



Early Access – What If?

- Why not make cancer drugs accessible to all patients who fit the required profile after Phase II with tight remote patient monitoring and pharmacovigilance?
- Our regulatory environment and the requirement for three phases dates from the analog 1960s. Today, we are able to capture patient data in a more systematic and robust way than ever before.
- Patients are willing to take risks if it means that their chances of survival increase.

Early Access – What If?

- Better data capture, including non-clinical information through patient organisations could significantly improve insights into therapeutic value

Figure 2. Potential Outcomes in Early Access Programs[29]

Source: Evidera, Spring 2018

New cancer indications since 1999

2000	2001	2002	2003	2004	2005	2006	2007	2008	2009	2010	2011	2012	2013	2014	2015	2016	2017	2018	2019	2020
																				Ayvakit
																				Sarclisa
															Alecensa					Tazverik
															Cotellic					Koselugo
												Abraxane			Darzalex			Mektovi		Tukysa
												Afinitor			Empliciti			Braftovi		Pemazyre
												Afinitor			Farydak		Aliqopa	Tibsovo		Trodelvy
												Bosulif			Ibrance		Alunbrig	Poteligeo		Tabrecta
												Cometriq			Imlygic		Bavencio	Lumoxiti		Retevmo
												Erivedge	Gazyva		Keytruda		Besponsa	Copiktra		Qinlock
												Iclusig	Gilotrif		Lenvima		Calquence	Vizimpro	Balversa	Cerianna
											Adcetris	Inlyta	Imbruvica		Lonsurf		IDHIFA	Libtayo	Piqray	Zepzelca
											Afinitor	Kyprolis	Kadcyla	Beleodaq	Ninlaro	Cabometyx	Imfinzi	Talzenna	Polivy	Monjuvi
									Afinitor		Erwinaze	Marqibo	Mekinist	Blincyto	Odomzo	Keytruda	Kisqali	Lorbrena	Xpovio	Blenrep
									Arzerra		Sutent	Perjeta	Pomalyst	Cyramza	Onivyde	Lartruvo	Kymriah	Daurismo	Nubeqa	Detectnet
	Campath			Alimta					Avastin		Sylatron	Picato	Revlimid	Imbruvica	Opdivo	Lenvima	Nerlynx	Vitrakvi	Turalio	Gavreto
	Femara	Eloxatin	Bexxar	Avastin			Hycamtin		Cervarix	Halaven	Vandetanib	Stivarga	Stivarga	Keytruda	Opdivo	Opdivo	Rydapt	Xospata	Rozlytrek	Danyelza
	Gleevec	Faslodex	Iressa	Clolar		Gardasil	Ixempra		Elitek	Herceptin	Xalkori	Synribo	Tafinlar	Lynparza	Portrazza	Opdivo	Verzenio	Asparlas	Ga-68-DOTATOC	Gallium 68 PSMA-11
	Trelstar LA	Gleevec	Plenaxis	Erbitux		Sprycel	Tasigna	Degarelix	Folotyn	Jevtana	Yervoy	Votrient	Valchlor	Opdivo	Tagrisso	Rubraca	Vyxeos	Elzonris	Brukinsa	Margenza
Mylotarg	Xeloda	Zevalin	UroXatral	Sensipar	Arranon	Sutent	Torisel	Mozobil	Istodax	Provenge	Zelboraf	Xtandi	Xgeva	Zydelig	Unituxin	Tecentriq	Yescarta	Erleada	Padcev	Orgovyx
Trisenox	Zometa	Zometa	Velcade	Tarceva	Nexavar	Vectibix	Tykerb	Treanda	Votrient	Xgeva	Zytiga	Zaltrap	Xofigo	Zykadia	Yondelis	Venclexta	Zejula	Lutathera	Enhertu	Gemtesa

>200!

Blockbusters: more than 1 billion USD/year

(Source: FDA, 2021)

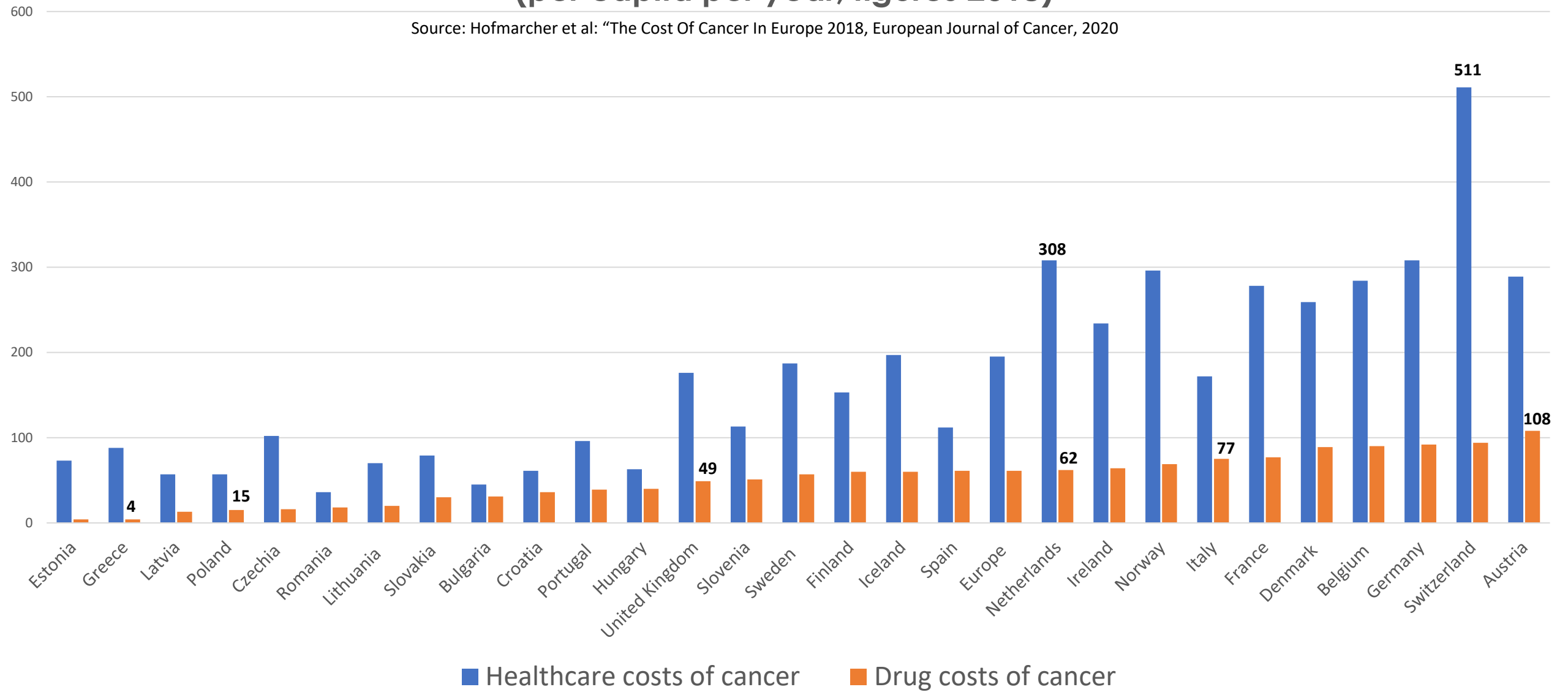
Observation – EMA approval is not access

- The timeframe between formal approval by the European Medicines Agency and patient access in Member States is unacceptable
- Patients are hostage in a negotiation dialogue between industry and governments
- Even a European HTA is not likely to change this

Cancer Healthcare cost and cancer drug cost in the European Union

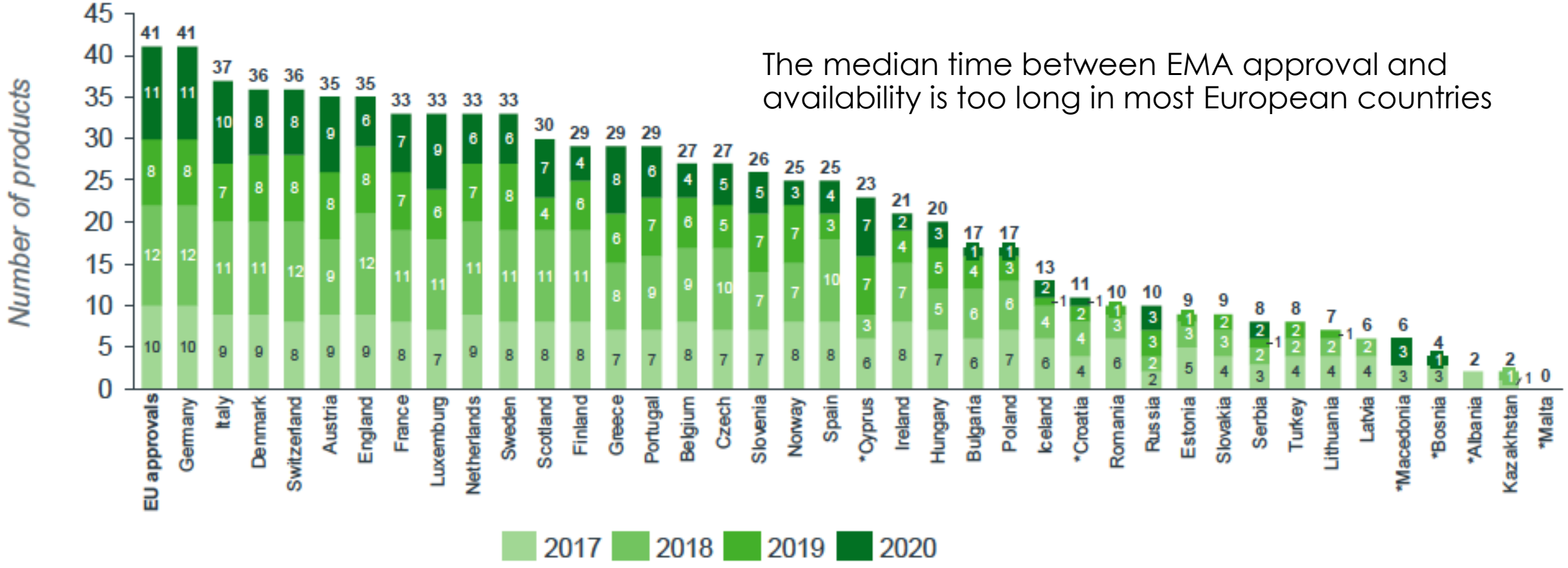
(per capita per year, figures 2018)

Source: Hofmarcher et al: "The Cost Of Cancer In Europe 2018, European Journal of Cancer, 2020



Oncology availability by approval year (2017-2020)

The **total availability by approval year** is the number of medicines available to patients in European countries as of 1st January 2022 (for most countries this is the point at which the product gains access to the reimbursement list†), split by the year the product received marketing authorisation in Europe.



The median time between EMA approval and availability is too long in most European countries

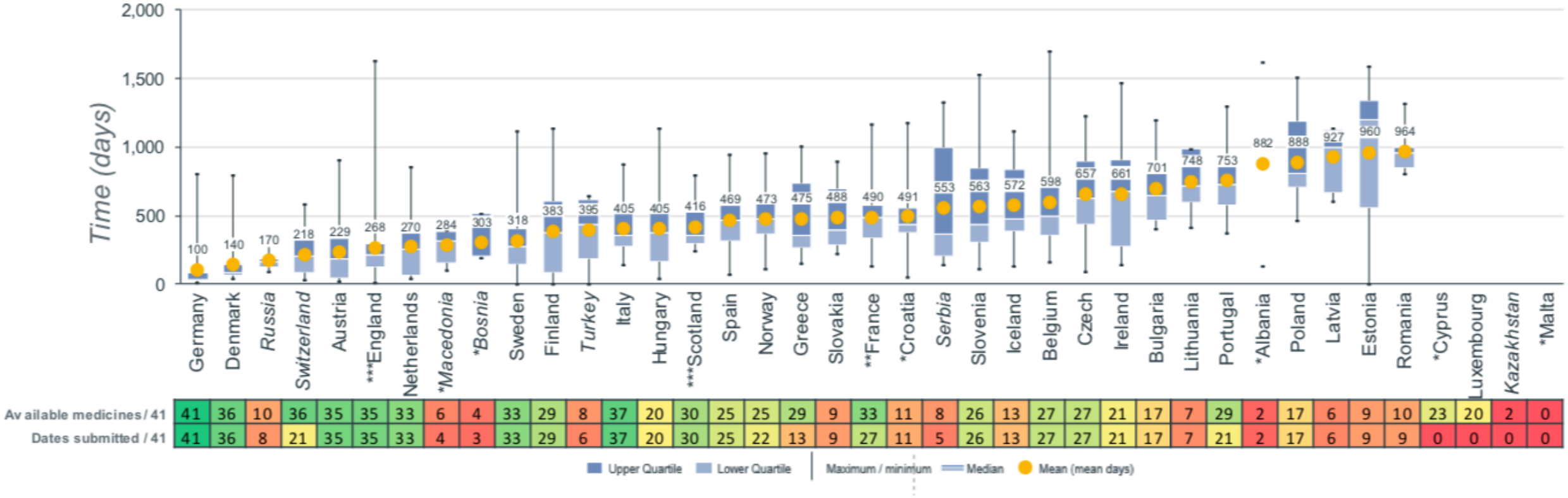
European Union average: 24 products available (59%) †In most countries availability equates to granting of access to the reimbursement list, except in DK, FI, LU, NO, SE some hospital products are not covered by the general reimbursement scheme. *Countries with asterisks did not complete a full dataset and therefore availability may be unrepresentative.

IQVIA

Source: EFPIA Patients W.A.I.T. Indicator 2021 Survey, July 2022

Oncology time to availability (2017-2020)

The **time to availability** is the days between marketing authorisation and the date of availability to patients in European countries (for most this is the point at which products gain access to the reimbursement list†). The marketing authorisation date is the date of central EU authorisation in most countries, except for countries shown in italics where local authorisation dates have been used. Data is correct to 1st January 2022.



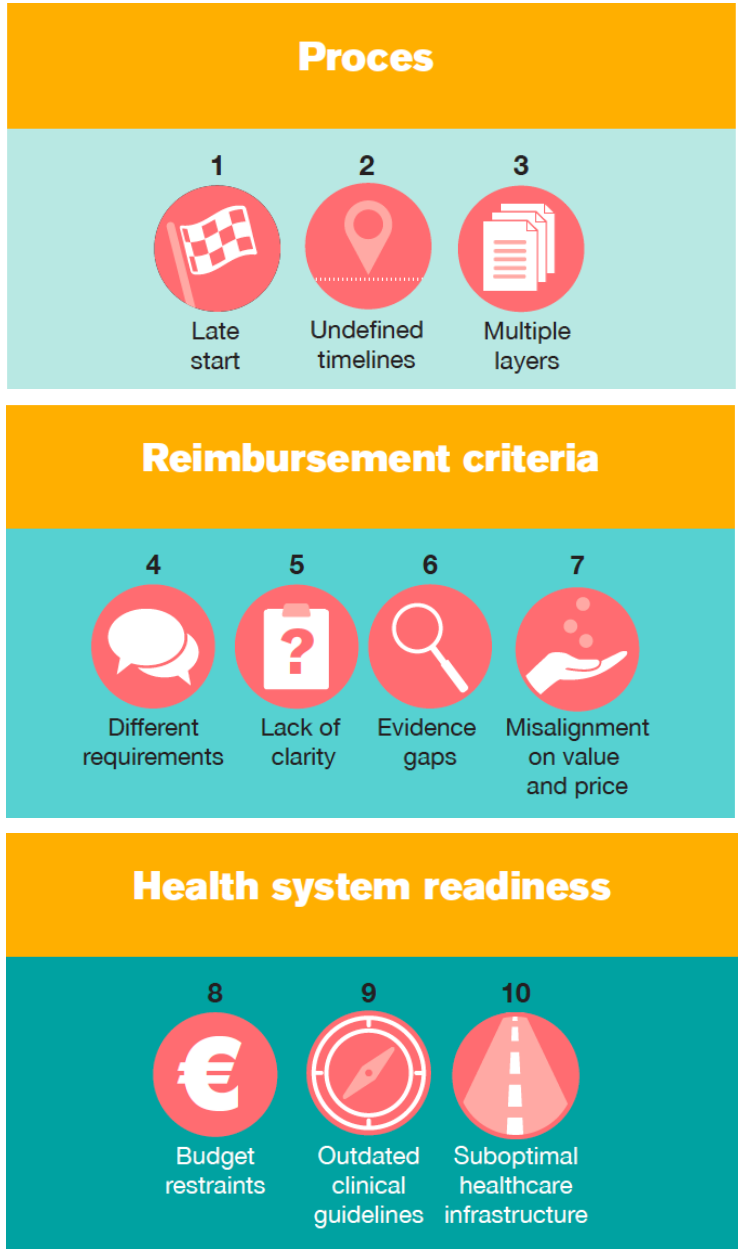
Source: EFPIA Patients W.A.I.T. Indicator 2021 Survey, July 2022

Observation

- Treatment costs should be covered by public health insurance by means of solidarity mechanism. "Affordability" should be a public issue, not a private one.
- Most healthcare systems are "sustainable", but they are badly organised and measured
  - eg – colorectal cancer screening : early detection leads to 90% survival at 1/10th of cost of fase III & IV treatment, but still most patients are detected in phase III or IV

Observation

- We identified 10 barriers to fast market access for oncology drugs in the European Union
- The report comes with 6 core recommendations for all stakeholders involved



”Every Day Counts: Improving Time To Patient Access To Innovative Oncology Therapies In Europe”, Ventura, July 2020

Patient Pathway Leadership

- Only patients know the full experience of the entire pathway in all its complexity
- Patient organisations can use the full extent of their **collective intelligence** to make patient pathways more effective and more efficient
- Patient organisations should be part of the decision-making process.
- Only patients can appreciate the full value of any treatment
- By 2030 patient organisations will lead full pathway design with all other stakeholders

To conclude

- We welcome the current dynamic environment of cancer drug development
- It is the fundamental right of patients to get access to any treatment that has a potential efficacy
- Cancer drugs create value – Focus on the value
- Despite the technological possibility, we have barely any real-time data on use, outcome and value of cancer treatments
- Early access programmes should not be an excuse to delay Pricing & Reimbursement
- Don’t abuse words like “Affordability” and “sustainability” for inaction
- Make patient organisations part of the decision-making process

Thank you!

Total Patient Support

- Being ill is much more than the clinical aspects of a disease
- Patient organisations make sure that patients receive **Total Patient Support**
- All patients should be directed to a **disease-specific patient organisation** after diagnosis
- Patient organisations can prepare patients for consultations, helping them to get the best possible care, asking the right questions and understanding all the consequences
- Patients who are fully supported have better treatment outcomes and less treatment regret
- All disease-specific patient organisations should receive **public funding** to allow for **professional added-value services** complementary to the clinical care in hospitals

Patient Pathway Leadership

- Only patients know the full experience of the entire pathway in all its complexity
- Patient organisations can use the full extent of their **collective intelligence** to make patient pathways more effective and more efficient
- Only patients can decide on PROMs and PREMs
- By 2030, patient organisations will assume full leadership of the entire patient pathway, offering significant added value to patients and society
- Patient organisations will lead full pathway design with all other stakeholders

Patient Pathway Leadership

- Our membership is our biggest asset
- They can provide the "collective intelligence" to what works well and what less so
  - Surveys
  - Qualitative interviews
  - Helpdesk monitoring
  - Patient queries
  - …
- We know our disease and all its ramifications

Collective

Quality of Care

Collective Intelligence & Outcomes Data

Systems improvement and health policy

Prevention

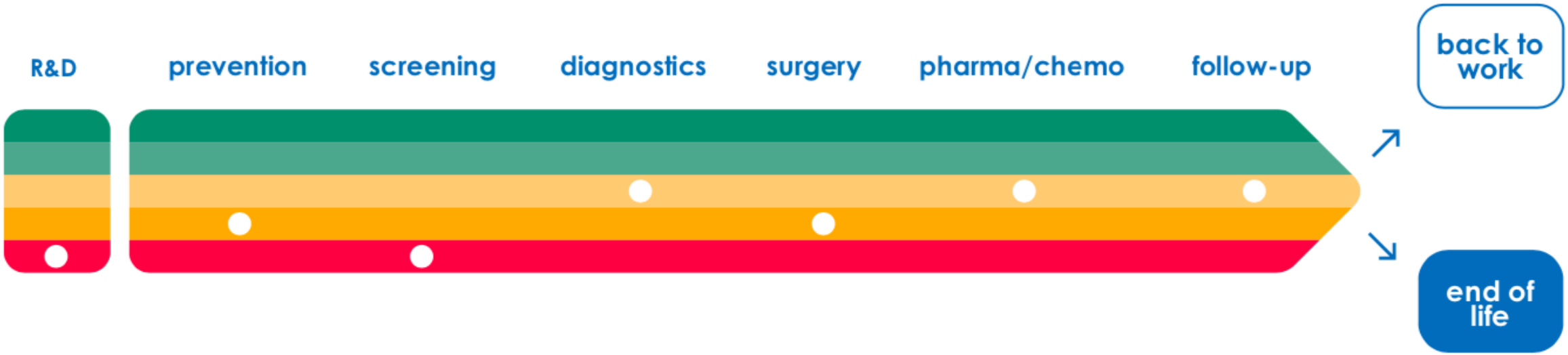
Access

Research and innovation

Patient Pathway Leadership

Value generation: making the patient journey more effective and efficient

Example of colorectal cancer



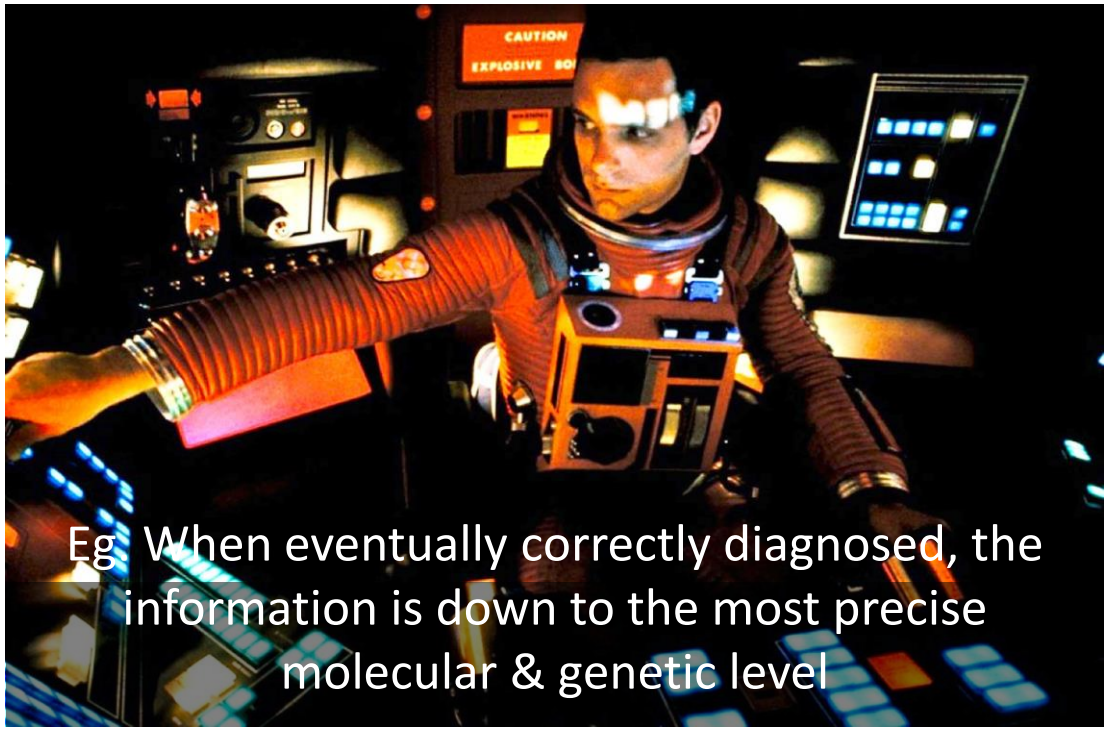
In every step of the way, significant improvements can be made, based on patient insights

Current best practice
Current worst practice
Current average

Source: Roadmap for the Prevention and Treatment of Colorectal Cancer in Europe, Digestive Cancers Europe, 2021

Observation #1
Prehistory & space age co-exist in healthcare

Eg. Family doctors don't ask about family history of cancer



Eg. When eventually correctly diagnosed, the information is down to the most precise molecular & genetic level

Observation #2
There is no health systems approach

Provides individual treatment

Seeks treatment

Approves and reimburses infrastructure & technology

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No systematic health policy for each disease/patient population

Observation #3
No Systematic Data Collection

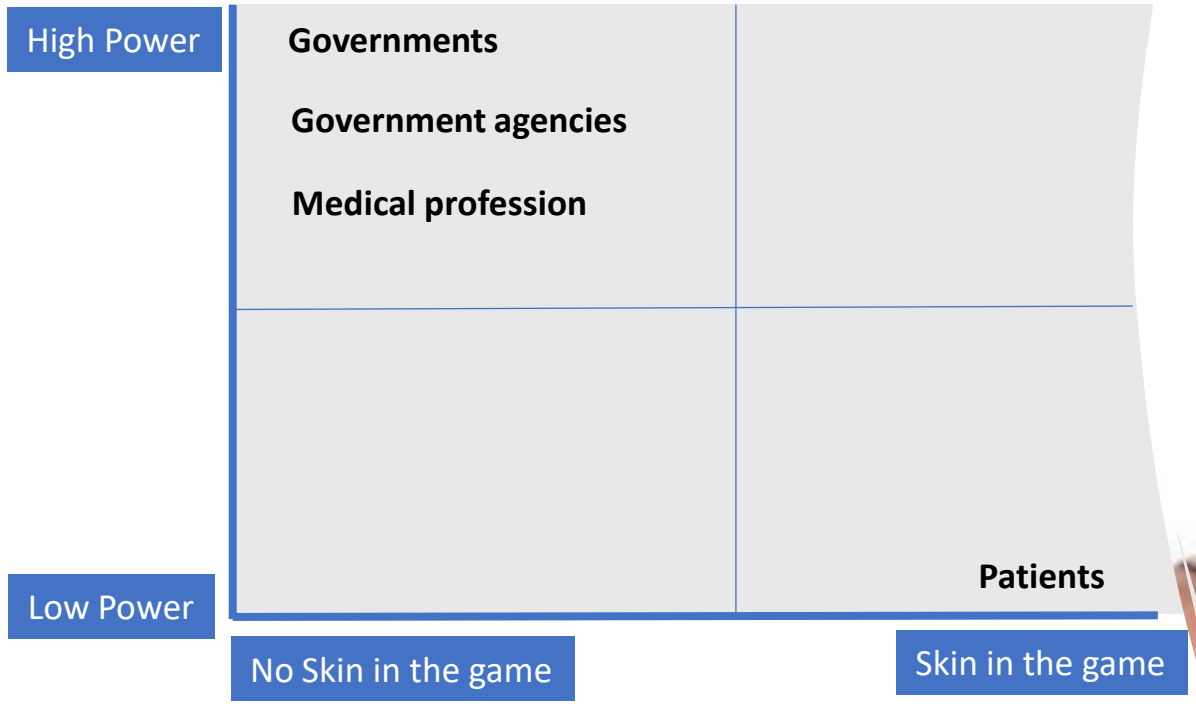
- Data collection and data sharing do not happen systematically and not in a transparent way
- For the majority of diseases, including cancer, recent data are totally absent
- The absence of granular data leads to an often emotional and ideological healthcare debate, leading us away from what an intelligent and rational systems approach should look like
- All stakeholders who work with tax-payers' money, should be fully transparent and accountable about the money invested and the results obtained

Observation #3
No Systematic Data Collection

"Show me what you measure ...

... and I will tell you what you care about"

Observation #4 – People with ‘skin in the game’ have nothing to say



Observation #5 – The higher the mortality, the lower the research spending

Mortality by type of (digestive) cancer

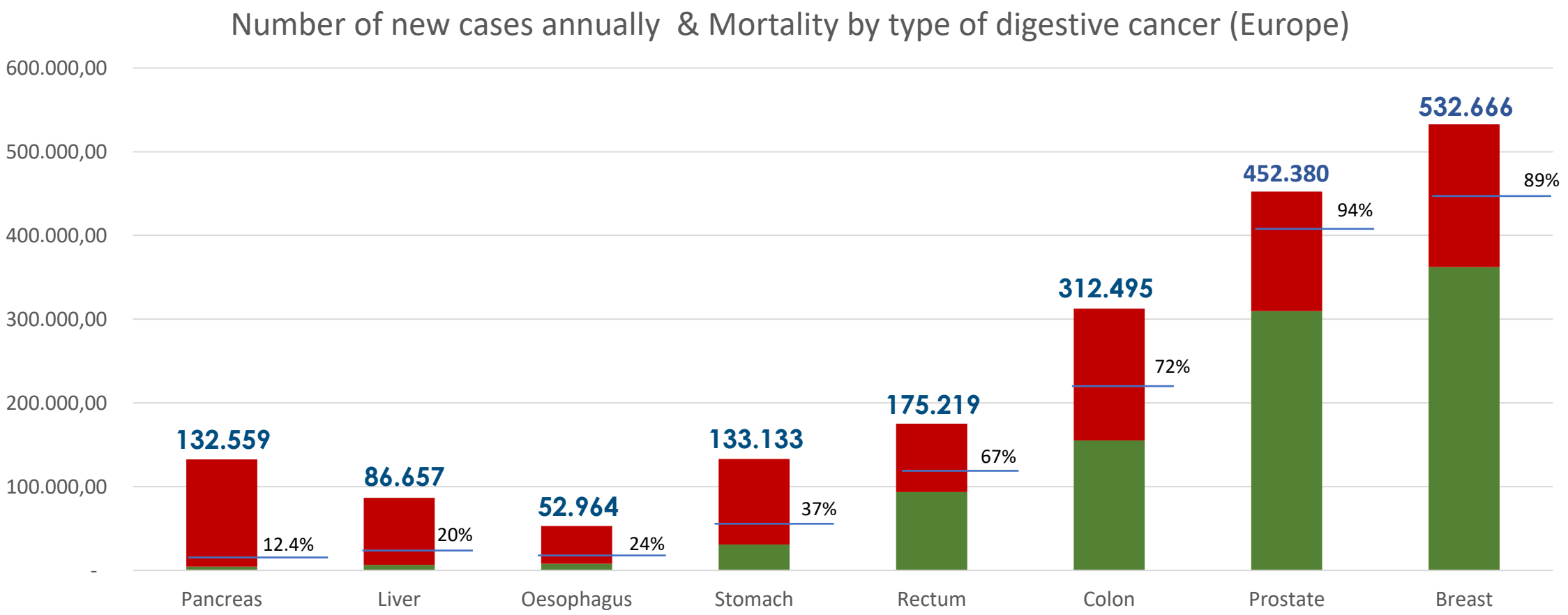


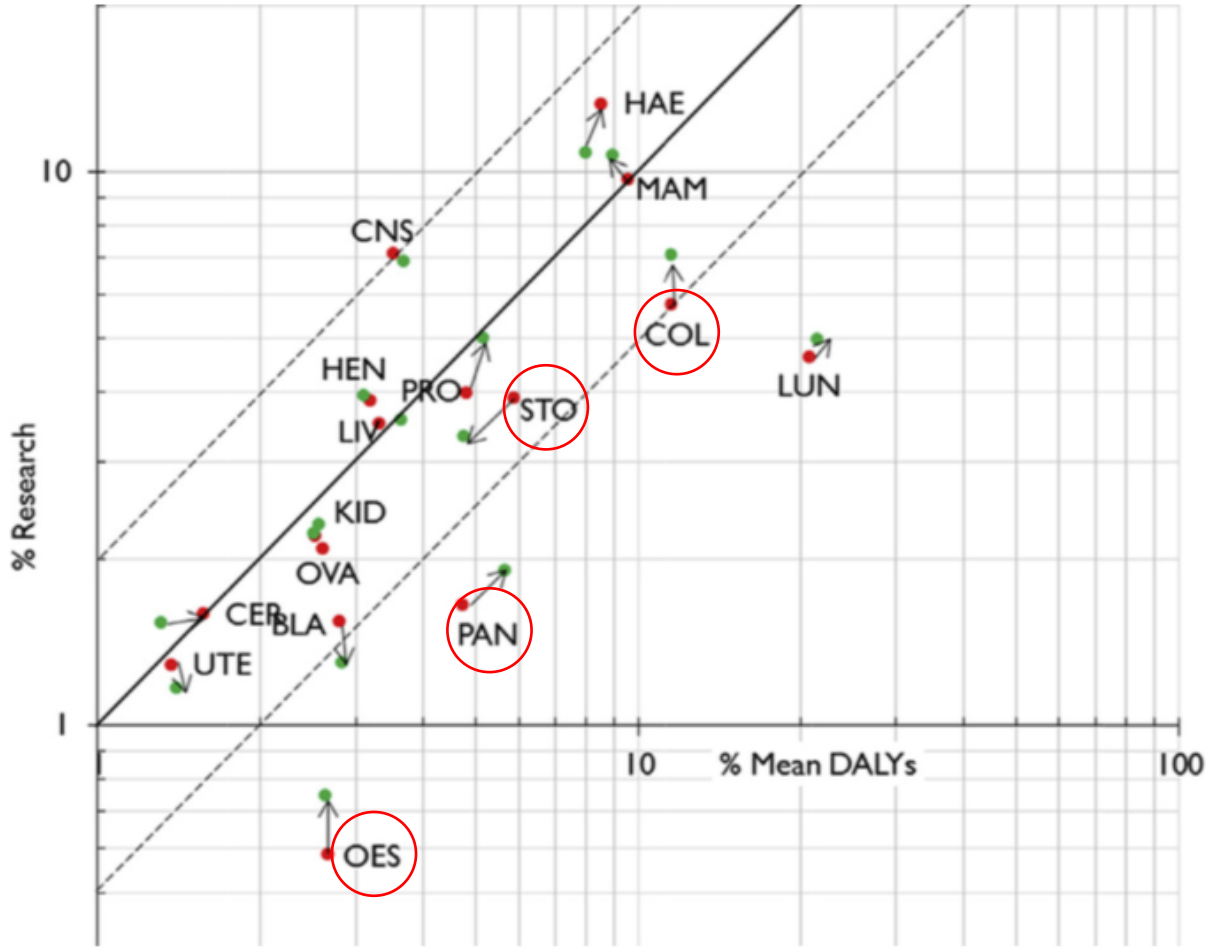
FIGURE Incidence by type of cancer (Source: European Cancer Information System, 2018)

Mortality as part of total number of new cases (Source: European Cancer Information System, 2018)

EU member state with best 5-year survival (Source: CONCORD Programme, The Lancet, 2018))

Cancer research in Europe vs Burden of Disease

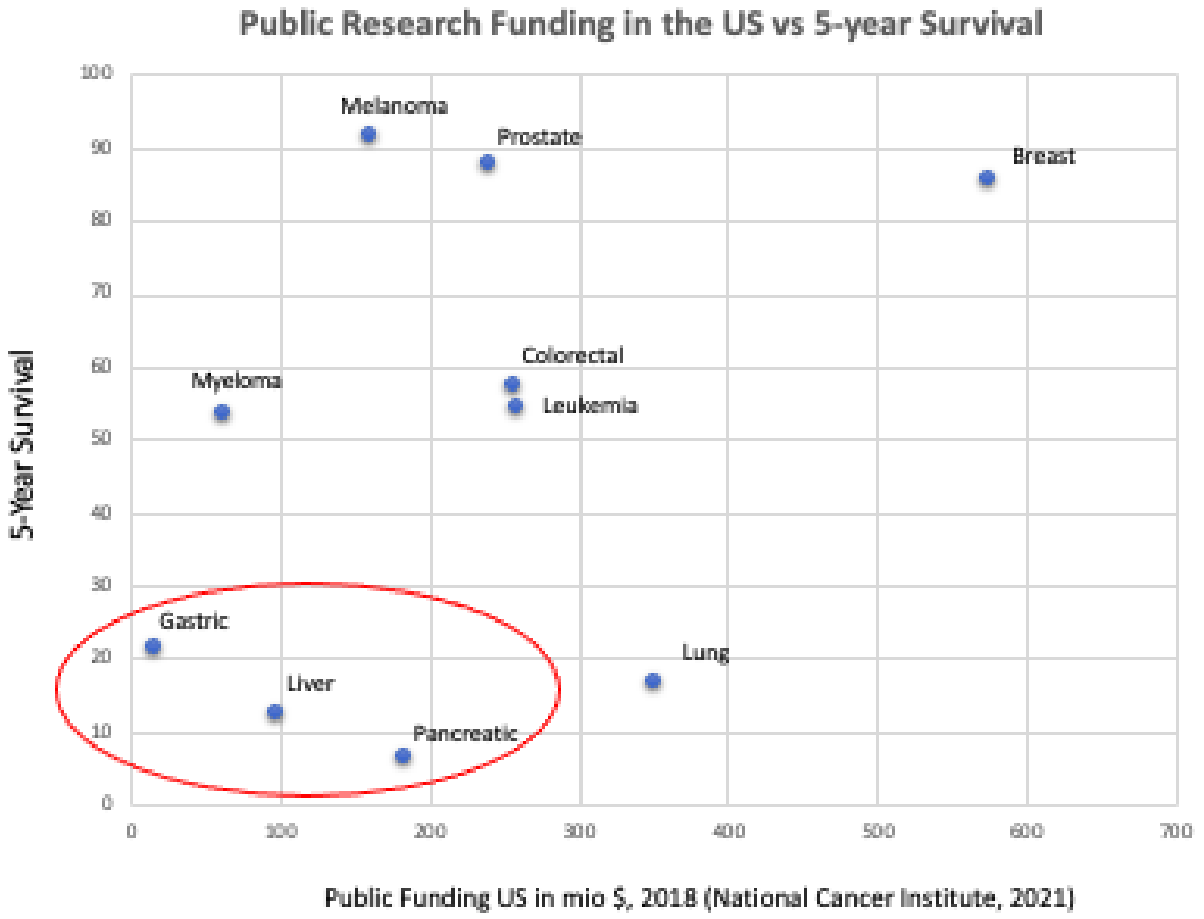
- Big discrepancy between burden of disease and research efforts (based on number of articles)
- Oesophageal, pancreatic, stomach and colorectal cancer are under-researched



"Mapping the European cancer research landscape: An evidence base for national and Pan-European research and funding, European Journal of Cancer, September 2018"

Cancer research in Europe vs Burden of Disease

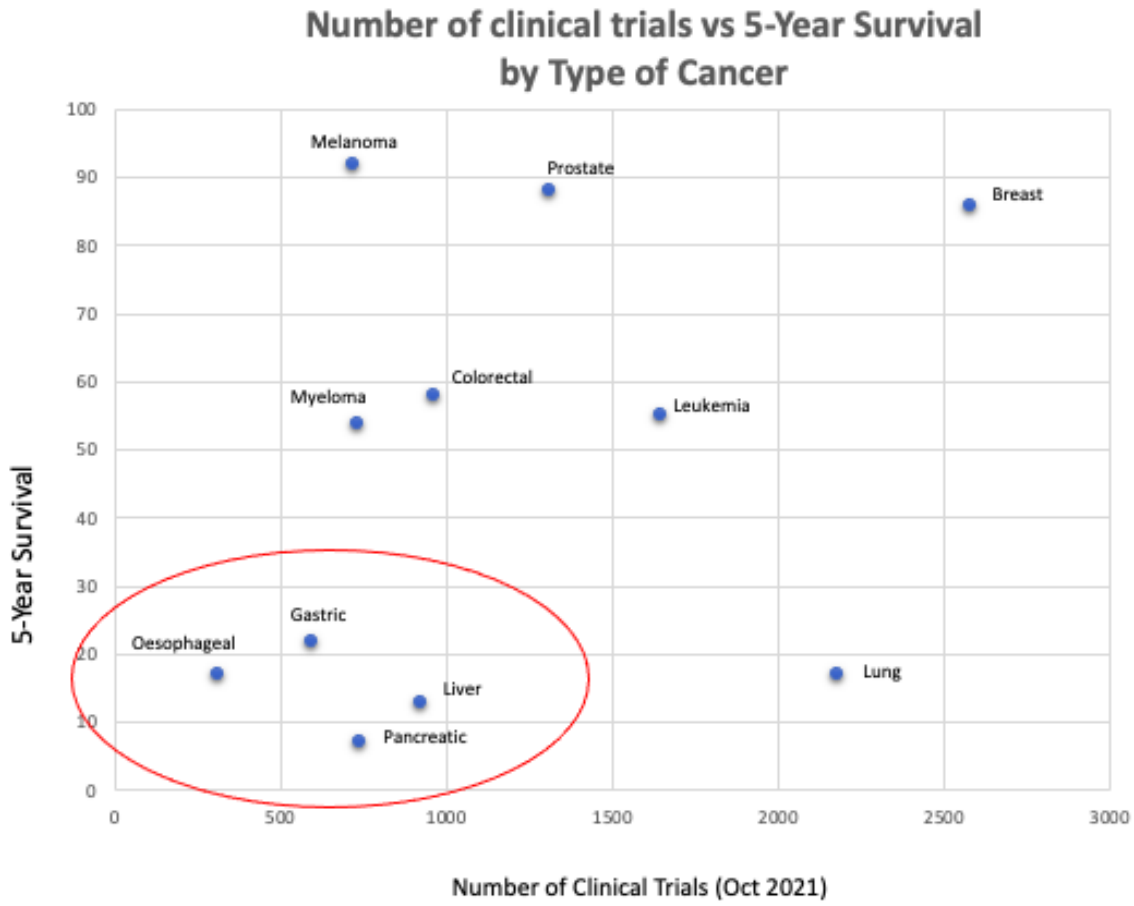
- Research goes where results are already achieved
- European public research statistics by type of cancer are non-existent



Sources: Clinicaltrials.gov (2021) and Cancer Research UK (2021)

Cancer research in Europe vs Burden of Disease

- Research goes where results are achieved
- The higher the mortality, the higher the risk, the lower the research efforts



Sources: Clinicaltrials.gov (2021) and Cancer Research UK (2021)