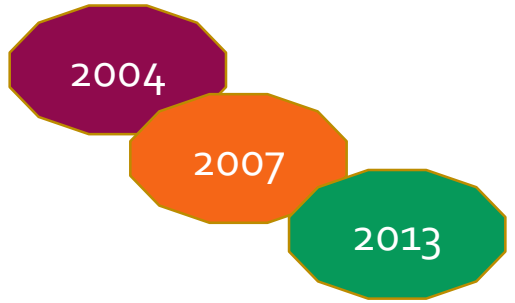
IMPORTANCE OF DATA EVIDENCE FOR PATIENT VALUE AND HEALTH OUTCOMES BASED APPROACH

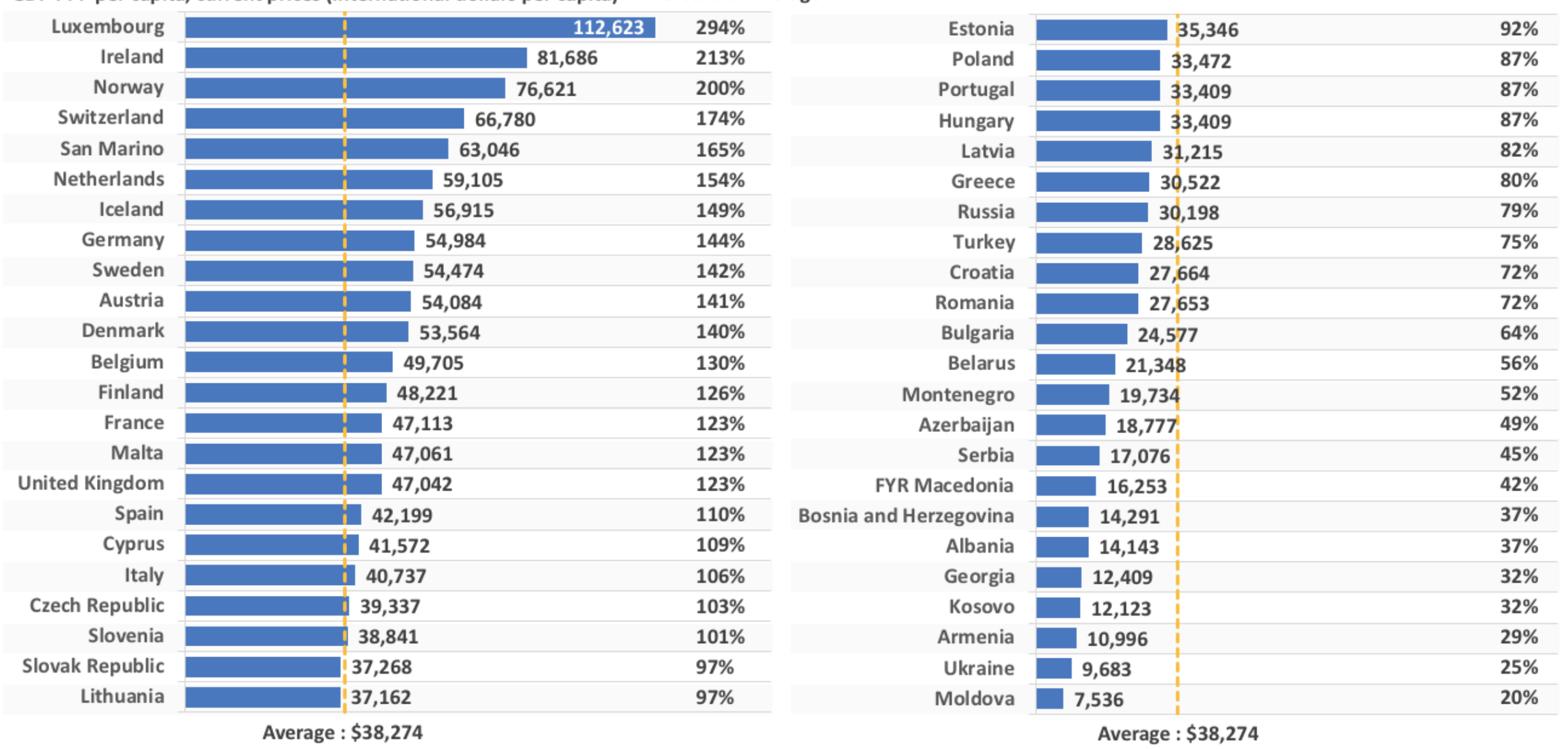
KEY HEALTH SYSTEM DATA AND CHALLENGES IN CEE COUNTRIES



- Shorter life expectancy and worst health indicators compare to EU15
- Health is not a priority for governments
- Low healthcare financing
- Low access to modern treatments
- Health inequalities
- Unhealthy life style and low health education

EUROPE GDP PPP PER CAPITA – 2019 FORECAST

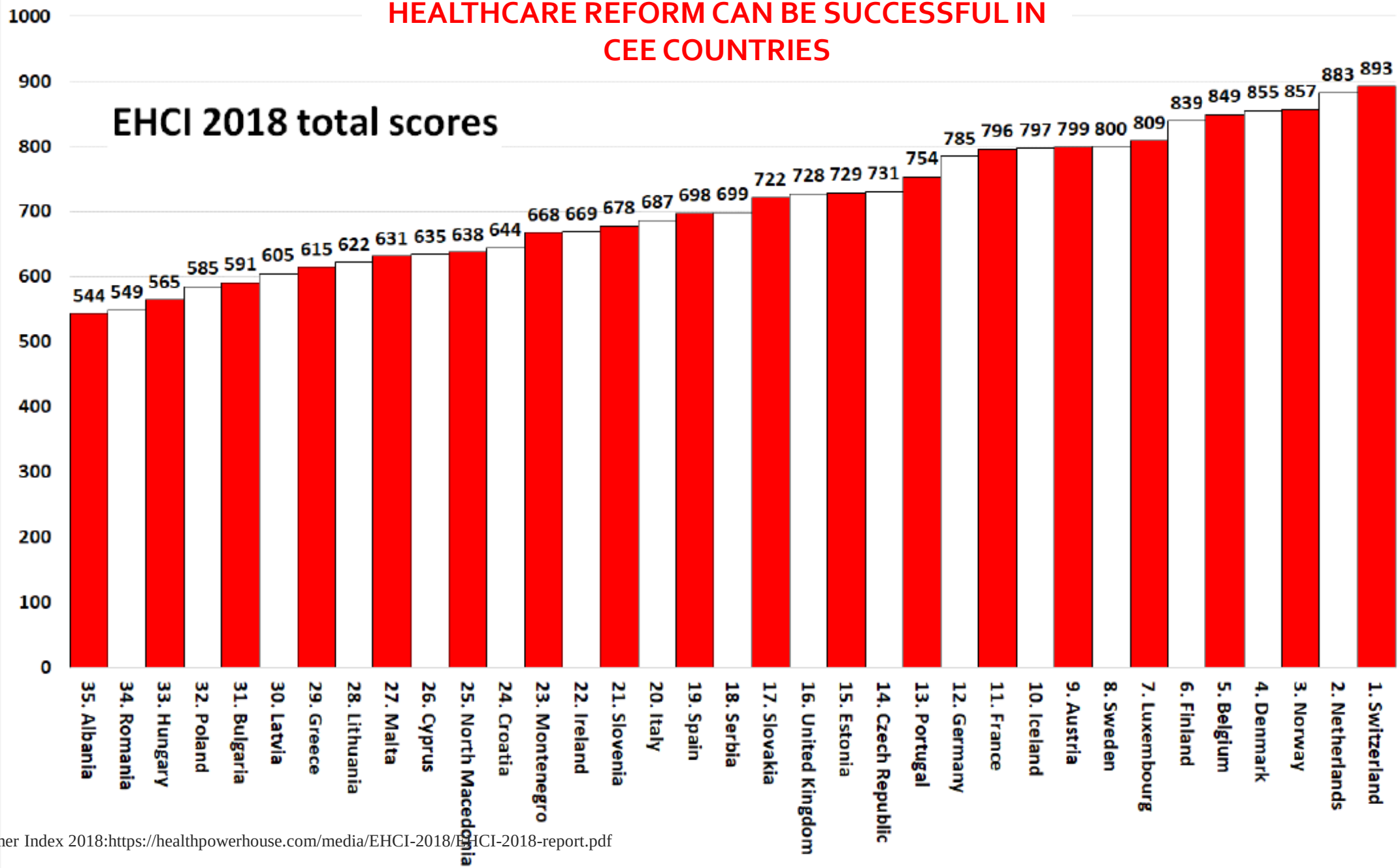
NEED FOR SMART INVESTMENT IN CEE HEALTHCARE



Data Source: IMF World Economic Outlook, October 2018

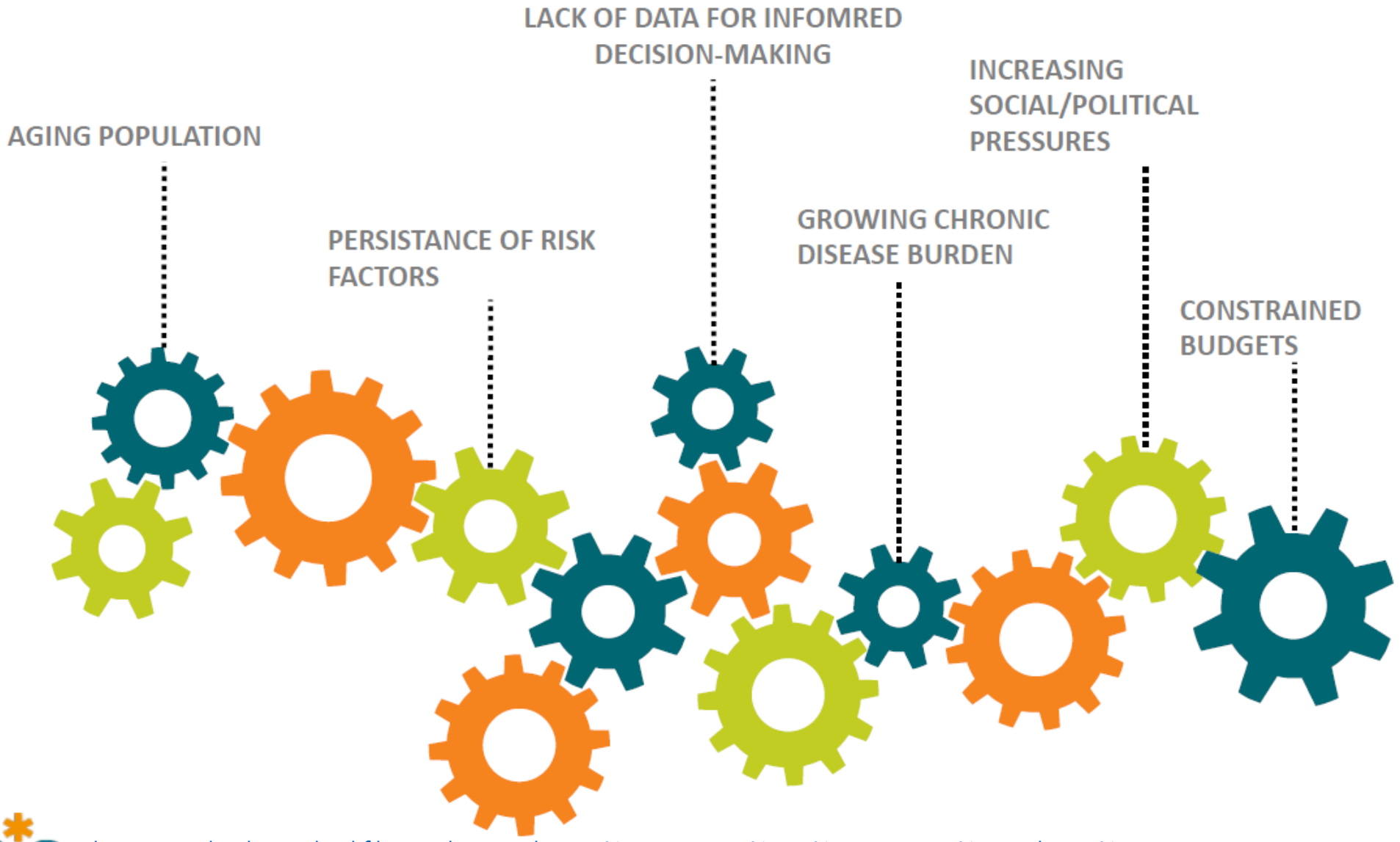
Data Analysis by: MGM Research

CZECH REPUBLIC AND SLOVAKIA SHOW THAT HEALTHCARE REFORM CAN BE SUCCESSFUL IN CEE COUNTRIES



Euro Health Consumer Index 2018:https://healthpowerhouse.com/media/EHCI-2018/EHCI-2018-report.pdf

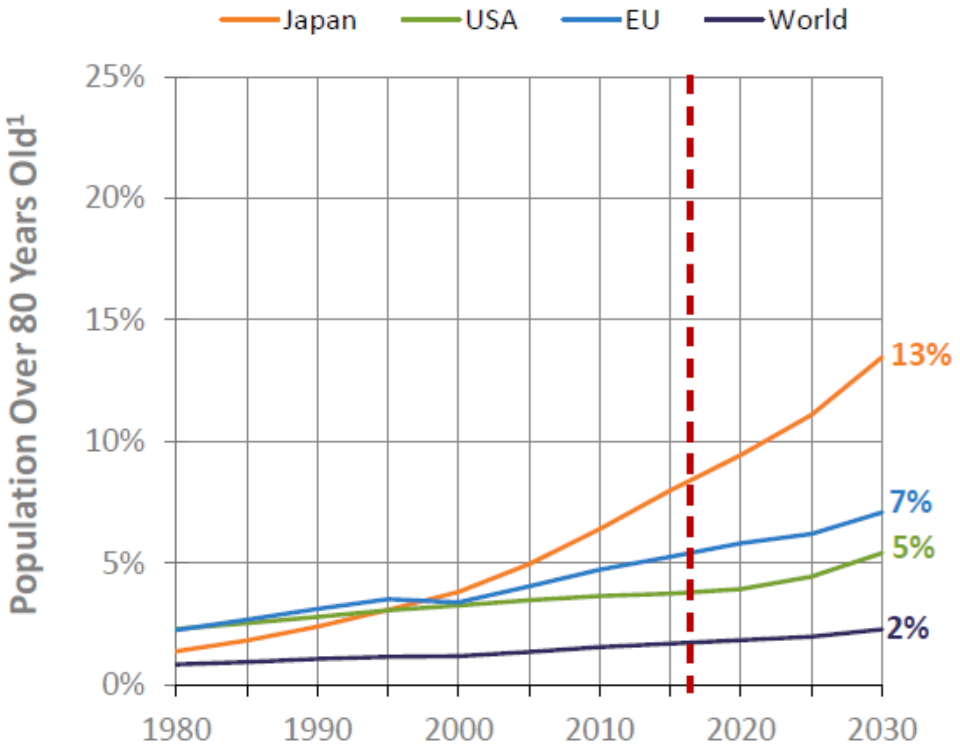
Healthcare systems face significant challenges in
Expanding access to healthcare while managing constrained budgets

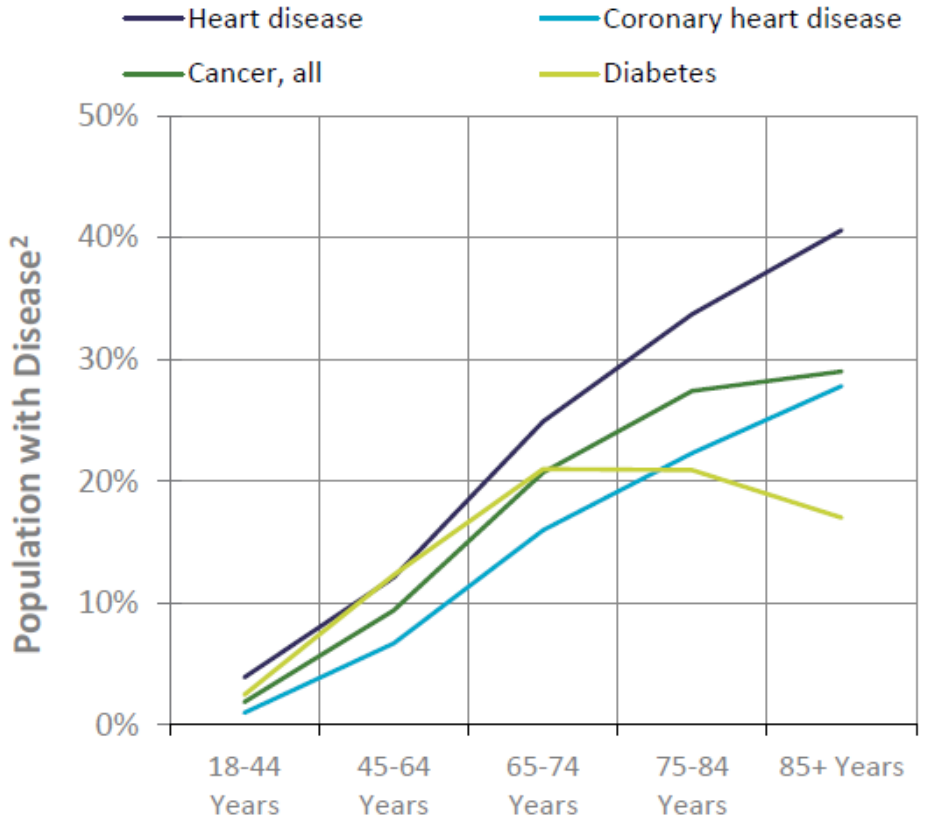


http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

An aging population and increasing prevalence of chronic disease
Are burdening healthcare systems

Ageing populations worldwide are leading to more chronic disease and greater demand for care





http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

Source: Health Advances analysis; [1]OECD Health Statistics Database (accessed February 2016), [2]CDD Health Interactive Data from NHIS , UK (accessed February 2016).

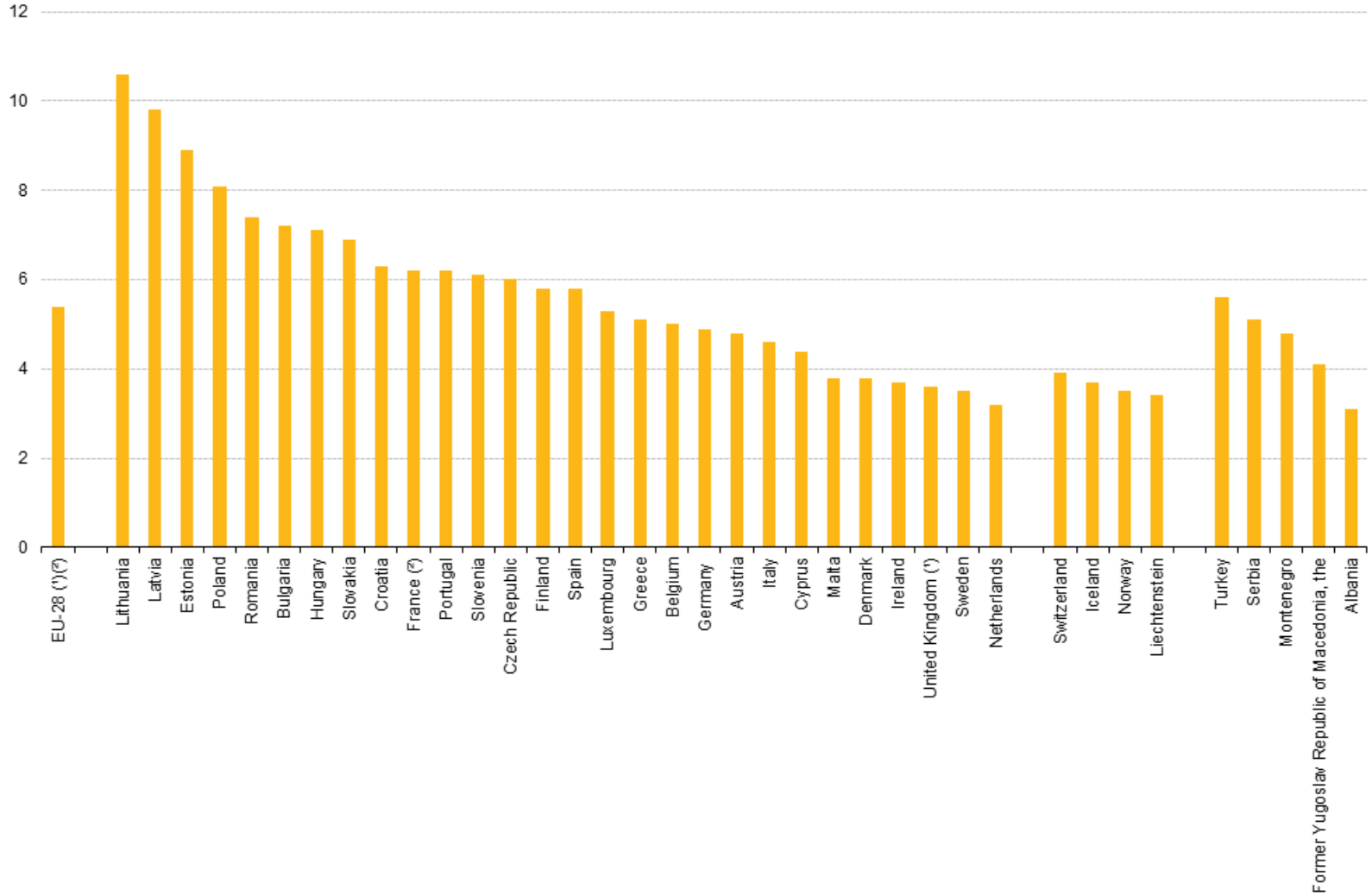
Life expectancy at birth, 1980-2016

(years)

	Total							Males							Females						
	1980	1990	2000	2010	2014	2015	2016	1980	1990	2000	2010	2014	2015	2016	1980	1990	2000	2010	2014	2015	2016
EU-28 (1)	:	:	:	79.9	*80.9*	*80.6*	*81.0*	:	:	:	76.9	*78.1*	*77.9*	*78.2*	:	:	:	82.8	*83.6*	*83.3*	*83.6*
Belgium	73.3	76.2	77.9	80.3		81.1	81.5	69.9	72.7	74.6	77.5	78.8	78.7	79.0	76.7	79.5	81.0	83.0	83.9	83.4	84.0
Bulgaria	71.1	71.2	71.6	73.8	74.5	74.7	74.9	68.4	68.0	68.4	70.3	71.1	71.2	71.3	73.9	74.7	75.0	77.4	78.0	78.2	78.5
Czech Republic	70.4	71.5	75.1	77.7	78.9	78.7	79.1	66.9	67.6	71.6	74.5	75.8	75.7	76.1	74.0	75.5	78.5	80.9	82.0	81.6	82.1
Denmark	74.2	74.9	76.9	79.3	80.7	80.8	80.9	71.2	72.0	74.5	77.2	78.7	78.8	79.0	77.3	77.8	79.2	81.4	82.8	82.7	82.8
Germany	73.1	75.4	78.3	80.5	81.2	80.7	81.0	69.6	72.0	75.1	78.0	78.7	78.3	78.6	76.2	78.5	81.2	83.0	83.6	83.1	83.5
Estonia (2)	69.5	69.9	71.1	76.0	77.4	78.0	78.0	64.2	64.7	65.6	70.9	72.4	73.2	73.3	74.3	74.9	76.4	80.8	81.9	82.2	82.2
Ireland	:	74.8	76.6	80.8	81.4	81.5	81.8	:	72.1	74.0	78.5	79.3	79.6	79.9	:	77.7	79.2	83.1	83.5	83.4	83.6
Greece	75.3	77.1	78.6	80.6	81.5	81.1	81.5	73.0	74.7	75.9	78.0	78.8	78.5	78.9	77.5	79.5	81.3	83.3	84.1	83.7	84.0
Spain	75.5	76.9	79.3	82.4	83.3	83.0	83.5	72.3	73.3	75.8	79.2	80.4	80.1	80.5	78.5	80.6	82.8	85.5	86.2	85.7	86.3
France (3)	:	:	79.2	81.8	82.8	*82.4*	*82.7*	:	:	75.3	78.2	79.5	*79.2*	*79.5*	:	:	83.0	85.3	86.0	*85.6*	*85.7*
Croatia	:	:	:	76.7	77.9	77.5	78.2	:	:	:	73.4	74.7	74.4	75.0	:	:	:	79.9	81.0	80.5	81.3
Italy	:	77.1	79.9	82.2	83.2	82.7	83.4	:	73.8	76.9	79.5	80.7	80.3	81.0	:	80.3	82.8	84.7	85.6	84.9	85.6
Cyprus	:	:	77.7	81.5	82.3	81.8	82.7	:	:	75.4	79.2	80.3	79.9	80.5	:	:	80.1	83.9	84.3	83.7	84.9
Latvia	:	:	:	73.1	74.5	74.8	74.9	:	:	:	67.9	69.1	69.7	69.8	:	:	:	78.0	79.4	79.5	79.6
Lithuania	70.5	71.5	72.1	73.3	74.7	74.6	74.9	65.4	66.4	66.7	67.6	69.2	69.2	69.5	75.4	76.3	77.4	78.9	80.1	79.7	80.1
Luxembourg	72.8	75.7	78.0	80.8	82.3	82.4	82.7	70.0	72.4	74.6	77.9	79.4	80.0	80.1	75.6	78.7	81.3	83.5	85.2	84.7	85.4
Hungary	69.1	69.4	71.9	74.7	76.0	75.7	76.2	65.5	65.2	67.5	70.7	72.3	72.3	72.6	72.8	73.8	76.2	78.6	79.4	79.0	79.7
Malta	70.4	:	78.5	81.5	82.1	82.0	82.6	68.0	:	76.3	79.3	79.9	79.8	80.6	72.8	:	80.5	83.6	84.3	84.1	84.4
Netherlands	:	77.1	78.2	81.0	81.8	81.6	81.7	:	73.8	75.6	78.9	80.0	79.9	80.0	:	80.2	80.7	83.0	83.5	83.2	83.2
Austria	72.7	75.8	78.3	80.7	81.6	81.3	81.8	69.0	72.3	75.2	77.8	79.1	78.8	79.3	76.1	79.0	81.2	83.5	84.0	83.7	84.1
Poland (4)	:	70.7	73.8	76.4	77.8	77.5	78.0	:	66.3	69.6	72.2	73.7	73.5	73.9	:	75.3	78.0	80.7	81.7	81.6	82.0
Portugal	71.5	74.1	76.8	80.1	81.3	81.3	81.3	67.9	70.6	73.3	76.8	78.0	78.1	78.1	74.9	77.5	80.4	83.2	84.4	84.3	84.3
Romania (5)	69.2	69.9	71.2	73.7	*75.0*	*75.0*	75.3	66.6	66.7	67.7	70.0	*71.3*	*71.5*	71.7	71.9	73.1	74.8	77.7	*78.7*	*78.7*	79.1
Slovenia	:	73.9	76.2	79.8	81.2	80.9	81.2	:	69.8	72.2	76.4	78.2	77.8	78.2	:	77.8	79.9	83.1	84.1	83.9	84.3
Slovakia	70.4	71.1	73.3	75.6	77.0	76.7	77.3	66.7	66.7	69.2	71.8	73.3	73.1	73.8	74.4	75.7	77.5	79.3	80.5	80.2	80.7
Finland	73.7	75.1	77.8	80.2	81.3	81.6	81.5	69.2	71.0	74.2	76.9	78.4	78.7	78.6	78.0	79.0	81.2	83.5	84.1	84.4	84.4
Sweden	75.8	77.7	79.8	81.6	82.3	82.2	82.4	72.8	74.8	77.4	79.6	80.4	80.4	80.6	79.0	80.5	82.0	83.6	84.2	84.1	84.1
United Kingdom (6)	:	:	78.0	80.6	*81.4*	*81.0*	*81.2*	:	:	75.5	78.6	*79.5*	*79.2*	*79.4*	:	:	80.3	82.6	*83.2*	*82.8*	*83.0*
Iceland	76.8	78.1	79.7	81.9	82.9	82.5	82.2	73.5	75.5	77.8	79.8	81.3	81.2	80.4	80.4	80.7	81.6	84.1	84.5	83.8	84.1
Liechtenstein	:	:	77.0	81.8	82.1	82.7	82.3	:	:	73.9	79.5	81.0	80.9	80.6	:	:	79.9	84.3	83.2	84.5	84.0
Norway	75.8	76.6	78.8	81.2	82.2	82.4	82.5	72.4	73.4	76.0	79.0	80.1	80.5	80.7	79.3	79.9	81.5	83.3	84.2	84.2	84.2
Switzerland	75.7	77.5	80.0	82.7	83.3	83.0	83.7	72.3	74.0	77.0	80.3	81.1	80.8	81.7	79.0	80.9	82.8	84.9	85.4	85.1	85.6
Montenegro	:	:	:	76.1	76.5	76.5	76.5	:	:	:	73.6	74.1	74.4	74.1	:	:	:	78.5	78.9	78.6	78.9
Former Yugoslav Republic of Macedonia, the	:	:	73.0	75.0	75.5	75.5	75.4	:	:	70.8	72.9	73.5	73.5	73.4	:	:	75.2	77.2	77.5	77.4	77.5
Albania (7)	:	:	:	:	*78.2*	*77.9*	78.5	:	:	:	:	*76.3*	*76.2*	77.0	:	:	:	:	*80.2*	*79.7*	80.1
Serbia	:	:	71.6	74.4	75.4	75.3	75.7	:	:	68.9	71.8	72.8	72.8	73.2	:	:	74.4	77.0	78.0	77.9	78.3
Turkey	:	:	:	76.8	78.1	78.2	78.1	:	:	:	74.2	75.4	75.4	75.4	:	:	:	79.4	80.9	81.0	81.0

(1) 2010 and 2015: break in series. 2014: estimate. 2015 and 2016: estimate and provisional.
(2) 2015: break in series.
(3) 2015: break in series and provisional. 2016 : provisional
(4) 2000 and 2010: break in series
(5) 2014 and 2015: estimate
(6) 2014, 2015 and 2016: estimate
(7) 2014 and 2015: estimate
Source: Eurostat (online data code: demo_mlexpec)

Life expectancy at birth, gender gap, 2016
(years, female life expectancy - male life expectancy)



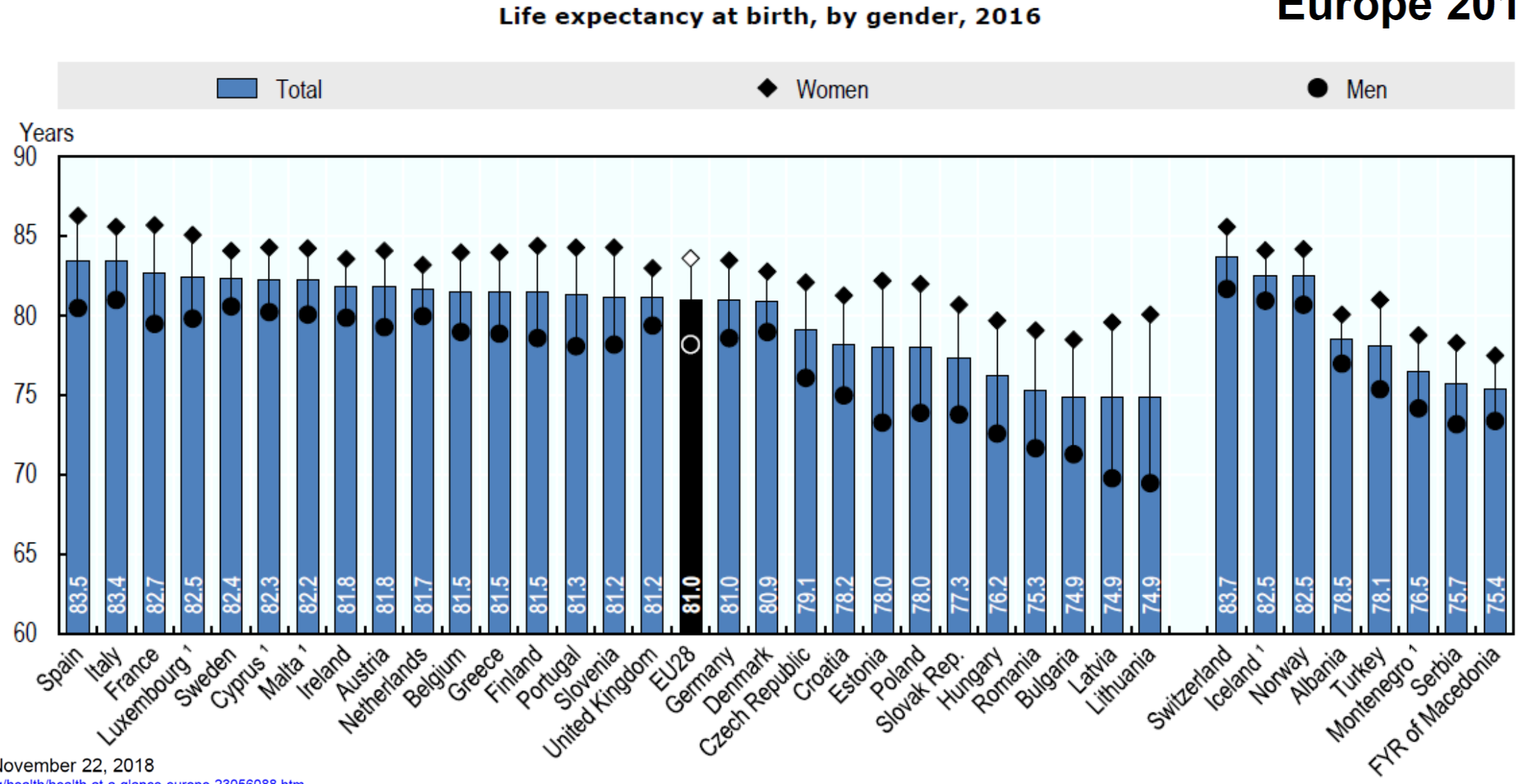
(1) Estimate.

(2) Provisional.
Source: Eurostat (online data code: demo_mlexpec)

eurostat

Life expectancy exceeds 81 years in a majority of EU countries, but the gap between the highest and lowest countries is still over 8 years

Health at a Glance: Europe 2018

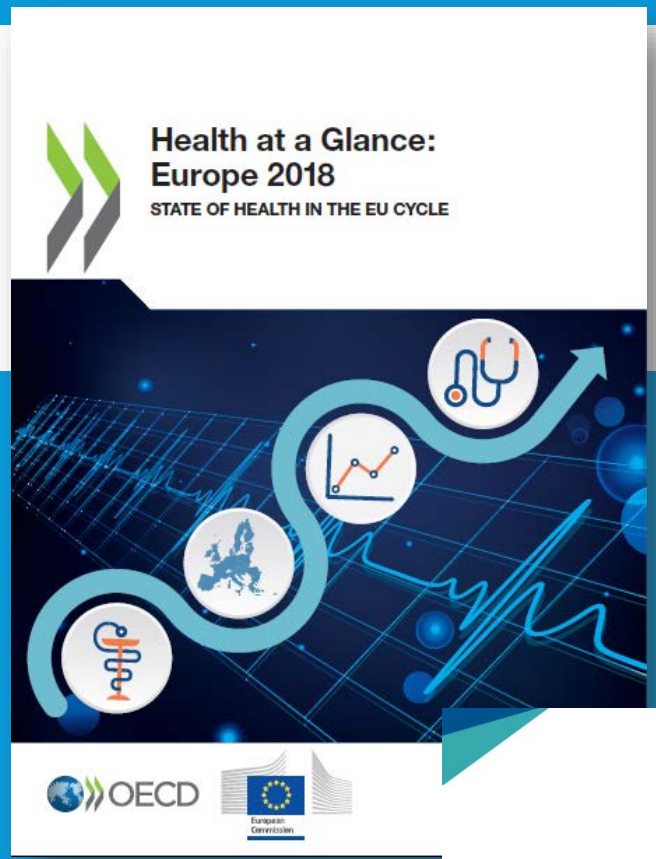


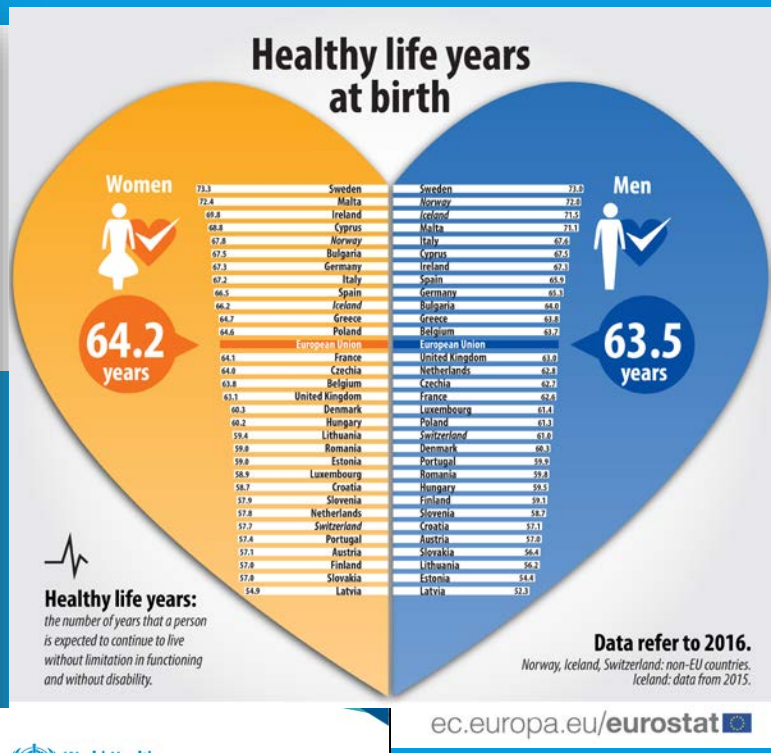
Released on November 22, 2018

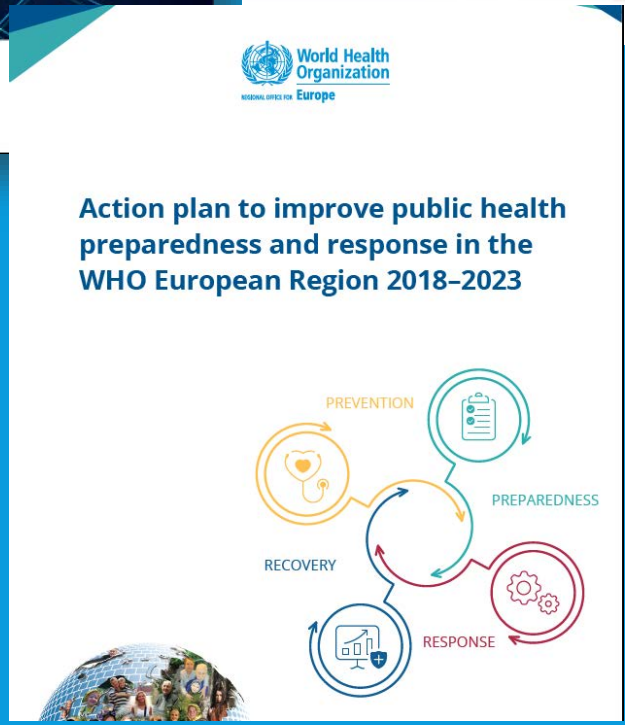
http://www.oecd.org/health/health-at-a-glance-europe-23056088.htm

RELIABLE DATA

- OECD
  http://www.oecd.org/health/
- EUROSTAT:
  https://ec.europa.eu/eurostat/statistics-explained/index.php/Health_in_the_European_Union_%E2%80%93_facts_and_figures
- WHO Europe
  http://www.euro.who.int/en/home

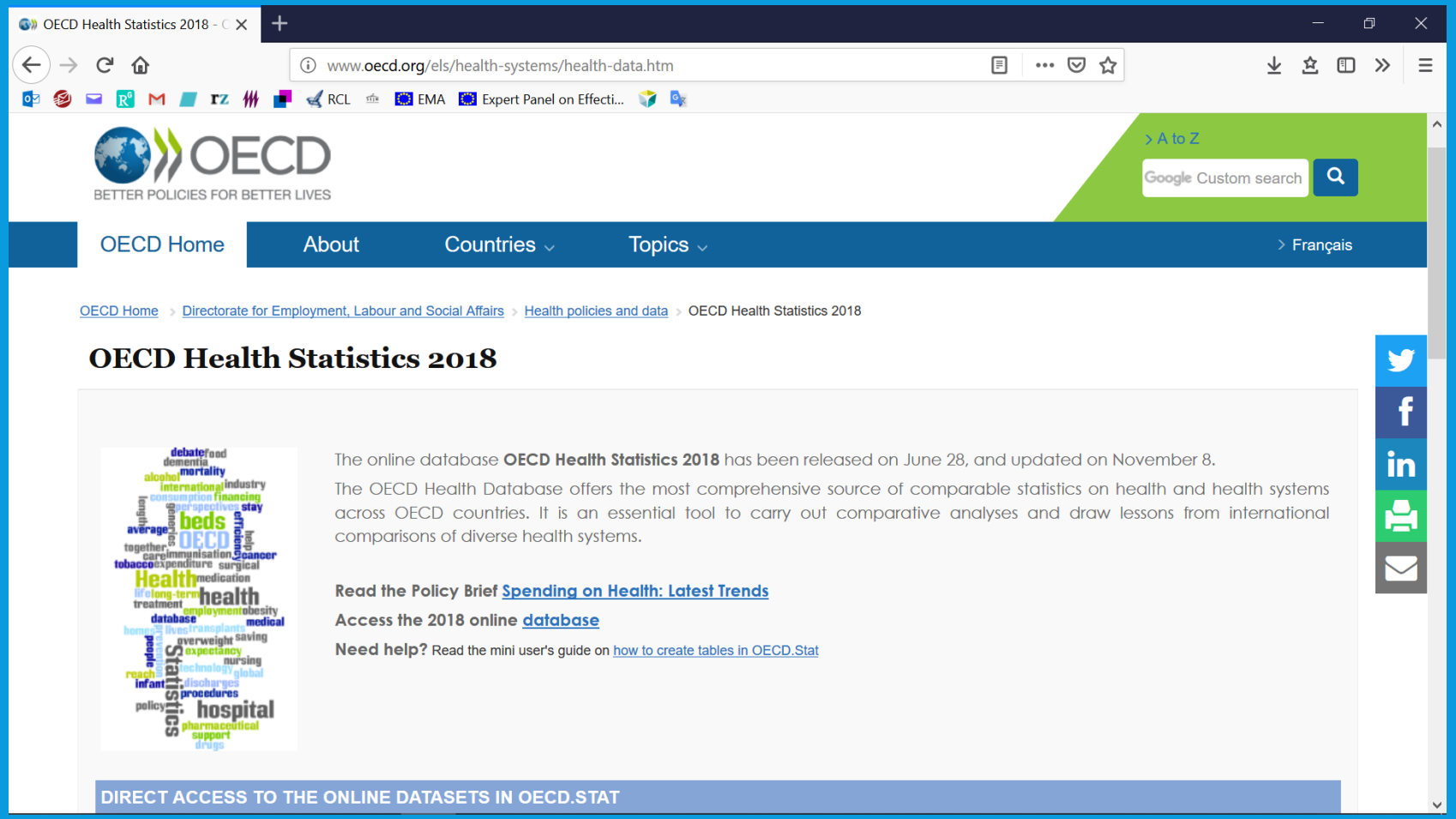






RELIABLE DATA

http://www.oecd.org/els/health-systems/health-data.htm

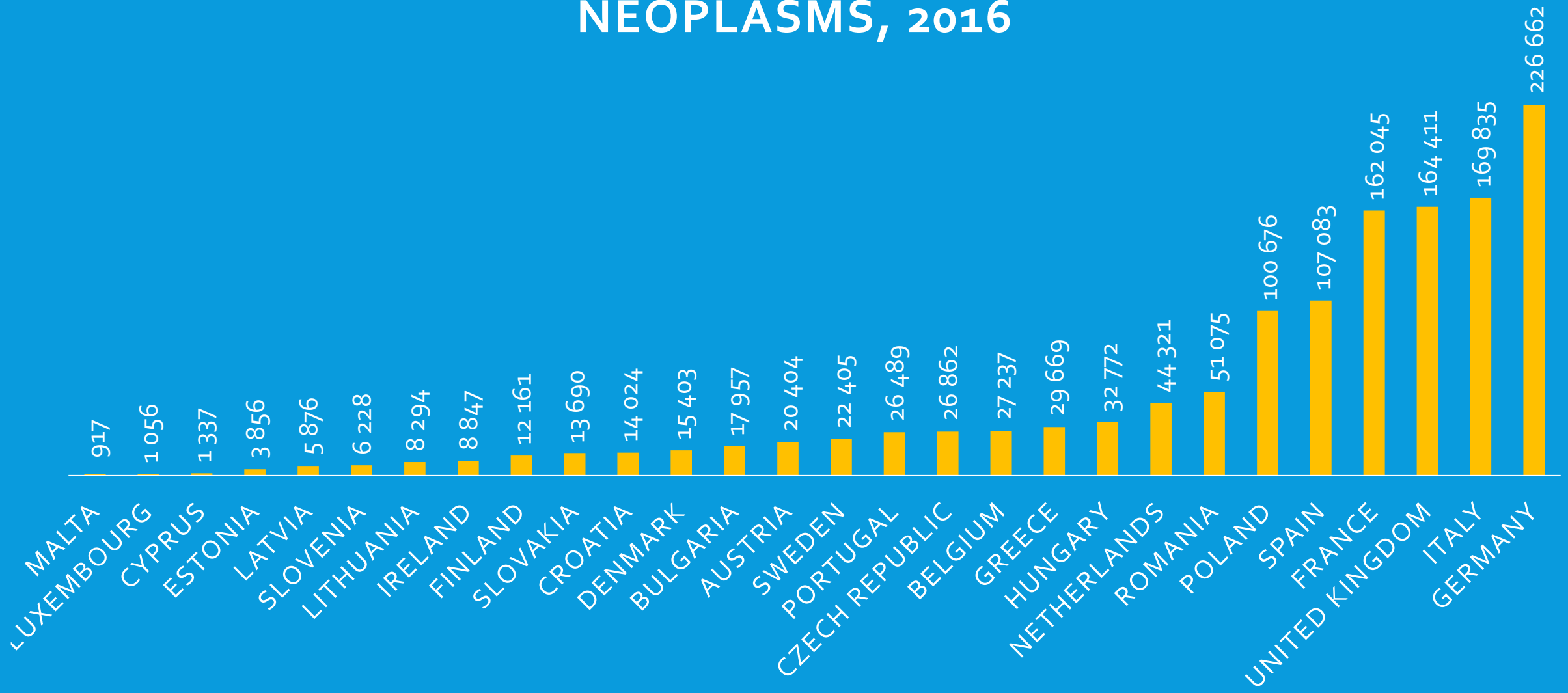


Causes of death — malignant neoplasms, residents, 2015

	Number of deaths	Share of all deaths			Standardised death rates				
		Total	Males	Females	Total	Males	Females	Persons aged < 65 years	Persons aged 65 and over
	(number)	(%)			(per 100 000 inhabitants)				
EU-28	1 321 592	25.4	28.7	22.1	260.6	346.2	200.8	77.9	1 014.8
Belgium	27 237	24.8	28.5	21.3	249.7	329.3	194.4	72.1	983.0
Bulgaria	17 957	16.4	18.4	14.3	241.9	330.4	179.3	102.4	817.8
Czech Republic	26 862	24.2	26.6	21.8	278.6	373.3	216.3	79.0	1 102.7
Denmark	15 403	29.4	31.1	27.8	292.3	355.3	250.1	72.3	1 200.6
Germany	226 662	24.4	27.3	21.7	252.1	324.1	202.4	72.4	993.6
Estonia	3 856	25.1	28.4	22.1	299.1	472.6	213.1	90.4	1 160.5
Ireland	8 847	29.4	31.0	27.7	275.2	334.2	232.5	66.0	1 138.8
Greece	29 669	24.6	29.5	19.5	250.0	345.1	174.1	74.0	976.4
Spain	107 083	25.4	30.8	20.0	230.6	332.3	155.5	70.6	891.2
France	162 045	27.3	31.4	23.3	245.3	337.1	179.4	78.9	932.1
Croatia	14 024	25.8	30.4	21.5	335.7	481.1	243.4	106.5	1 281.8
Italy	169 835	26.4	31.0	22.3	244.6	325.8	188.1	64.4	988.7
Cyprus	1 337	22.7	25.1	20.1	206.7	266.2	159.5	54.3	836.0
Latvia	5 876	20.8	23.1	18.8	293.7	449.7	219.6	103.9	1 077.5
Lithuania	8 294	20.1	22.9	17.4	285.5	449.2	198.9	104.6	1 032.5
Luxembourg	1 056	26.8	28.4	25.1	239.6	284.0	202.4	57.4	992.1
Hungary	32 772	24.9	27.8	22.2	345.9	471.8	267.3	137.3	1 207.2
Malta	917	26.6	29.0	24.2	226.2	286.9	183.5	65.0	892.1
Netherlands	44 321	30.3	34.1	26.8	284.0	357.4	234.4	75.1	1 146.4
Austria	20 404	24.9	28.0	22.0	243.4	315.0	195.0	68.1	967.0
Poland	100 676	25.4	27.1	23.6	304.5	429.0	228.8	99.5	1 150.5
Portugal	26 489	24.4	29.2	19.7	241.6	349.7	166.3	79.3	911.7
Romania	51 075	19.7	22.4	16.8	275.3	381.8	197.9	117.5	926.7
Slovenia	6 228	31.5	36.0	27.2	310.5	435.3	233.3	88.0	1 229.0
Slovakia	13 690	25.5	28.0	22.9	320.1	453.3	237.5	100.9	1 225.0
Finland	12 161	23.3	25.1	21.5	218.7	284.4	177.6	54.0	898.6
Sweden	22 405	24.8	26.5	23.1	231.6	277.9	200.8	50.8	977.6
United Kingdom	164 411	27.4	30.0	25.0	275.3	336.9	231.7	67.5	1 132.9
Iceland	597	27.7	28.9	26.5	244.2	275.3	224.3	53.1	1 032.7
Liechtenstein	62	25.3	29.3	21.7	191.3	232.6	157.4	52.1	766.0
Norway	10 766	26.9	30.3	23.9	245.9	310.0	203.2	55.8	1 030.8
Switzerland	17 297	25.6	29.4	22.1	221.6	289.3	175.4	55.1	909.3
Serbia	21 394	20.7	23.8	17.5	297.2	391.5	226.3	119.9	1 029.2
Turkey	76 581	19.7	23.5	15.1	198.7	295.0	125.6	66.5	744.5

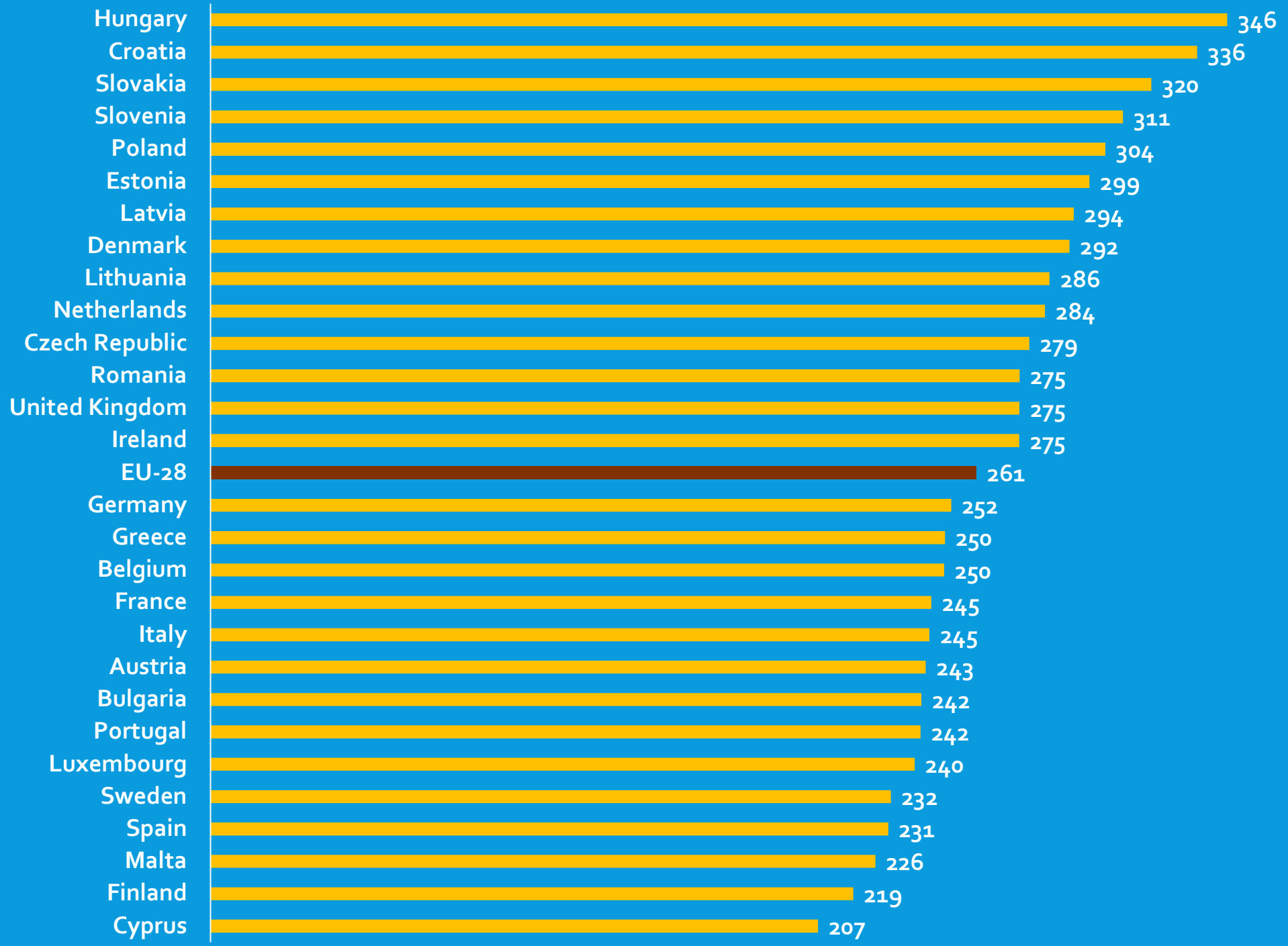
Source: Eurostat (online data codes: hlth_cd_aro and hlth_cd_asdr2)

NUMBER OF DEATHS DUE TO MALIGNANT NEOPLASMS, 2016



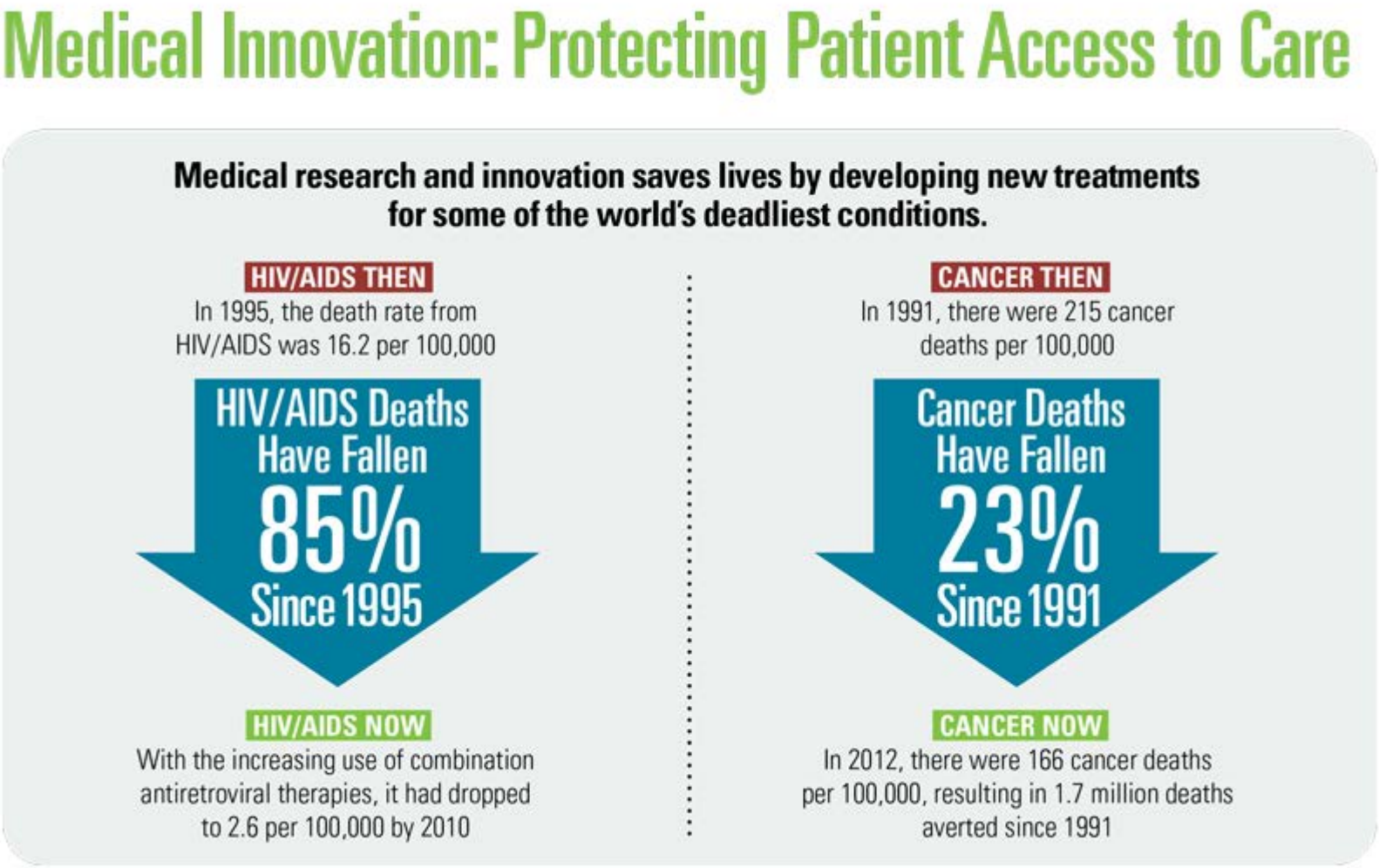
https://ec.europa.eu/eurostat/statistics-explained/index.php/Cancer_statistics#Deaths_from_cancer

STANDARIZED DEATH RATES DUE TO MALIGNANT NEOPLASMS, 2016, PER 100 000 INHIBITANTS



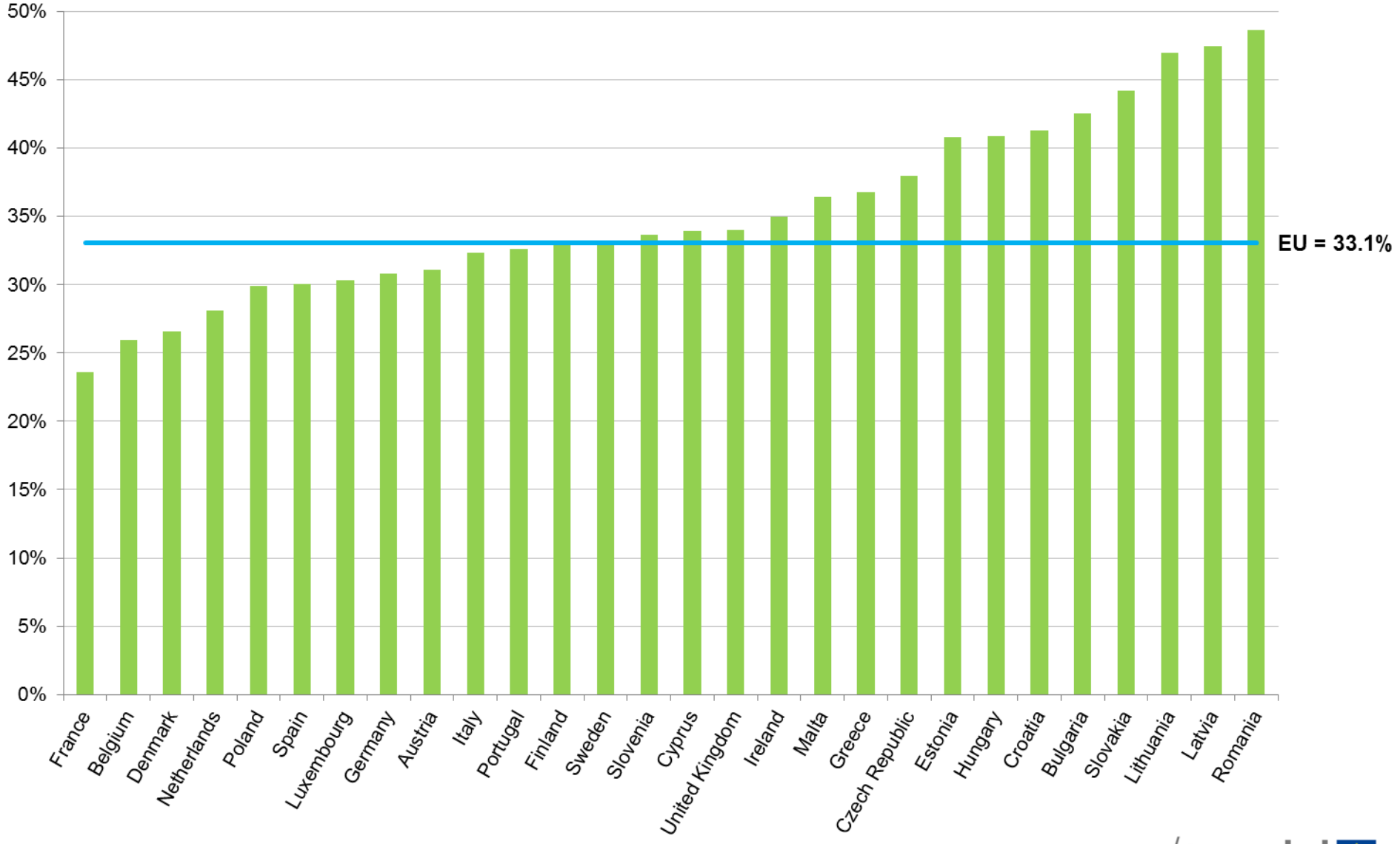
https://ec.europa.eu/eurostat/statistics-explained/index.php/Cancer_statistics#Deaths_from_cancer

EVALUATION AND ARGUMENTS FOR INNOVATION (VALUE OF INNOVATION)



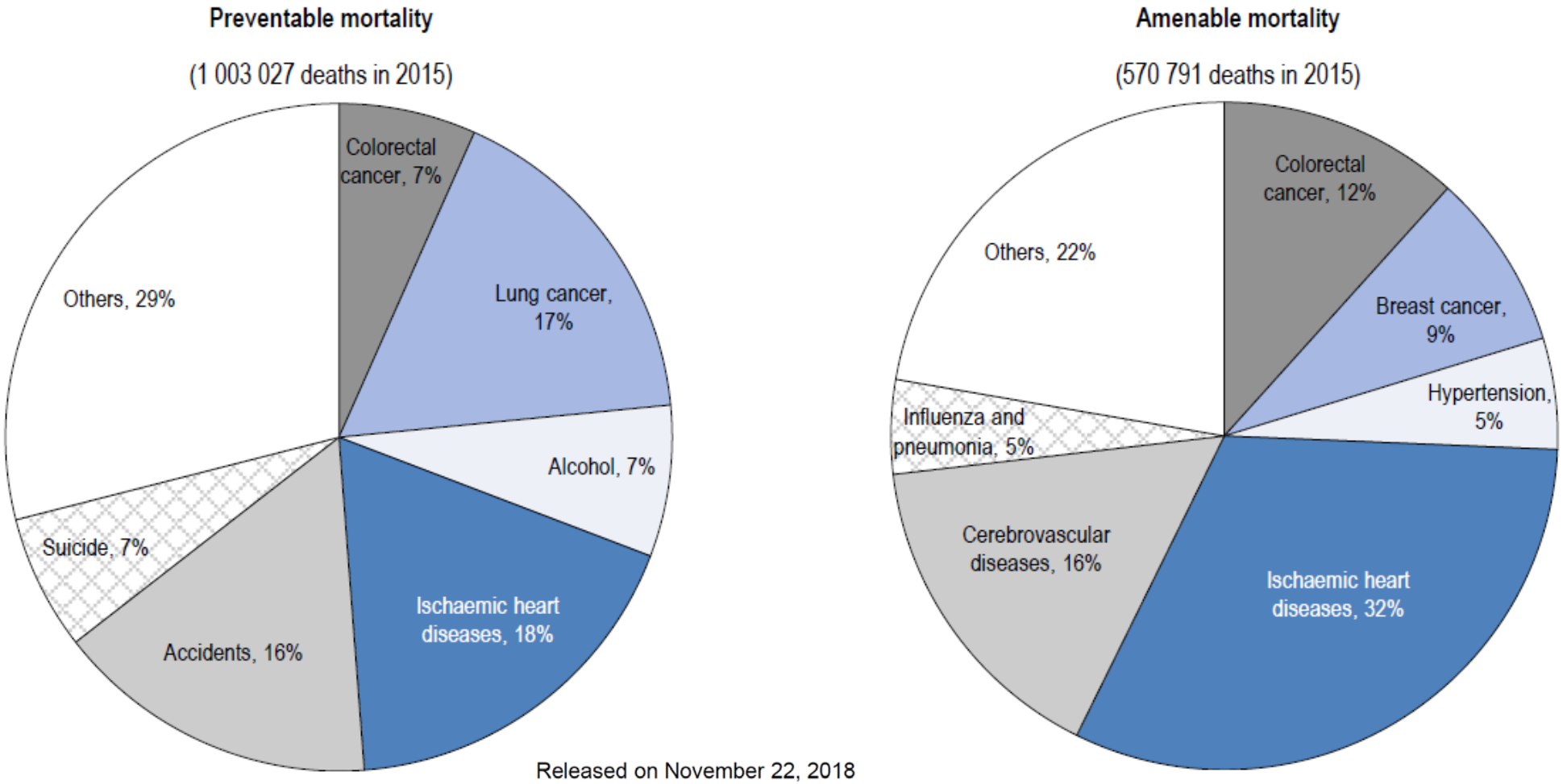
Share of avoidable deaths in the light of current medical knowledge and technology in the EU Member States, 2015

(as % of total deaths of population aged less than 75)



More than 1.2 million deaths could be avoided through better public health and prevention policies and more effective and timely health care

Leading causes of preventable and amenable mortality in the European Union, 2015

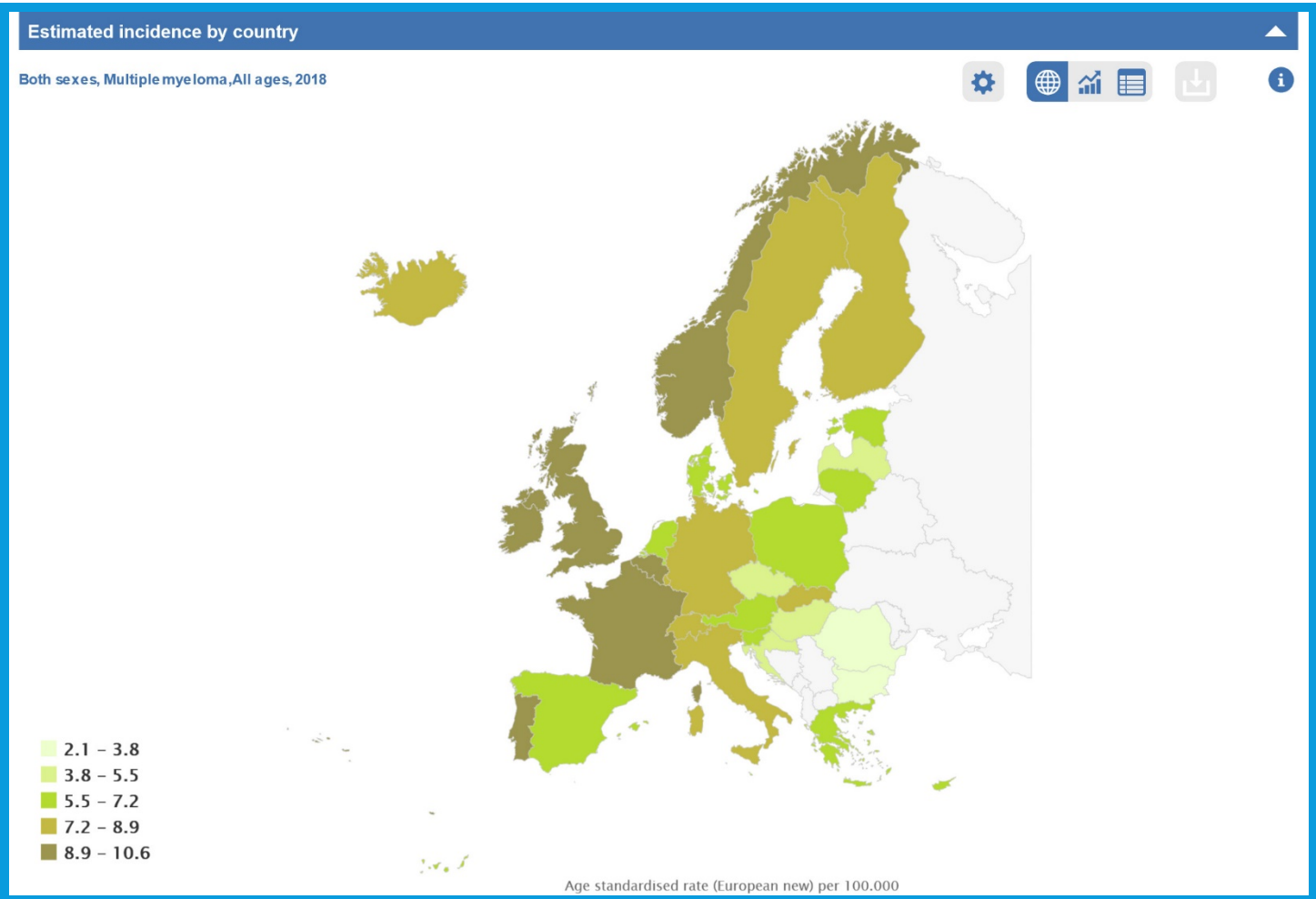


Released on November 22, 2018
http://www.oecd.org/health/health-at-a-glance-europe-23056088.htm

Note: Preventable mortality is defined as deaths that could be avoided through public health and prevention interventions, whereas amenable (or treatable) mortality is defined as deaths that could be avoided through effective and timely health care. A number of causes of death are included in both preventable and amenable mortality resulting in double-counting; this explains why the total number of avoidable deaths is lower than the sum of the two parts.

MEASURING CANCER BURDEN AND ITS TIME TRENDS ACROSS EUROPE

ECIS - European Cancer Information System
https://ecis.jrc.ec.europa.eu/

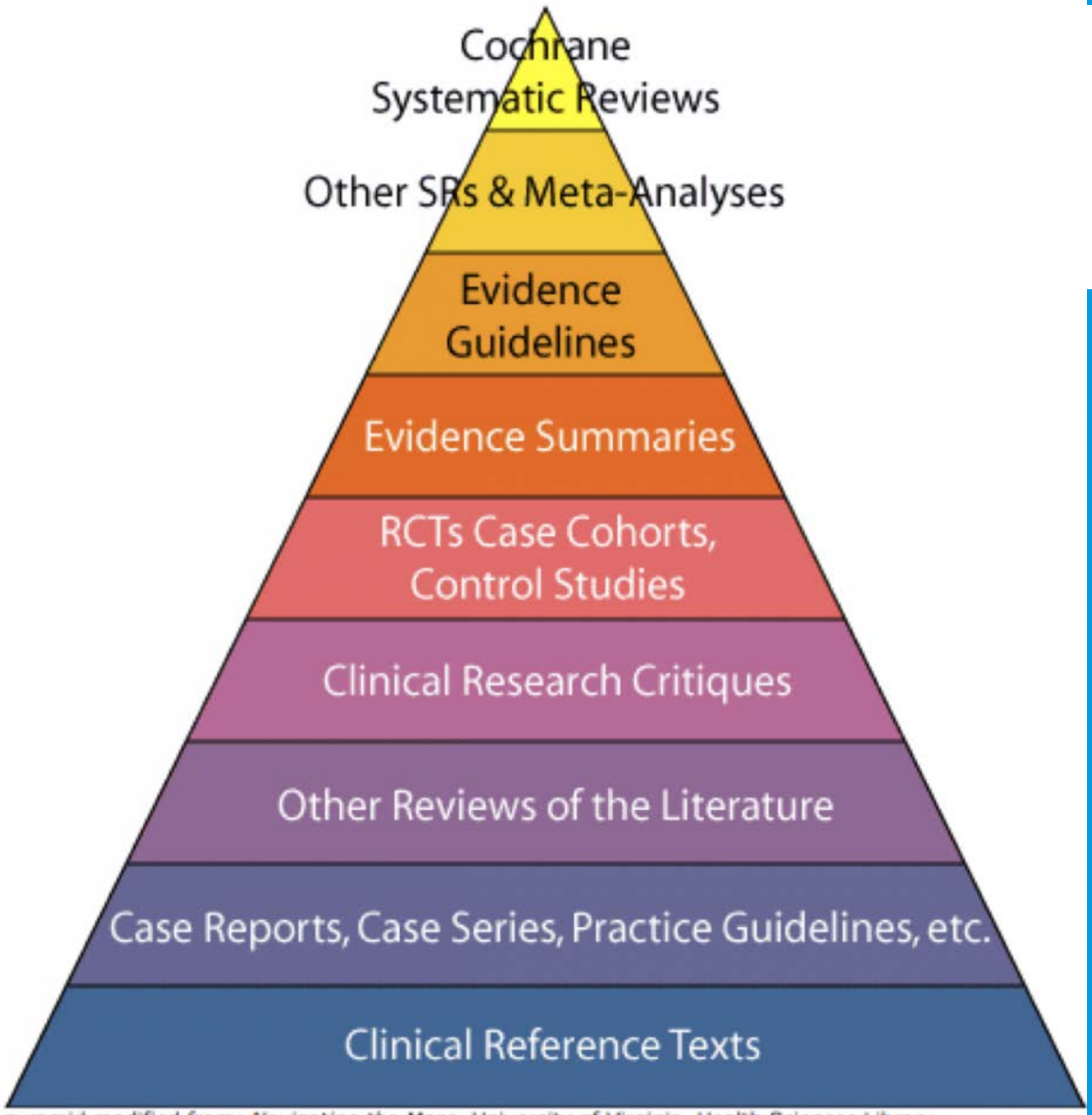


DEFINITIONS FOR EBM

Evidence-based medicine (EBM) can be defined as the formal practice of making decisions regarding the best treatment of patients based on the systematic and detailed approach of current best research evidence.

EBM is of paramount importance in the provision of the best health care policy.

Sacket DJ, Rosenberg WMC, Gray JAM, Haynes RB, Richardson WS, Evidence based medicine: what it is and what it isn't. Br Med J 1996;312:71.

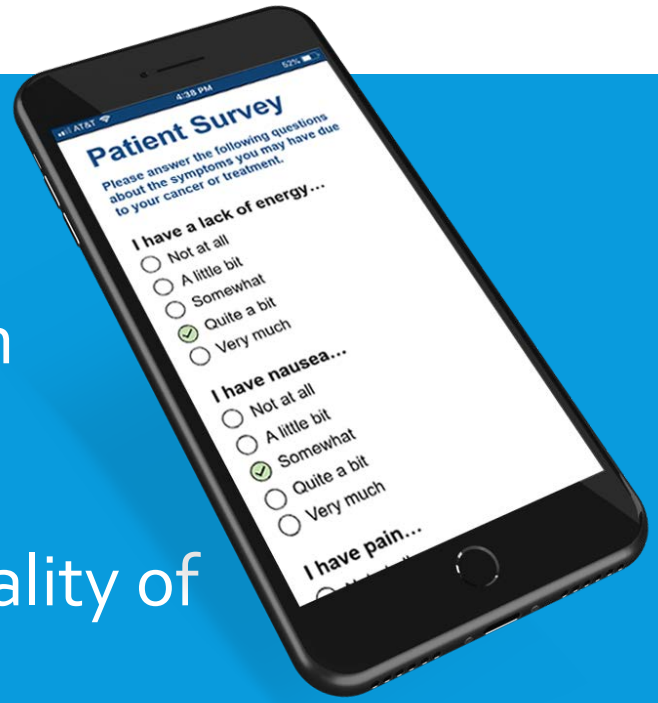


pyramid modified from: *Navigating the Maze*, University of Virginia, Health Sciences Library

P	I	C	O
Population Patient Problem	Intervention Or Exposure	Comparison	Outcome
Who are the patients? What is the problem?	What do we do to them? What are they exposed to?	What do we compare the intervention with?	What happens? What is the outcome?

Systematic Reviews, http://libguides.mq.edu.au/content.php?pid=371659&sid=3291432

DOMAINS OF QUALITY: CAPTURING PATIENTS' PERSPECTIVES ON THE EFFECTS OF CANCER AND ITS TREATMENT



The two main domains advocated for capturing patients' perspectives on the effects of cancer and its treatment are:

1) **Patient-Reported Outcome Measures (PROMs)**, which measure the impact of an illness and the effects of interventions on symptomatology and well-being (e.g. physical function, emotional distress, health-related quality of life, health status)

2) **Patient-Reported Experience Measures (PREMs)**, which capture patients' views of what happened during the health encounter (care processes)

Tremblay D et al. Determinants of patient-reported experience of cancer services responsiveness. BMC Health Serv Res. 2015; 15: 425.

PROM & PREM AS EVOLUATION OF QUALITY FROM PATIENT'S PERSPECTIVE

PROM

- **Effectiveness of care** – Does it reduce symptoms, improve function, improve quality of life?
- **Safety** – Does it cause harm eg mortality, complications?

PREM

- **Experience of care -** What do patients think of the process of care eg dignity, information, trust in staff, cleanliness, timeliness?

https://www.kingsfund.org.uk/sites/default/files/media/andrew-hutchings-prems-affect-proms-dec13.pdf

DEFINITIONS FOR EBM AND HTA

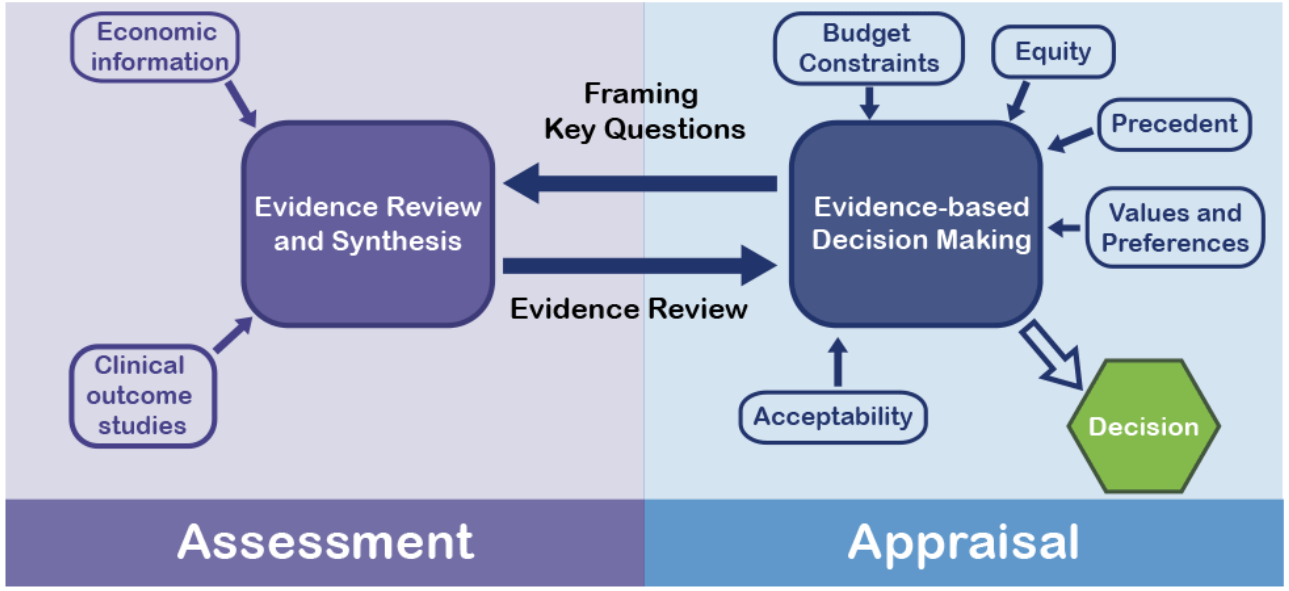
Health technology assessment (HTA) aims to inform decision making by health care policy makers.

It is a systematic process that considers health technologies and can involve a review of:

- clinical evidence compared to existing models of care,
- cost effectiveness,
- social and ethical impacts on the health care system and the lives of patients.

https://www.eupati.eu/health-technology-assessment/ht systems-in-europe/

The two main components of HTA: Assessment and Appraisal



EUPATI
European Patients' Academy on Therapeutic Innovation
www.eupati.eu

Adapted from Teutsch, S., Berger, M. (2005). 'Evidence synthesis and evidence-based decision making; Related but distinct processes. *Medical Decision Making*, pp. 487-489.

HEALTH INNOVATION

- According to WHO "Health innovation identifies new or improved health policies, systems, products and technologies, and services and delivery methods that improve people's health and wellbeing.
- Health innovation responds to unmet public health needs by creating new ways of thinking and working with a focus on the needs of vulnerable populations.
- It aims to add value in the form of improved efficiency, effectiveness, quality, sustainability, safety and/or affordability. Health innovation can be preventive, promotive, curative and rehabilitative and/or assistive care.
- WHO engages in health innovation to achieve universal health coverage within the context of the Sustainable Development Goals."

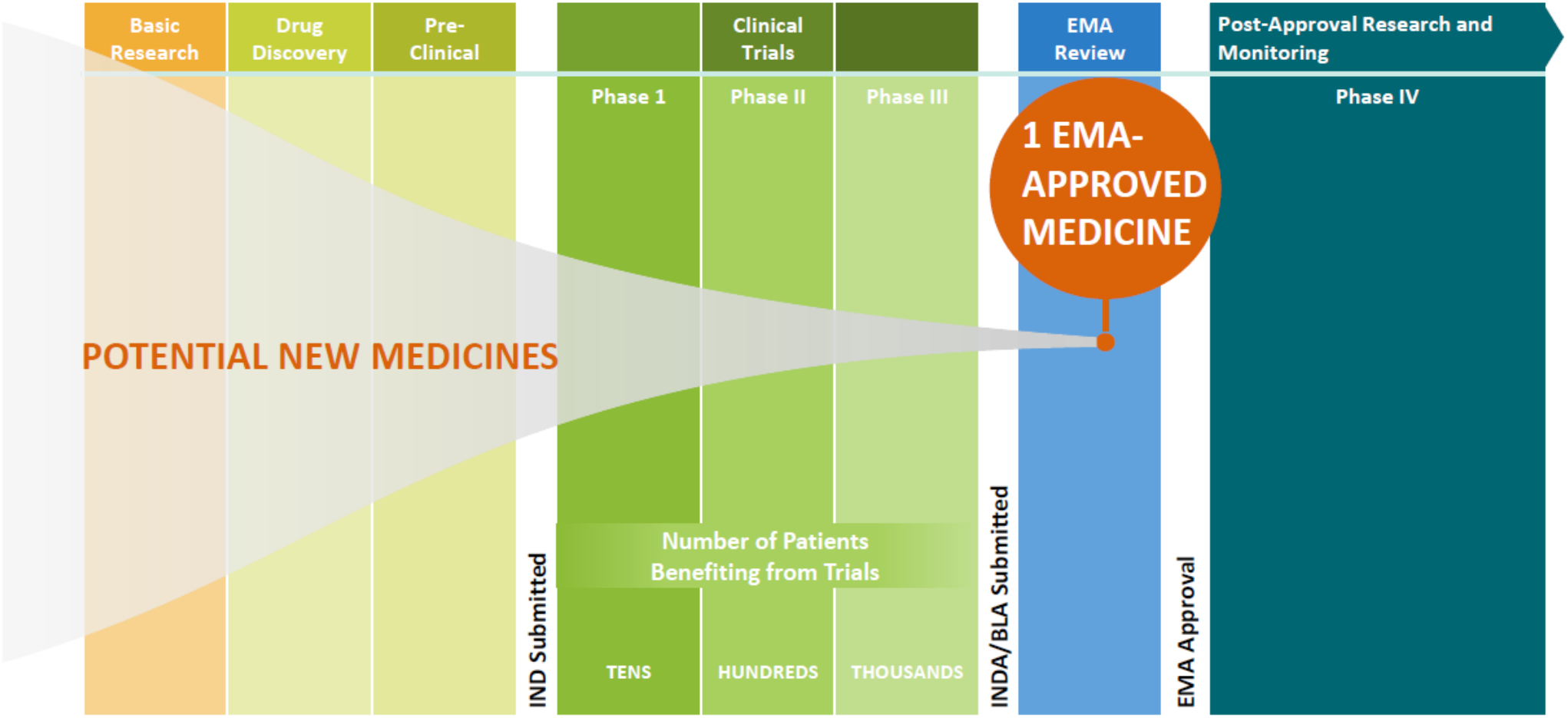
https://www.who.int/topics/innovation/en/

The biopharmacetical

research and development process

http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

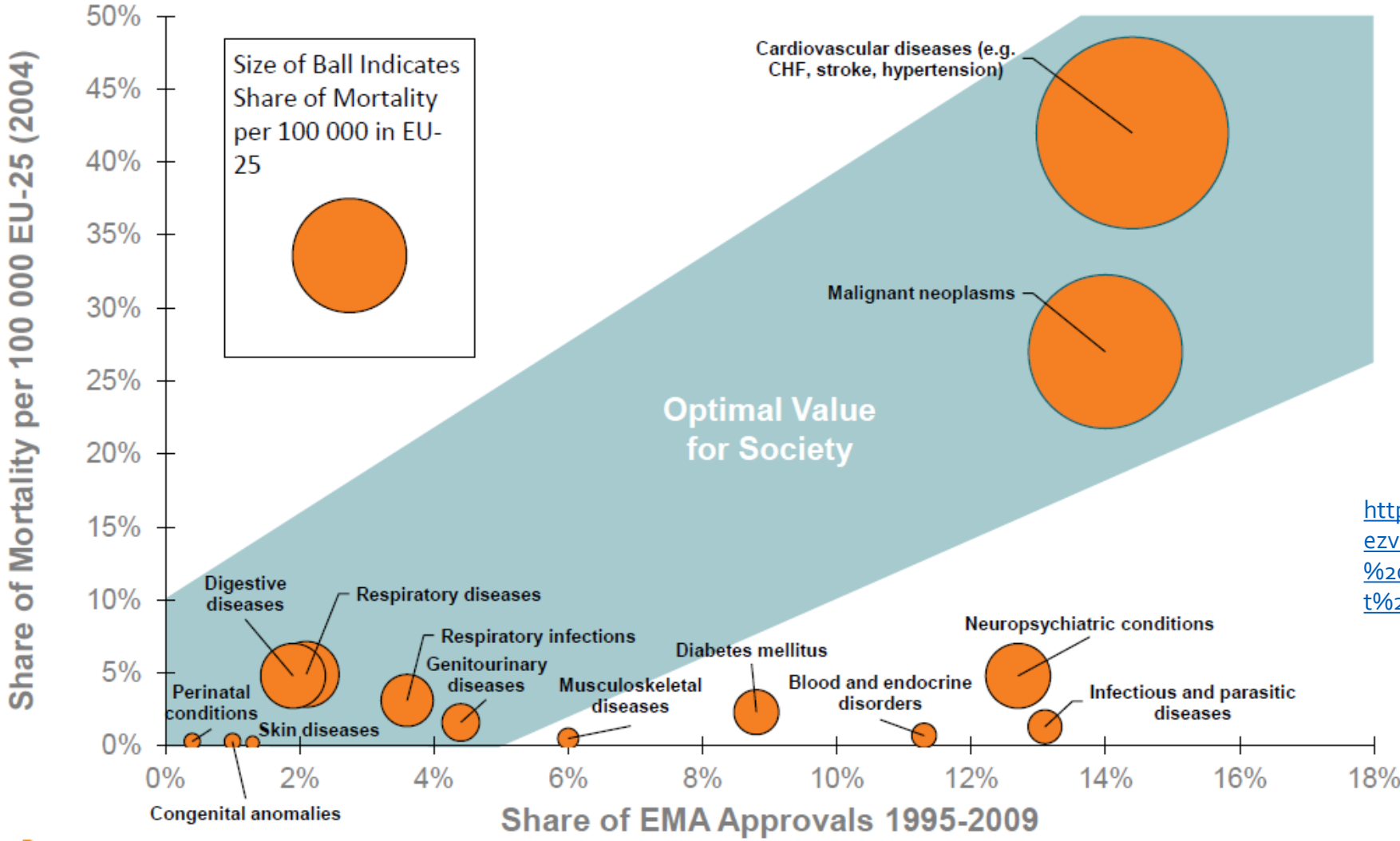
From drug discovery to EMA approval, developing a new medicine on average takes at least 10 years



Note: *The average R&D cost required to bring a new, FDA approved medicine to patients is estimated to be $2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.
Key: IND: Investigational New Drug Application, NOA: New Drug Application. BLA: Biologics License Application.
Source: PhRMA 2016 Prescription Medicines: Costs in Context.

Industry development and subsequent approvals have historically been **Targeting major unmet medical need**

There is a strong association between industry development and priority disease areas with the largest impact on society



http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

Source: Health Advances analysis; Catala-Lopez 2010 Does the development of new medicinal products in the European Union address global and regional health concerns? Pop Health Met.

BY THE NUMBERS: THE VALUE OF MEDICINES

Reduction in direct medical costs attributable to cardiovascular medicines like statins:
27%[115]

Amount of potential savings from correct use of medications for chronic conditions:
$213 billion[116]

Total healthcare dollars spent on medicines:
14%[117]

Decline in cancer deaths between 1991 and 2016:
An overall decline of 27%
in 25 years, meaning an estimated 2.6 million fewer cancer deaths than expected if death rates had remained at their peak.[118]

For patients with early-stage HIV, **lifetime cost savings after the introduction of antiretroviral therapies:**
$402,000[119]

Percentage of **prescriptions that are for generic medicines:**
90%[120]

Proportion of recent gains in **life expectancy attributable to medicines:**
70%[121]

FAST FACT

When we assess the value of our medicines, we follow four principles:

1. What matters most in determining a medicine's value is its impact on patients.[127]

2. The value of a medicine should include its impact on the healthcare system and society.[128]

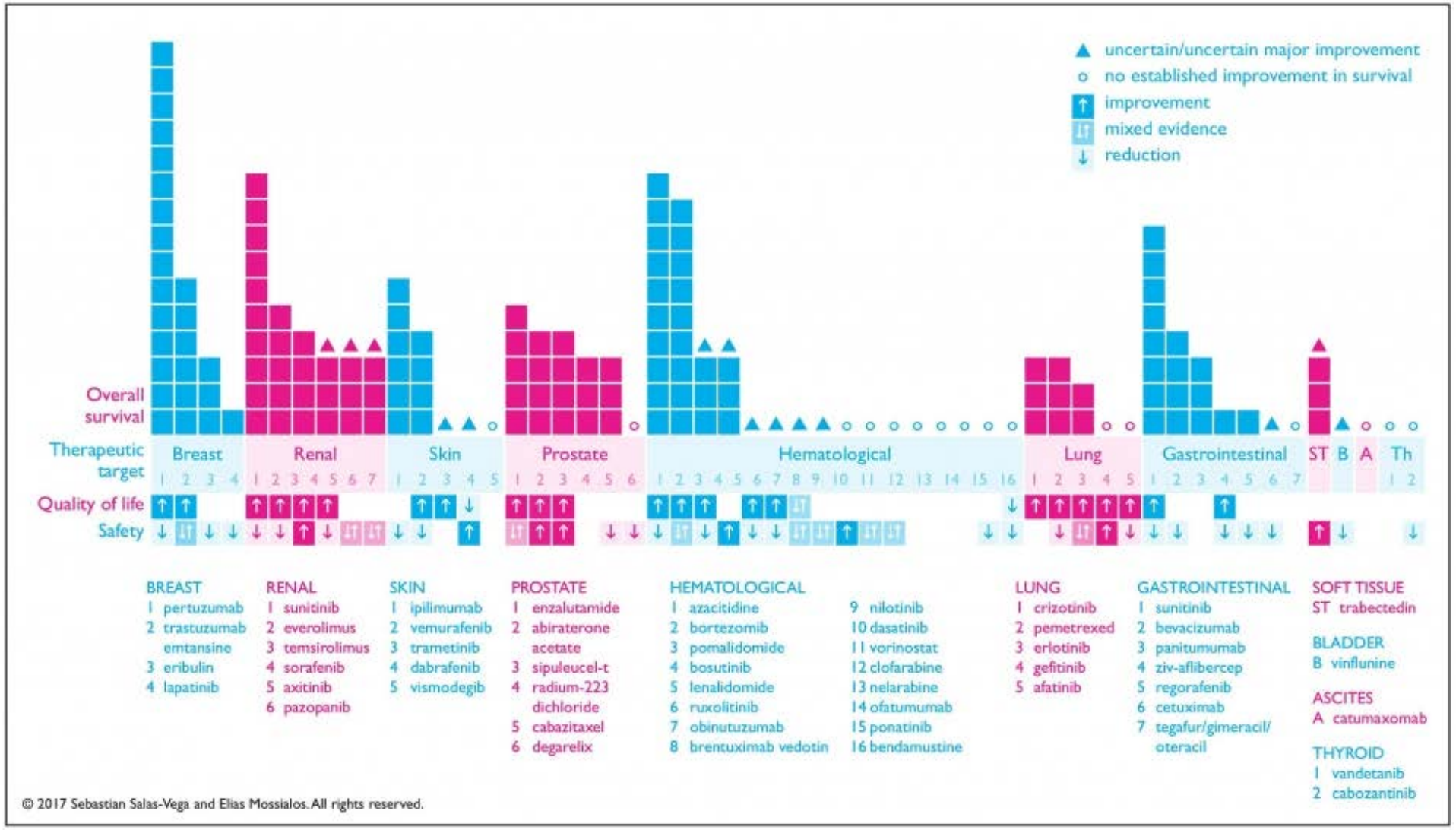
3. Treatment outcomes should be assessed over an appropriate timeframe to capture all the benefits and risks for patients, the healthcare system, and society.[129]

4. Evidence considered in assessing the value of a medicine should be high-quality, current, and relevant.[130]

https://transparencyreport.janssen.com/us/us-pharmaceutical-transparency-report

Of 53 new cancer drugs analysed, researchers found no known improvement in overall survival from new medicines for thyroid cancers and malignant ascites from 2003 to 2013, but an average improvement of 8.48 months for breast cancers over this period. The average improvement in overall survival for people with lung, haematological, gastrointestinal, prostate, skin, and renal cancers equalled 2.09, 2.61, 2.90, 3.17, 4.65, and 6.27 months, respectively. Within indications as well, there was often a wide range in the overall survival benefits associated with new medicines. On average, all newly licensed cancer drugs extended overall survival by 3.43 months over the past decade

Figure 1. New cancer medicines grouped by therapeutic target and effect on overall survival, quality of life and safety



© 2017 Sebastian Salas-Vega and Elias Mossialos. All rights reserved.

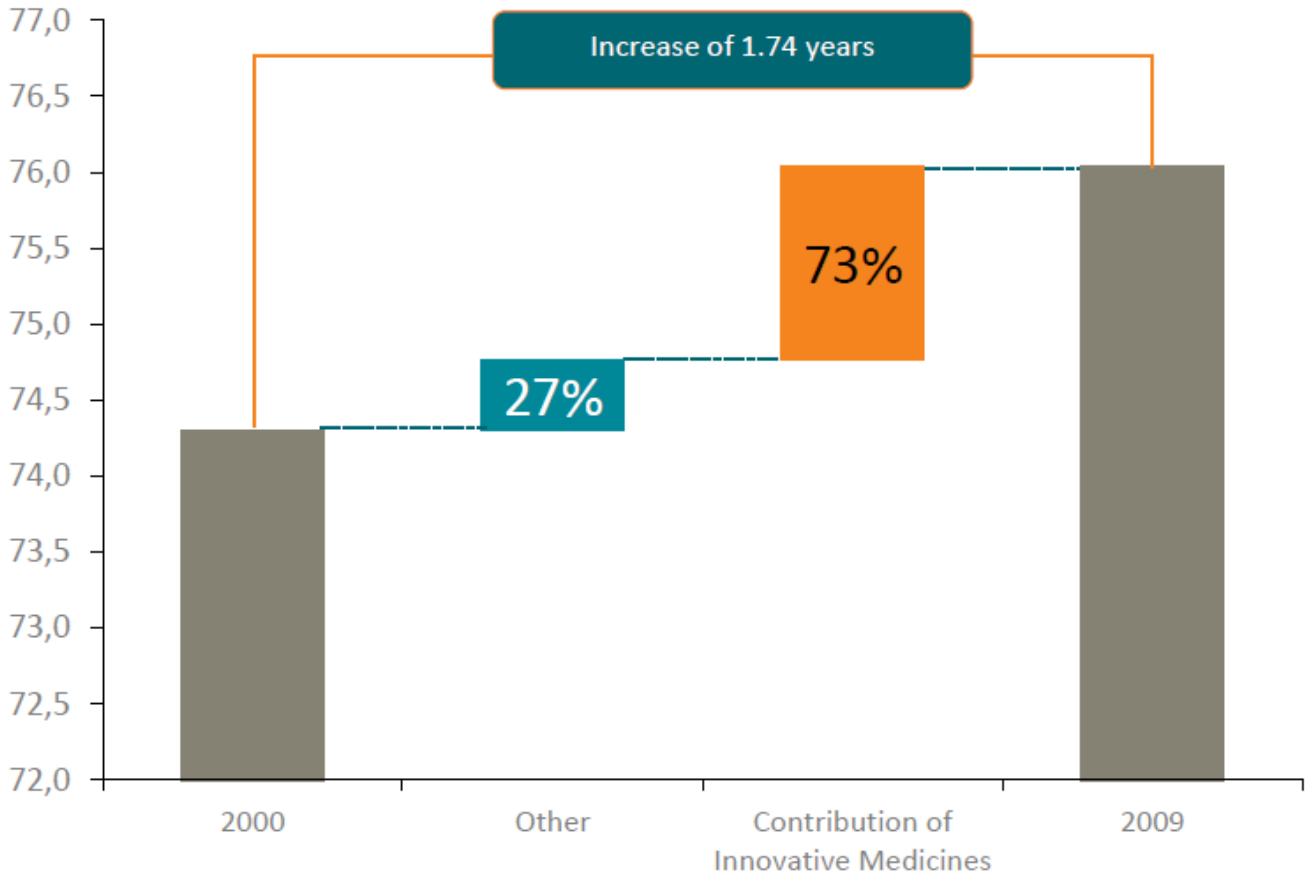
Source: Authors' analyses.
Notes: Clinical benefits of new medicines approved between 2003 and 2013 by US and EU authorities with a primary anti-cancer indication, as evaluated by health technology appraisals. Overall survival benefits are rounded to the nearest integer and represented as blocks, where each block corresponds to an improvement of one month relative to existing regimens or treatments. 'Uncertain major improvement' refers to increases in overall survival of at least three months, where the exact magnitude is unknown. Uncertain improvements are those where no further information is provided

https://blogs.lse.ac.uk/healthandsocialcare/2017/01/05/value-in-cancer-drug-spending-assessing-the-clinical-risks-and-benefits-from-a-decades-worth-of-innovation/

For cancer patients, life expectancy continues to improve the use of innovative **medicines has made major contribution to recent advances**

From 2000 – 2009, an **improvement** in population weighted **mean life expectancy** at birth of **1.74 years** was seen across 30 OECD countries.

Innovative medicines are estimated to have contributed **to 73% of this improvement** once other factors are taken into account (e.g. income, education, immunization, reduction in risk factors, health system access).



http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

Source: Lichtenberg, F: Pharmaceutical innovation and longevity growth in 30 developing OECD and high-income countries, 2000 - 2009 (2012)

An improved understanding of disease and personalised medicines have resulted in **increased patient survival**

Advances in personalized medicine have **improved the outlook for patients with blood cancers in Europe**[1]

Improved Understanding of the Disease

60 YEARS AGO

- "Disease of the Blood"

50 YEARS AGO

- Leukemia
- Lymphoma

40 YEARS AGO

- Chronic Leukemia
- Acute Leukemia
- Pre-leukemia
- Indolent Lymphoma
- Aggressive Lymphoma

TODAY

- **~40** unique **Leukemia types** identified
- **~50** unique **Lymphoma types** identified

Today, 230 medicines* [2] are in development for blood cancers in Europe

A greater understanding of the molecular basis of disease has transformed what was once known collectively as "disease of the blood," into multiple subtypes of leukemia and lymphomas, which can be targeted by personalized medicines

Chronic Lymphocytic Leukemia 5 year survival rates have **grown to 70%**[3]

Hodgkin's Lymphoma 5 year survival rates have **grown to 80%**[3]

http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

* Medicines in phases I through III for the treatment of all types of leukemia and lymphoma.

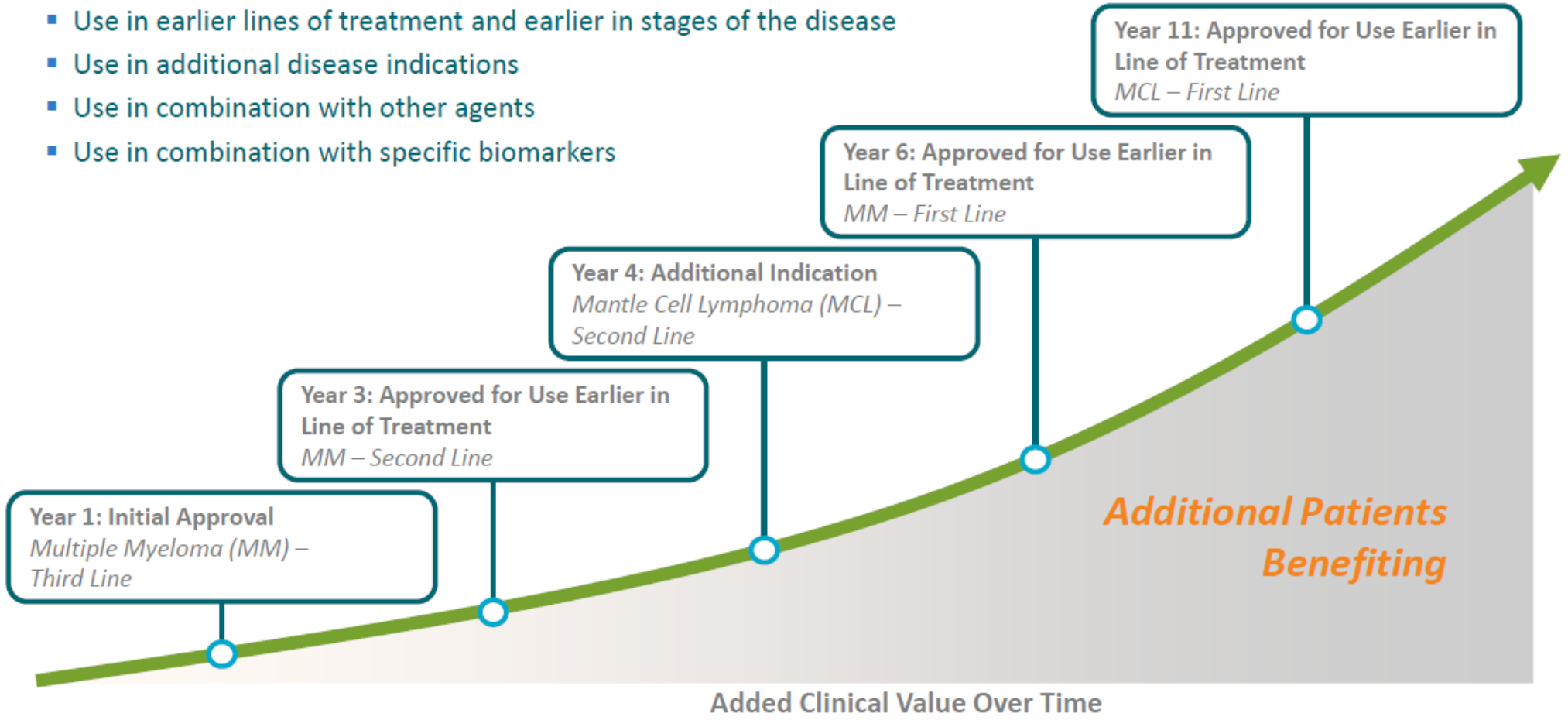
Source: Health Advances analysis; [1]PhRMA 2015 Value of Personalized Medicine; [2]PharmaProjects (accessed February 2016); [3]Cancer Survival in Europe Eurocare 5 Database 2000-2007.

Medicines often demonstrate
Far greater benefits than understood at time of approval

http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

Oncology therapeutics demonstrate **increasing clinical value over time** long after initial approval

- Use in earlier lines of treatment and earlier in stages of the disease
- Use in additional disease indications
- Use in combination with other agents
- Use in combination with specific biomarkers



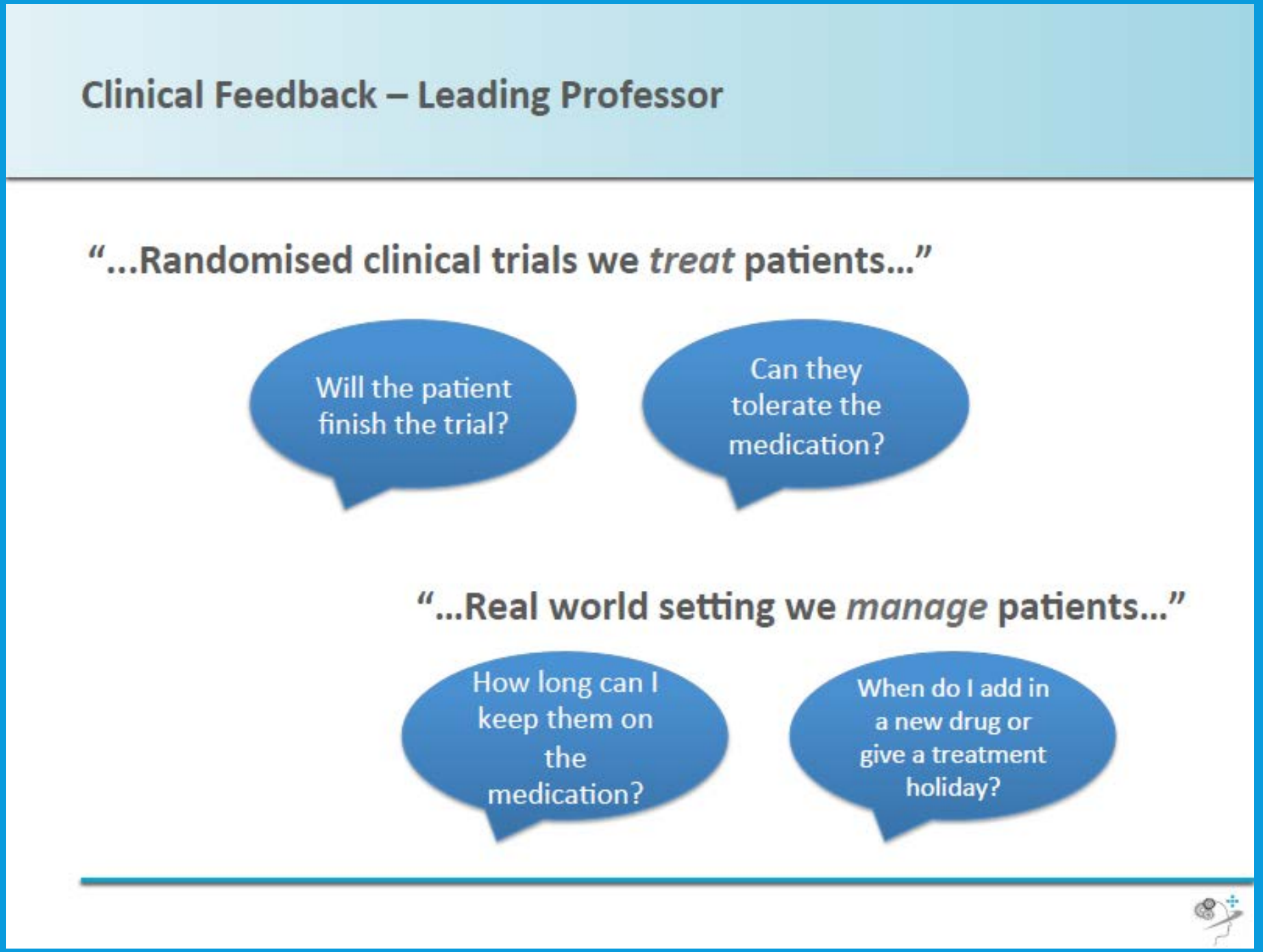
Note: Representation of the change in clinical value over time in the US as additional data and evidence became available for Bortezomib.
Source: Health Advances analysis; Boston Healthcare Assoc. 2015 The Value of Innovation in Oncology.

HEALTH INDICATORS IN HAEMATOLOGY

- **OS (overall survival)** - The length of time from either the date of diagnosis or the start of treatment for a disease, such as cancer, that patients diagnosed with the disease are still alive. In a clinical trial, measuring the overall survival is one way to see how well a new treatment works.
- **PFS (progression-free survival)** - The length of time during and after the treatment of a disease, such as cancer, that a patient lives with the disease but it does not get worse. In a clinical trial, measuring the PFS is one way to see how well a new treatment works.
- **QoL (quality of life)** - Many clinical trials assess the effects of cancer and its treatment on the quality of life. These studies measure aspects of an individual's sense of well-being and ability to carry out activities of daily living.

https://www.cancer.gov/publications/dictionaries/cancer-terms/def/overall-survival

RWD/RWE: medical and payer registries, big data



REAL-WORLD DATA ANALYSIS

- To improve health outcomes for patients by accelerating research and real-world data generation and analysis, increase transparency and information sharing, and enhance the understanding and utilization of real world data.
- The collaboration has focused on the follow-up and effects of disease and treatment, using routine data from health care ("Real World Data"), and how they relate to effects in clinical trials, all while social and economic factors are evaluated.

https://www.janssen.com/partnerships/collaborating-karolinska-institutet-advanced-real-world-data-analysis

RWD/RWE AS A SOLID BASIS FOR REIMBURSEMENT DECISIONS

Figure 3. Where are the biggest opportunities to leverage RWE?

Source: Deloitte 2017 RWE Benchmark Survey

REAL-WORLD DATA ANALYSIS

- The need to generate evidence in the real world, based on data derived in the clinical setting outside of the randomized clinical trial, is growing rapidly.
- Through extensive external collaborations, Janssen develops infrastructure and incorporate diverse capabilities and skillsets to interact with and analyze real world data (RWD) to generate real world evidence (RWE).
- The introduction of RWD/EHR (Electronic Health Records) platforms could facilitate optimization of clinical research and stimulate strategic collaborations to advance medical science overall.

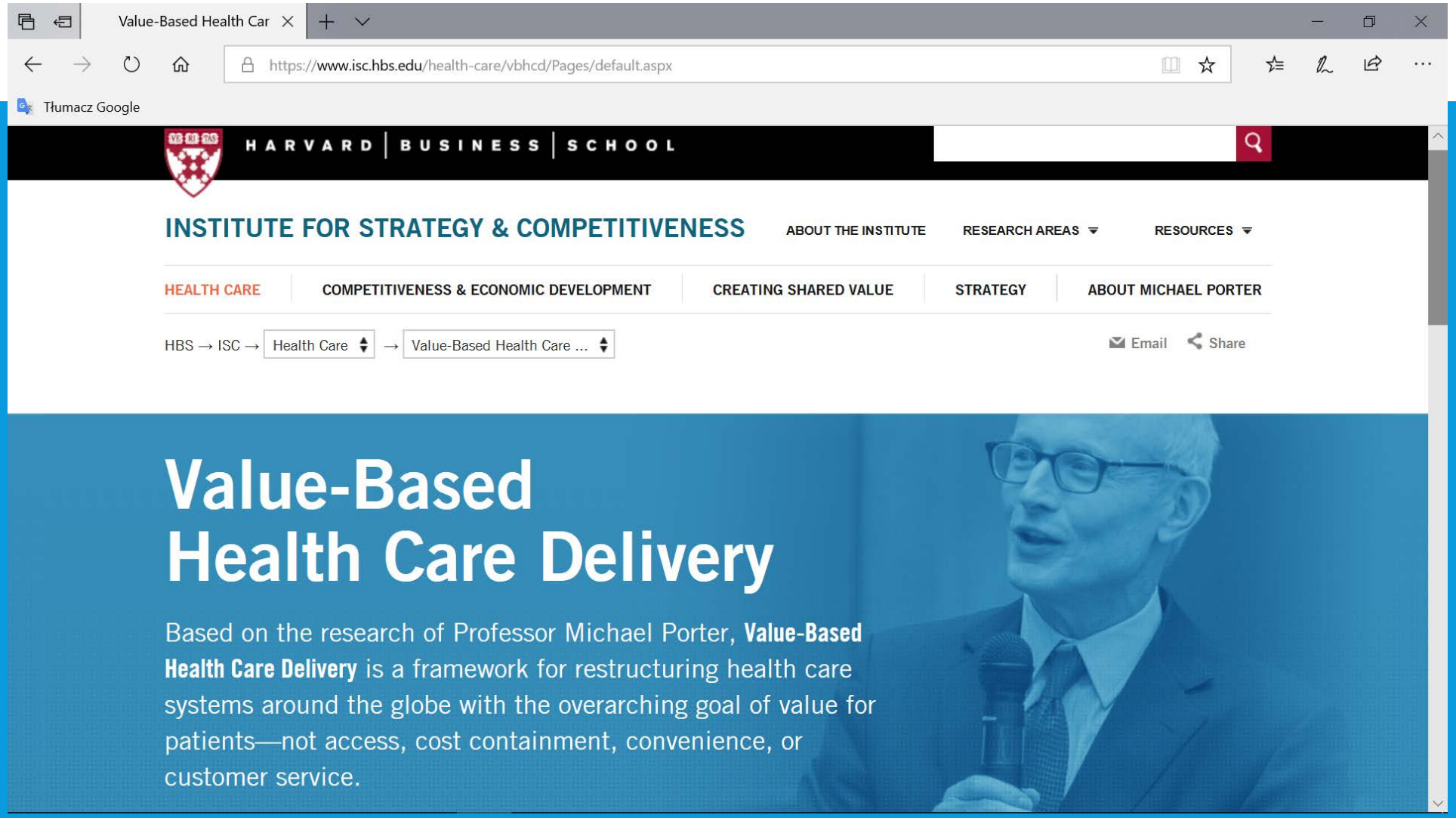
https://www.janssen.com/partnerships/collaborating-karolinska-institutet-advanced-real-world-data-analysis

REGISTRIES

- **European Platform on Rare Disease Registration (EU RD Platform) https://eu-rd-platform.jrc.ec.europa.eu/**
- **Myeloma**
  - Sweden http://www.haematologica.org/content/103/3/506
  - Denmark http://www.danishhealthdata.com/find-health-data/Dansk-Myelomatose-Database
  - Austria https://www.oncotyrol.at/amr/
  - Czech Rep. https://www.myeloma.cz/index-en.php?pg=rmg-registry-of-monoclonal-gammopathies

INTRODUCTION TO VALUE BASED HEALTH CARE

Porter's definition and methodology



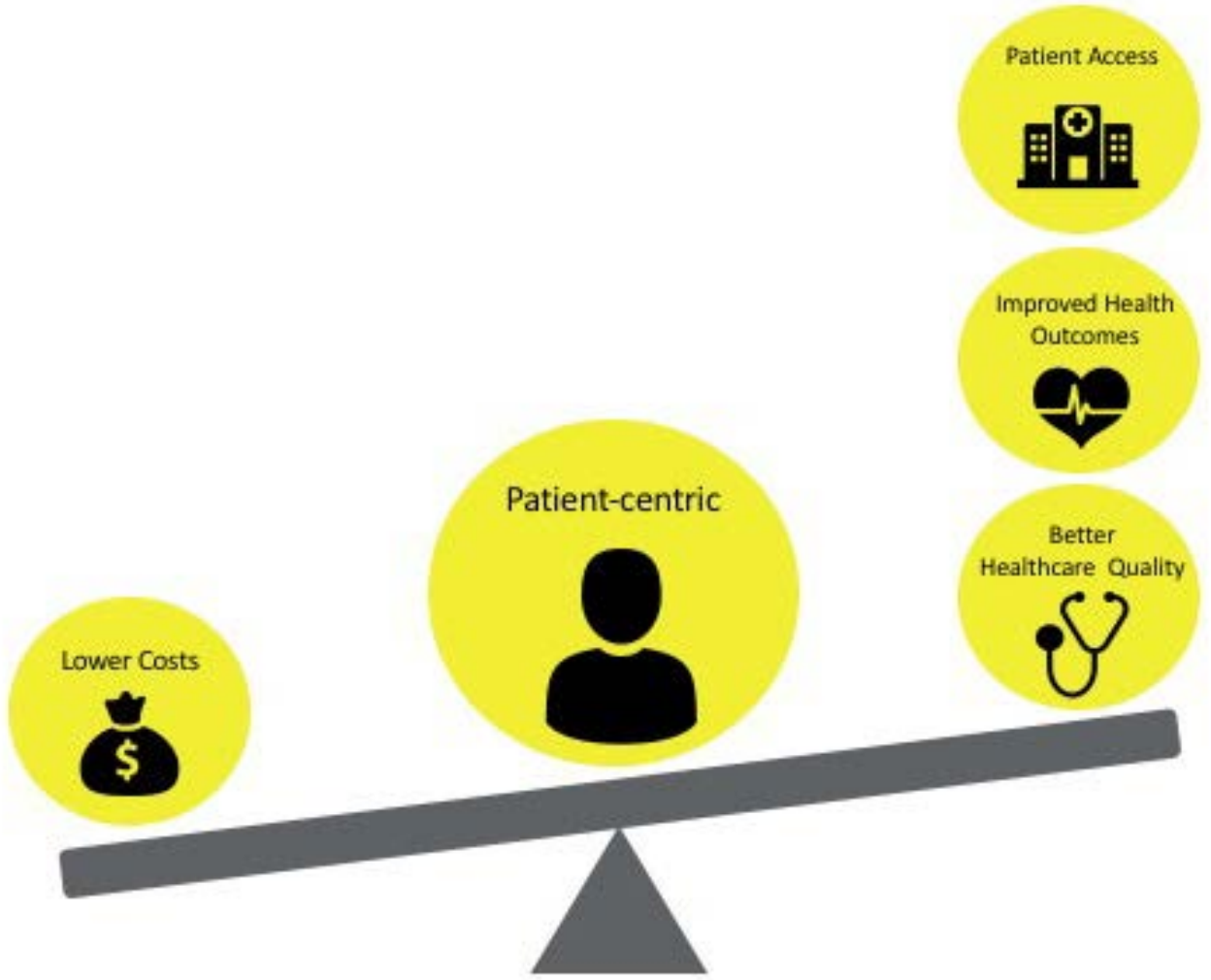
https://www.isc.hbs.edu/health-care/vbhcd/Pages/default.aspx

INTRODUCTION TO VALUE BASED HEALTH CARE

$$\text{Value} = \frac{\text{Quality}}{\$}$$

VALUE BASED HEALTH CARE - VBHC

Patient should be in the center of system with access to quality care, monitored in terms of improved health outcomes and optimal costs



https://crcaustralia.com/media-releases/from-evidence-based-healthcare-is-australia-ready/figure2/

INTRODUCTION TO VALUE BASED HEALTH CARE

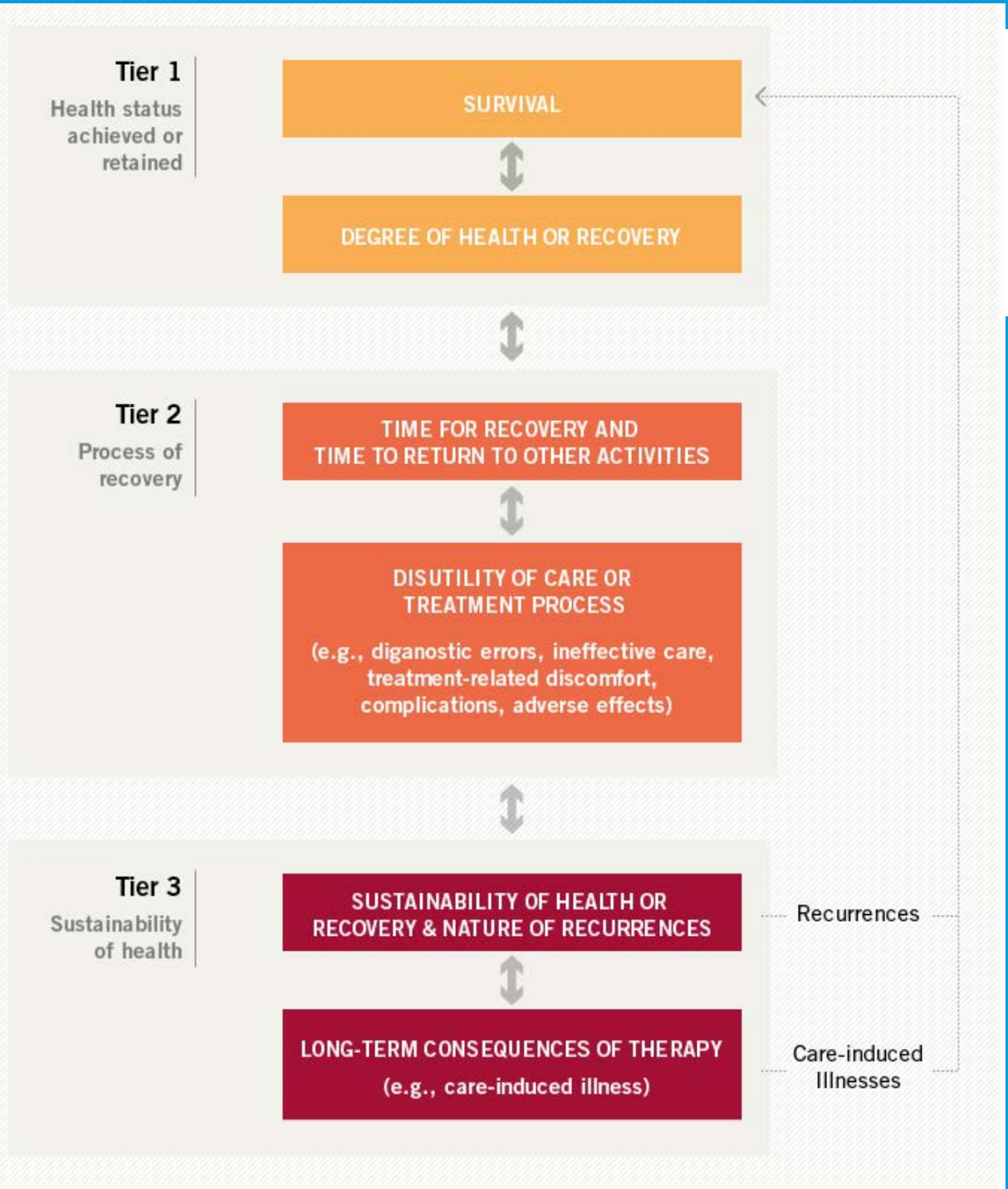
- Measure Outcomes & Costs for Every Patient
- Measuring Costs
- Systems Integration

https://www.isc.hbs.edu/health-care/vbhcd/Pages/default.aspx

THE OUTCOME MEASURES HIERARCHY

- Health care should focus on the things patients actually care about:
  - survival,
  - quality of life,
  - rates of complications after care

https://www.isc.hbs.edu/health-care/vbhcd/Pages/measuring-outcomes.aspx



THE OUTCOME MEASURES HIERARCHY

What is the value-based approach to outcome measurement?

- Outcomes are the ultimate measure of quality. In health care, measurement of value should focus on how well the care delivered meets individual patients' needs.
- Measuring success, or the results of treatment, requires following the patient through the process of care, and looking at medical conditions and patients holistically.
- For example, for patients with cancer, their medical condition includes co-existing fatigue, immunodifficency, and mal nutrition. Success in treating cancer incorporates the combined effect of caring for all of these needs.

https://www.isc.hbs.edu/health-care/vbhcd/Pages/measuring-outcomes.aspx

PRINCIPLES OF MEASURING THE COST OF CARE

- Cost is the **actual expense** of patient care, not the **charges** billed or collected
- Cost should be measured around the **patient**
- Cost should be aggregated over the **full cycle of care for the patient's medical condition**, not for departments, services, or line items
- Cost depends on the **actual use of resources** involved in a patient's care process (personnel, facilities, supplies):
  - The **time** devoted to each patient by these resources
  - The **capacity cost** of each resource
  - The **support costs** required for each patient-facing resource

https://www.isc.hbs.edu/health-care/vbhcd/Pages/measuring-outcomes.aspx

SYSTEMS INTEGRATION

- Value-based health care delivery organizes care around patient needs

Steps for Creating an Integrated Care Delivery System

- Choose an **overall scope of services** where the provider system can achieve excellence in value
- **Rationalize service lines / IPUs across facilities** to improve volume, better utilize resources, and deepen teams
- Offer specific services at the **appropriate facility** based on medical condition, acuity level, resource intensity, cost level, need for convenience
- Clinically integrate care **across units and facilities** using an IPU structure:
  - Integrate services across the care cycle
  - Integrate preventive/primary care units with specialty Integrated Patient Units (IPUs)

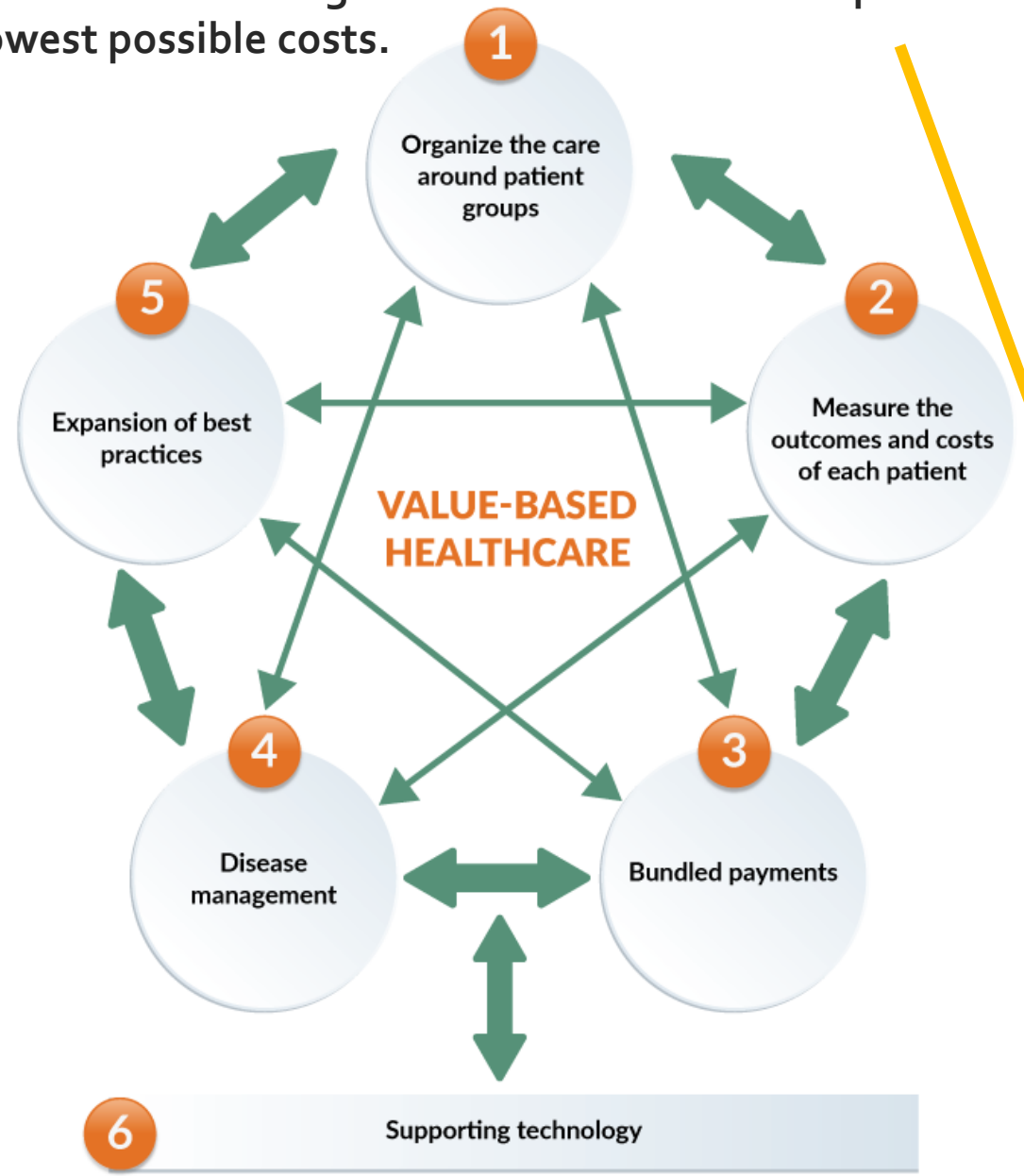
https://www.isc.hbs.edu/health-care/vbhcd/Pages/measuring-outcomes.aspx

A VBHC APPROACH OF MULTIPLE MYELOMA - A HIGH COMPLEX MEDICAL CONDITION THAT REQUIRES A FOCUS ON TRUE OUTCOMES

- In order to provide the best, most valuable care possible, Máxima Oncology Center developed a set of outcome measures that really matter to patients with Multiple Myeloma.
- Patients became part of the expert teams responsible for the integrated pathways of care
- The most important learning of the identification of outcome measures that really matter to patients with Multiple Myeloma is that all employees, working in Máxima Oncology Center, think about adding value for the patients every day. Every treatment, intervention and development always takes place in collaboration with the patient.
- Another learning of the development of outcome measures is to involve a multidisciplinary team with different perspectives. This team should include patient representatives from official patient organizations, registry experts and leading physicians. Use universal health outcomes to facilitate international collaboration.

https://www.vbhc.nl/wp-content/uploads/VBHC-Thinkers-Magazine-VBHC-Prize-2018-edition_v2.pdf

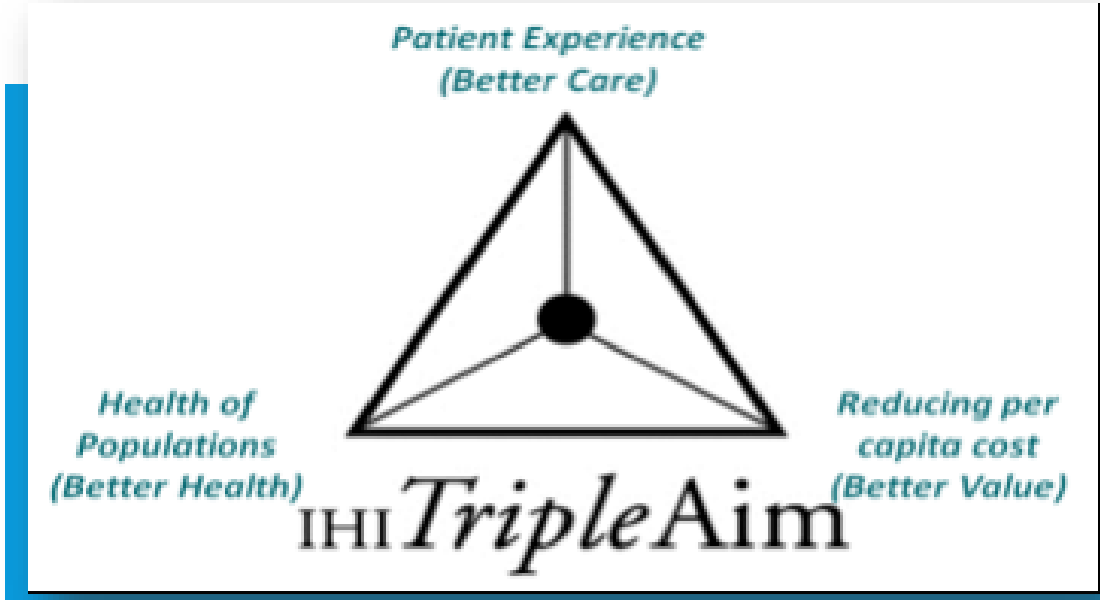
VBHC is about realizing the best outcome for the patient at the lowest possible costs.



https://www.essenburgh.com/en/blog/the-3-differences-between-value-based-healthcare-and-triple-aim-that-you-need-to-know

ESSENBURGH
CARE MATTERS

VBHC VS. TA



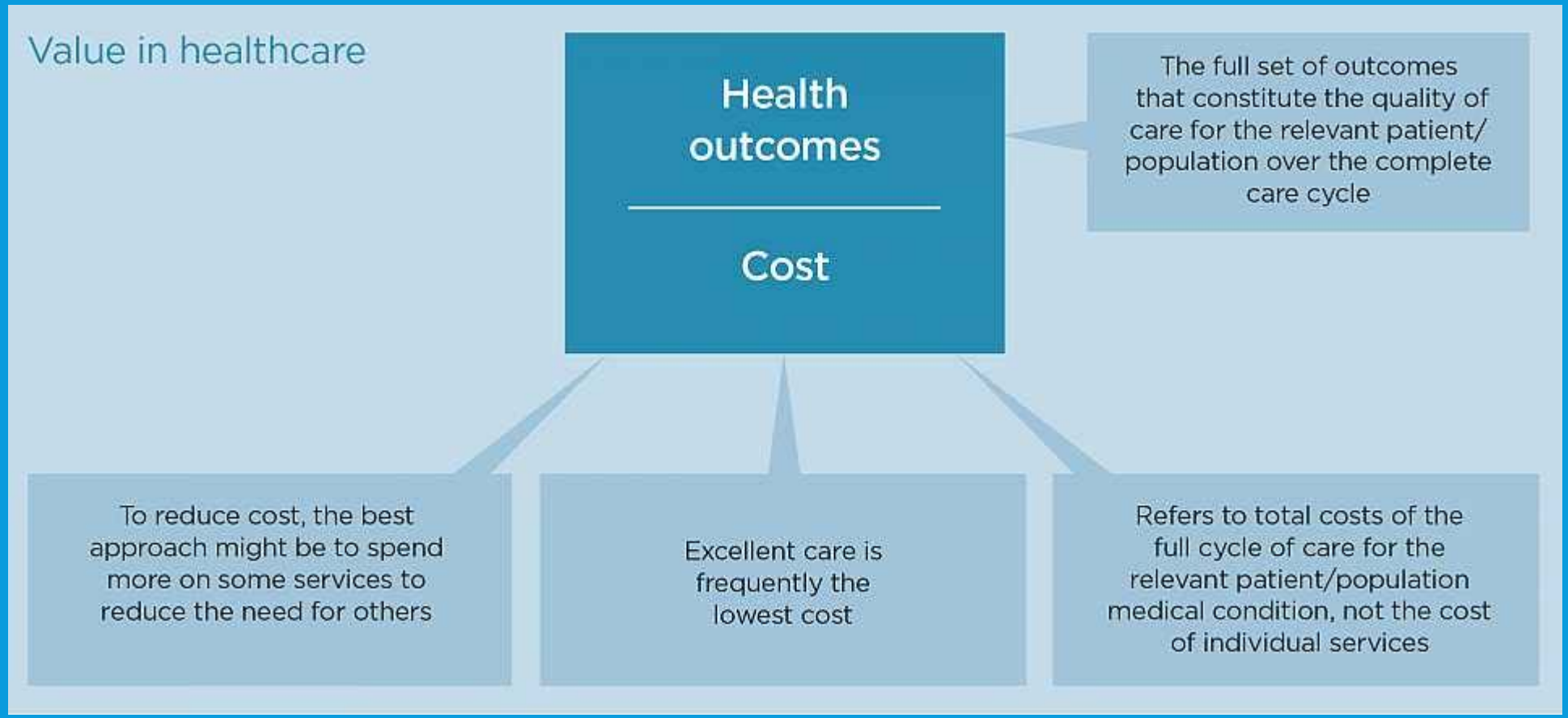
The Triple Aim approach goes one step further than VBHC and claims that a population approach and multi-stakeholder collaboration are needed to create value.

https://www.healthcatalyst.com/improving-healthcare-outcomes-keep-triple-aim-in-mind

HOW TO CONVINCE THE PAYERS WITH EVIDENCE

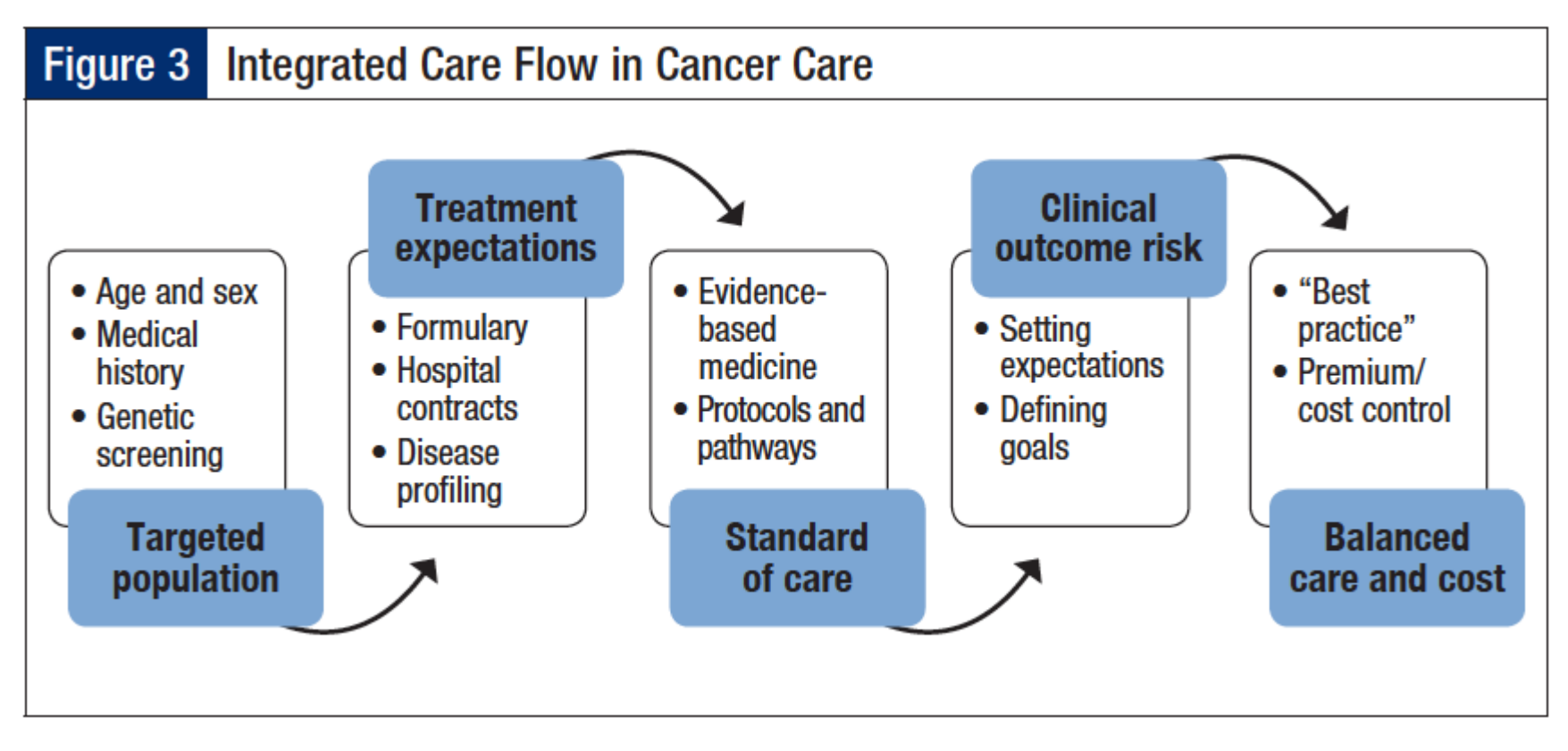
- Data evidence for Value based approach
- Patient's centric approach
- Managed entry agreements and key data - how to use them
- How to benchmark best examples from other CEE countries
- Networking in Data and Value evidence

DATA EVIDENCE FOR VALUE BASED APPROACH



https://www.hilldickinson.com/insights/articles/face-value-how-value-based-approach-healthcare-could-be-game-changer-nhs

DATA EVIDENCE FOR VALUE BASED APPROACH



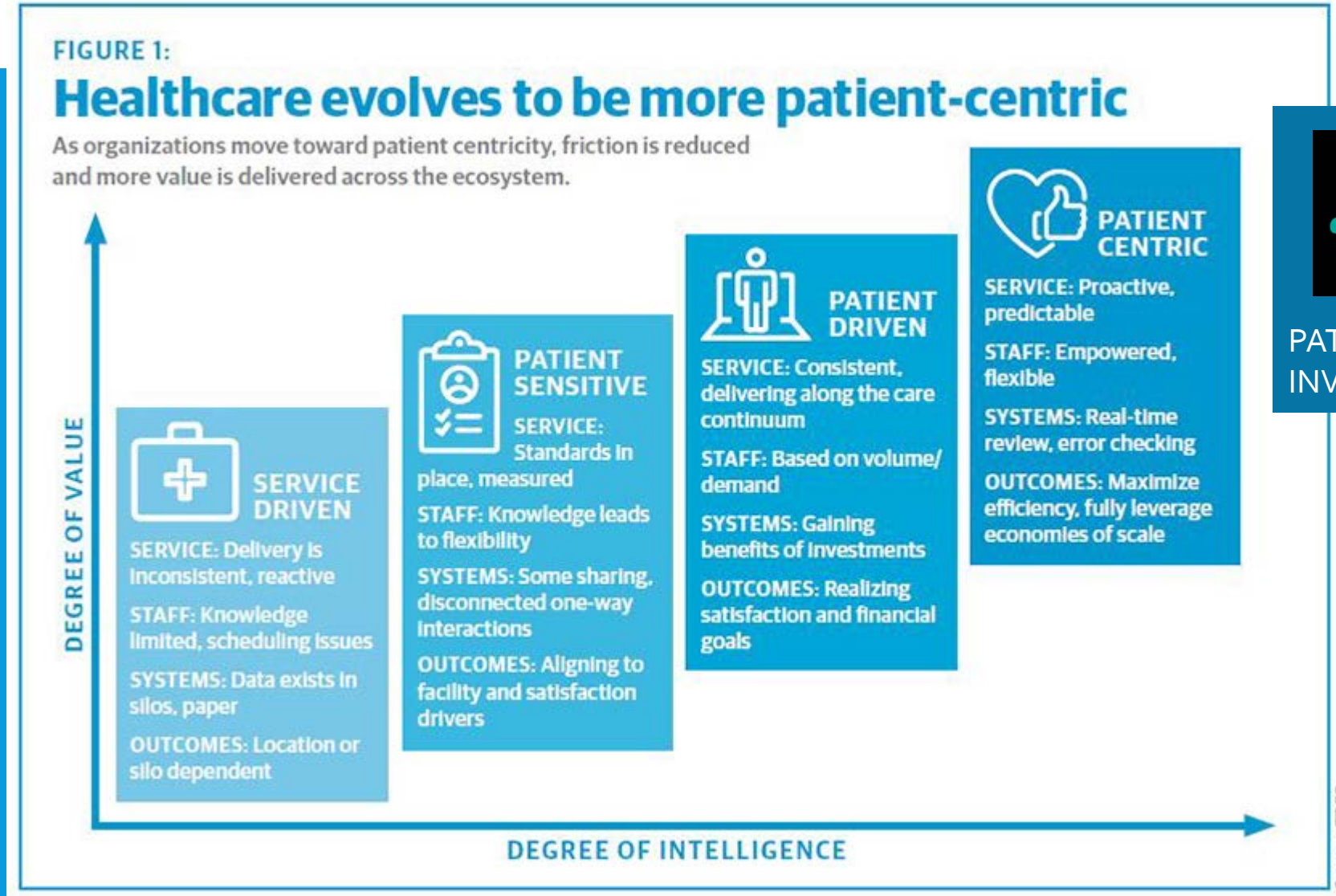
http://www.valuebasedcancer.com/issue-archive/2015/december-2015-vol-6-no-11/value-based-strategies-for-patients-with-multiple-myeloma-dec15/

SELECTED PRINCIPLES OF VALUE-BASED COMPETITION

- Refocus on the value for patients rather than lowering costs consistently at all levels within the healthcare institution
- Competition is driven by results, i.e., favor processes which demonstrate improved value of the care delivered
- Reduce cost of high-quality care
- Competition should expand from local areas to regional and national settings
- Provide transparency and accessibility of value-based results from all participants in value-based care
- Reward and endorse innovations that better the value of care provided for a medical condition

Johansen N. Value-Based Care in the Worldwide Battle Against Cancer, Cureus. 2017 Feb; 9(2): e1039 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5356991/

PATIENT'S CENTRIC APPROACH



https://www.ttec.com/articles/customer-focused-injection-patient-experience

Home - Myeloma Patients Euro

https://www.mpeurope.org

Przejdź do strony startowej

MPE Myeloma Patients Europe

Contact >

EMPOWERING MYELOMA ADVOCACY ACROSS EUROPE

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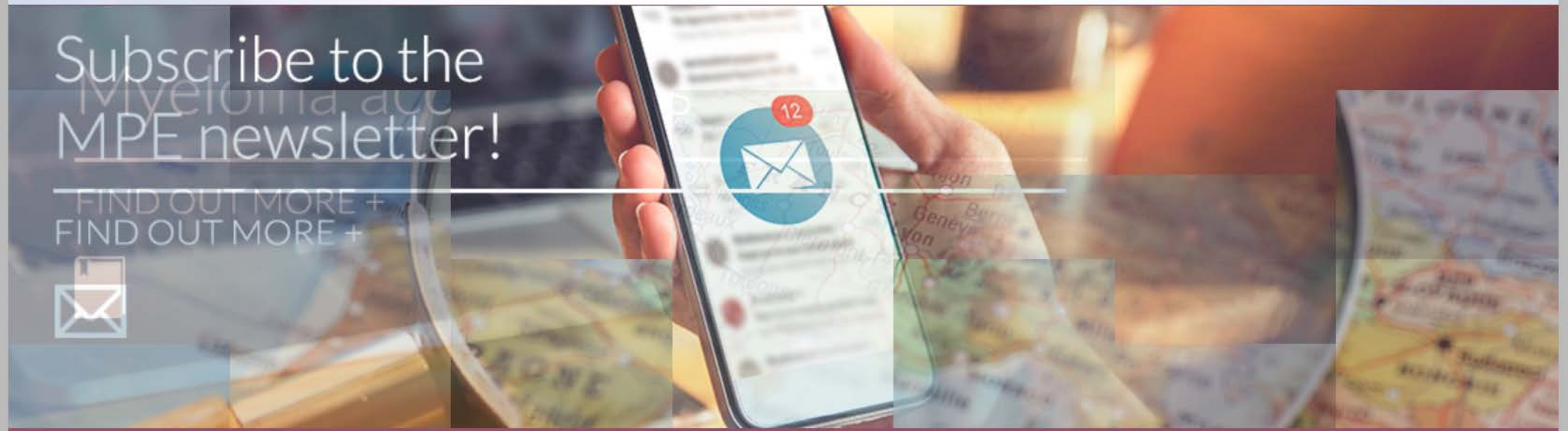

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Welcome to the Myeloma Patients

Europe - Lymphoma Coalition

https://www.lymphomacoalition.org/europe

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GLOBAL DATABASE

Information by country, region and/or subtype on treatments, clinical trials and incidence/mortality rates.

RESOURCE LIBRARY

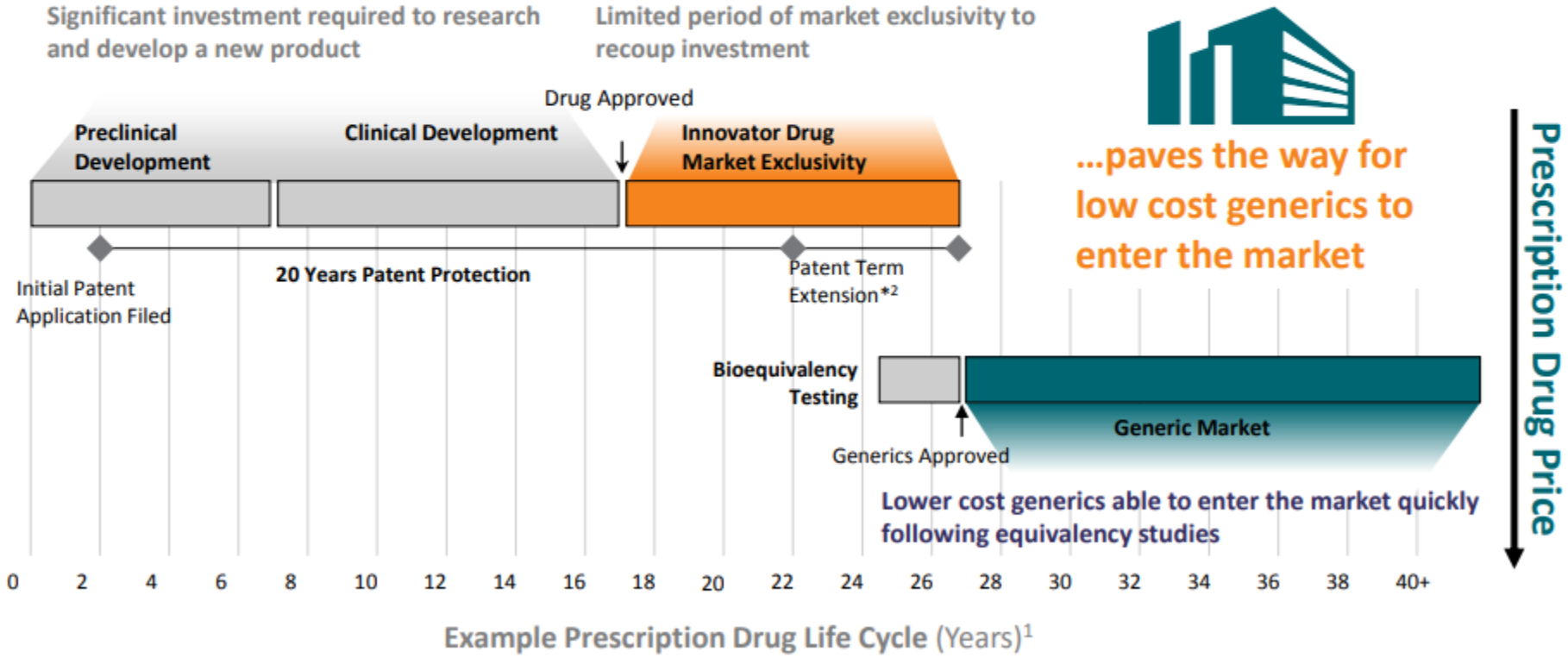
Over 2,000 pieces of information on lymphoma diagnosis, treatment, research, support and lifestyle.

LYMPHOMA COALITION
Worldwide Network of Lymphoma Patient Groups

The prescription drug life cycle enables **Low cost generic medicines to enter the market**

http://aipm.hu/data/upload/files/rendezvenyek/From%20innovation%20to%20outcomes,%20medicines%20cost%20in%20context.pdf

Initial investment in innovation by Biopharmaceutical companies...



Example Prescription Drug Life Cycle (Years)[1]

Possible to get additional exclusivity through the Hatch-Waxman Act which allows innovator companies to recoup up to half of time spent on clinical development and NDA filing to extend the effective patent life by up to 5 years. The total market exclusivity period cannot exceed 14 years.

Source: Health Advances analysis; [1]Shetty U 2013 Patents & Market Exclusivity Drugregulations.org; [2]Bitlaw 35 U.S.C. 156: Extension Of Patent Term November 2015.