

# Percorsi di Oncologia di Precisione:

## Appropriatezza diagnostica e Molecular Tumor Board

**30 GENNAIO 2026**  
**MILANO**

INNSiDE by Meliá Milano Torre Galfa  
Via Gustavo Fara, 41

Medicina basata sull'evidenza e evidenze  
derivate dalla medicina: cosa sappiamo?

**Pierfranco Conte**

*President, Periplo Foundation*

*Scientific Director, S Camillo Hospital, IRCCS*

*Senior Professor in Oncology, University of Padova*



# PierFranco Conte

## Disclosure of potential conflicts of interests (last 3 years)

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- **Consultant:**  
Novartis, Astra Zeneca, Daiichi-Sankyo, Gilead, Reveal Genomics
- **Honoraria:**  
Roche, EliLilly, Novartis
- **Research Funding from profit organizations:**  
Novartis, Astra Zeneca, Merck-KGa
- **Founder & Chairman:**  
Periplo Foundation
- **Patents:**  
Co-inventor: HER2Dx GEP for HER2+ eBC  
Co-inventor: Trail producing genetically engineered autologous stromal progenitor cells

# RWD & RWE: what can tell us more than EBM ?

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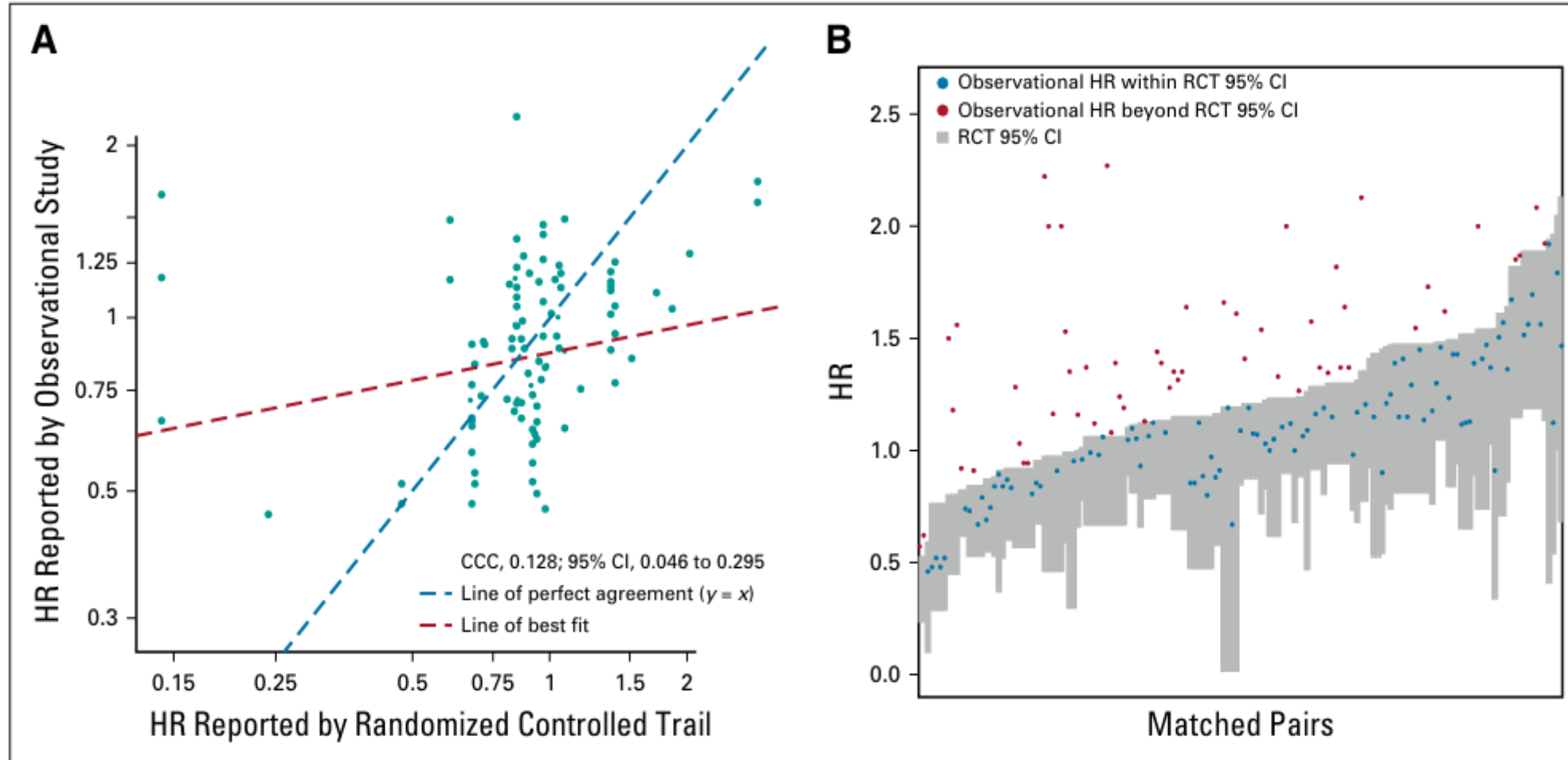
- **EBM as a faithful representation of reality**
  - Observational studies and Disease Registries confirm an improved OS over time for many tumor types

**BUT....**
- Effectiveness vs Efficacy
- Real World Data: not an easy task.....

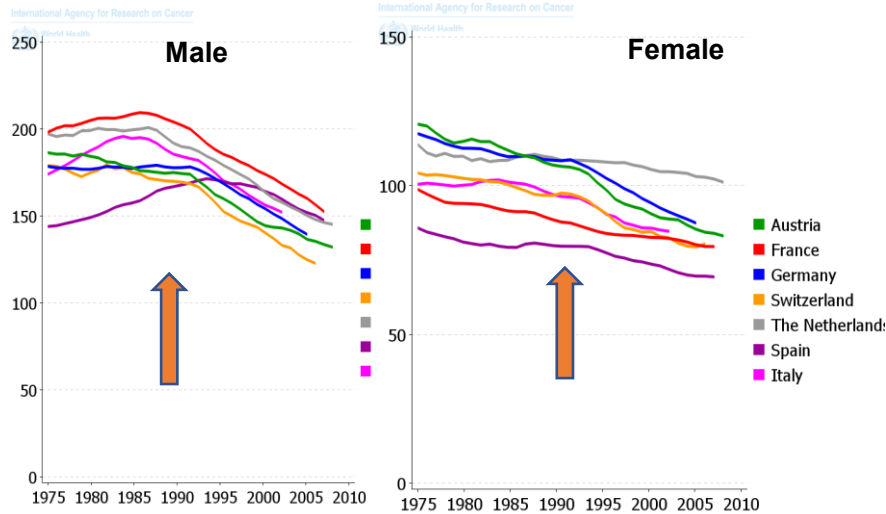
# Comparison of Population-Based Observational Studies With Randomized Trials in Oncology

Payal D. Soni, MD<sup>1</sup>; Holly E. Hartman, MS<sup>2</sup>; Robert T. Doss, MD<sup>2</sup>; Ahmed Abugharib, MD<sup>3</sup>; Steven G. Allen, PhD<sup>2</sup>; Felix Y. Feng, MD<sup>4</sup>; Anthony L. Zietman, MD<sup>5</sup>; Reshma Jagsi, MD, DPhil<sup>2</sup>; Matthew J. Schipper, PhD<sup>2</sup>; and Daniel E. Spratt, MD<sup>2</sup>

JCO 37: 1209-17, 2019



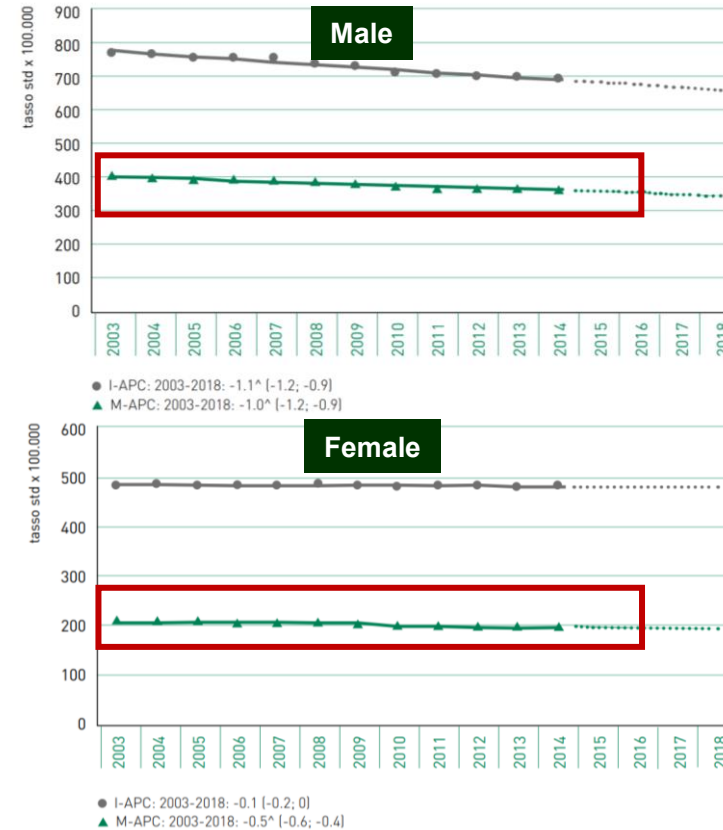
## Trends in mortality from cancer in Europe & Italy: age-standardised rate (W) per 100,000



Death rates started to decline sharply in early (female) and late (male) 80'.

### Main reasons for cancer death decline

- 1) Reduction in cigarette smoking
- 2) Healthy diet & refrigerator
- 3) Vaccination (HBV, HPV) & retroviral drugs
- 4) Screening & Early Diagnosis



No acceleration in death rate decline since early 2000'.

## Gains in life expectancy from decreasing cardiovascular disease and cancer mortality – an analysis of 28 European countries 1995–2019

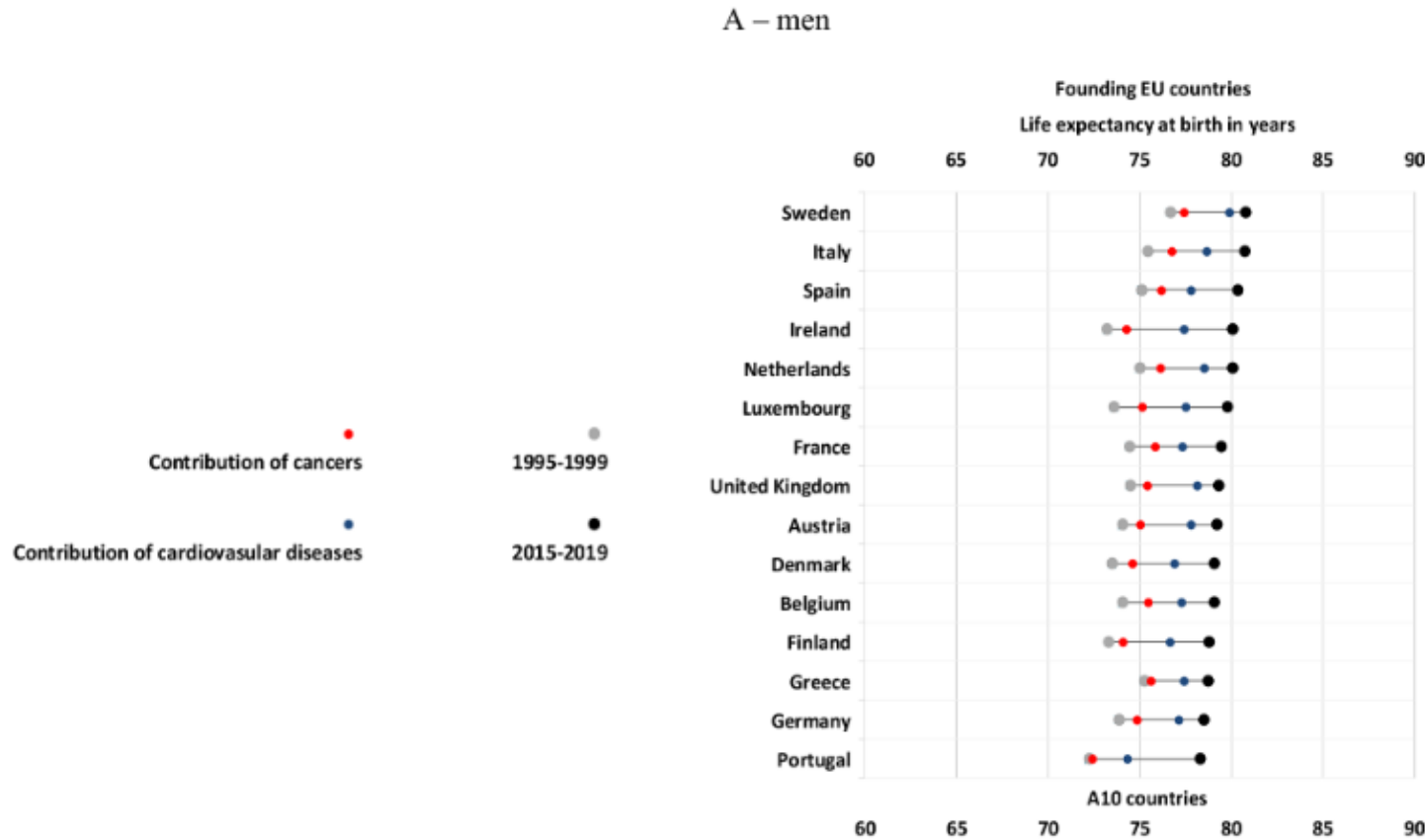
András Wéber<sup>1,2</sup> · Mathieu Laversanne<sup>1</sup> · Péter Nagy<sup>3,4,5</sup> · István Kenessey<sup>2,6</sup> · Isabelle Soerjomataram<sup>1</sup> · Freddie Bray<sup>1</sup>

Received: 17 April 2023 / Accepted: 2 August 2023  
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**Men:**  
2.26 years gained by CVD declines versus 1.07 years for cancer

**Women:**  
1.81 years gained by CVD declines versus 0.54 years for cancer

### Gains in life expectancy from decreasing cardiovascular disease and cancer mortality – an analysis of 28–...



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- **Real World Data: not an easy task.....**

**EBM is largely based on RCTs**  
**RCTs are in large part conducted to apply for marketing authorization**  
**Regulatory Agencies decide on the basis of the risk/benefit ratio**  
**This judgement does not take into account:**

- under-represented patients (elderly, unfit, with comorbidities or comedications)
- treatment duration (e.g. ICIs, antiHER2 drugs)
- treatment sequences
- rare & long term toxicities
- impact on diagnostic-therapeutic pathways
- budget impact

**Large proportion of new treatments only show a globally modest efficacy within RCTs**



Effect in clinical practice might be further diluted



Real value of results may fall under an acceptable threshold of relevance



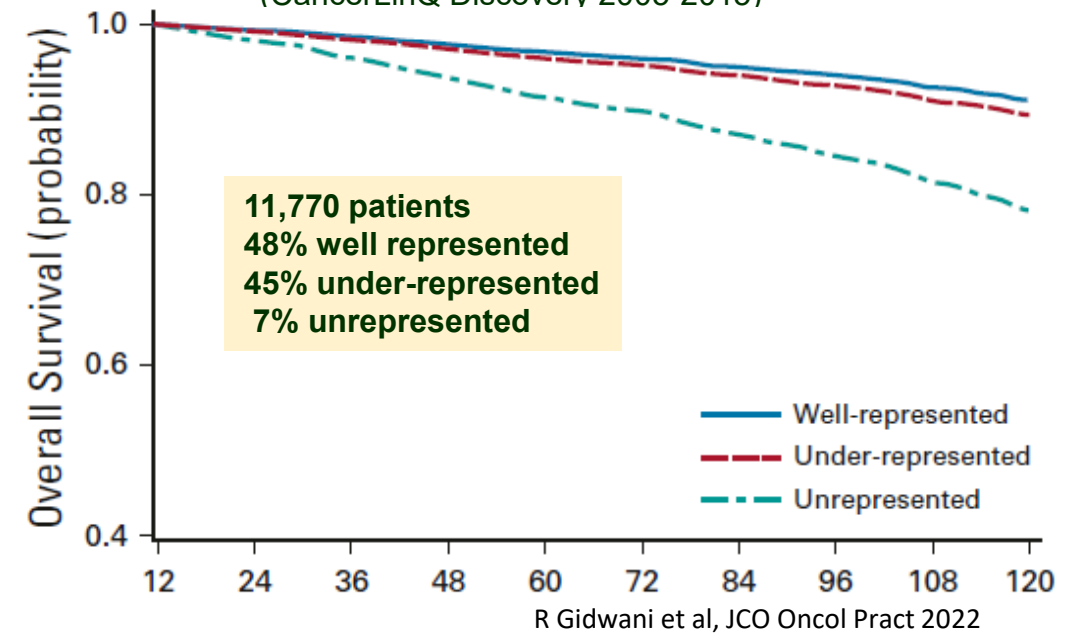
Post marketing studies could be useful to **confirm or refute the drug's benefit** on survival in real-world populations



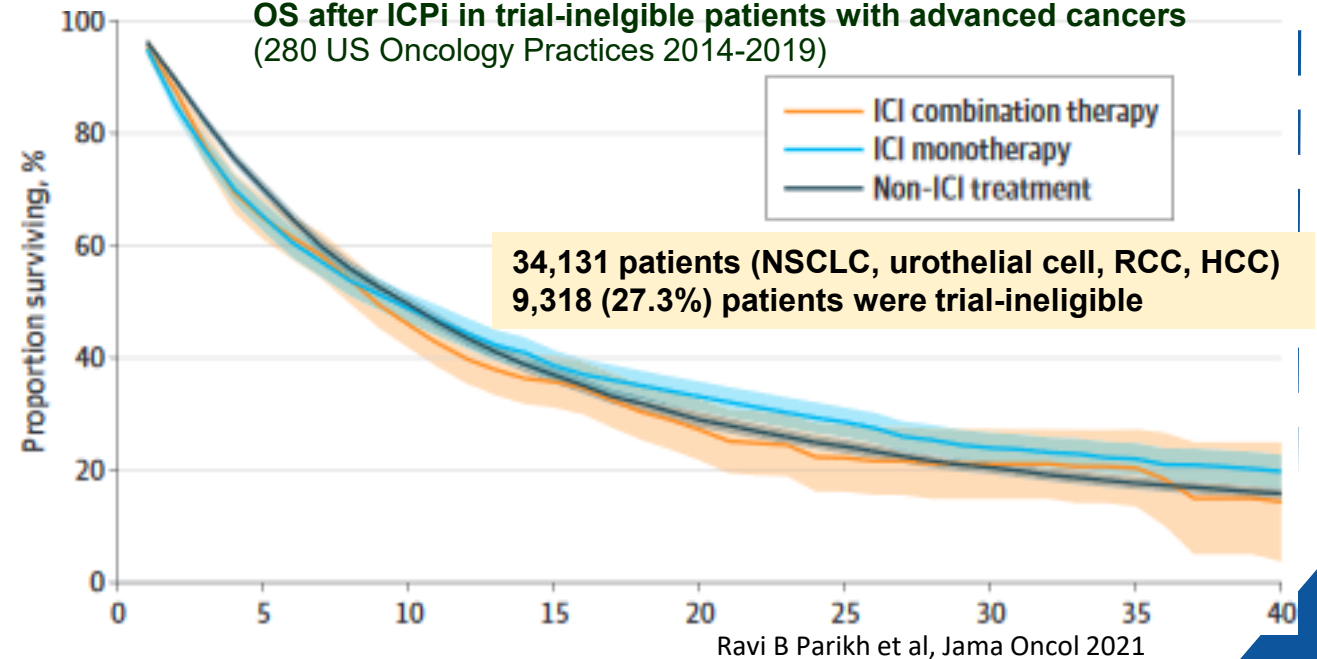
**RWE analysis may challenge the magnitude of the efficacy previously shown in RCTs**

Di Maio, Perrone, Conte;The Oncologist 2020

**eBC in Real World: OS by trial representation**  
 (CancerLinQ Discovery 2005-2015)



**OS after ICPI in trial-ineligible patients with advanced cancers**  
 (280 US Oncology Practices 2014-2019)



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- Real World Data: not an easy task.....

**Next-generation sequencing for guiding matched targeted therapies in people with relapsed or metastatic cancer (Review)**

Kazmi F, Shrestha N, Liu TF, Foord T, Heesen P, Booth S, Dodwell D, Lord S, Yeoh K-W, Blagden SP.  
*Cochrane Database of Systematic Reviews* 2025, Issue 3. Art. No.: CD014872.  
 DOI: [10.1002/14651858.CD014872.pub2](https://doi.org/10.1002/14651858.CD014872.pub2).

**RCTs of matched targeted therapy**  
**35 trials**  
**9.819 patients**  
**Outcomes: PFS, OS, ORR, G3/4 Aes, QoL**

**Summary of findings 1. Summary of findings table - Matched targeted therapy compared to standard-of-care treatment in adults (aged 18 and over) with confirmed evidence of unresectable, metastatic or refractory cancers and progression after at least one line of standard systemic anti-cancer therapy**

Outcomes	Anticipated absolute effects* (95% CI)		Relative effect (95% CI)	Nº of participants (studies)	Certainty of the evidence (GRADE)	Comments
	Risk with standard-of-care treatment	Risk with matched targeted therapy				
Progression-free survival follow-up: median 6 months	Moderate 580 per 1000	<b>698 per 1000</b> (668 to 725)	<b>HR 0.66</b> (0.59 to 0.74) [Progression] <sup>1,a</sup>	3848 (14 RCTs)	⊕⊕⊕⊙ Moderate <sup>b,c</sup>	Matched targeted therapy probably increases progression-free survival.
Overall survival follow-up: median 12 months	Moderate 820 per 1000	<b>845 per 1000</b> (825 to 862)	<b>HR 0.85</b> (0.75 to 0.97) [Death] <sup>1,a</sup>	3848 (14 RCTs)	⊕⊕⊕⊙ Low <sup>b,d,e</sup>	Matched targeted therapy may result in little to no difference in overall survival.
Overall response rate	210 per 1000	<b>445 per 1000</b> (384 to 508)	<b>OR 3.01</b> (2.34 to 3.89)	2872 (13 RCTs)	⊕⊕⊕⊙ Low <sup>b,f,g</sup>	Matched targeted therapy may result in an increase in overall response rate.
Severe adverse events follow-up: range 1.4 months to 13.8 months <sup>h</sup>	514 per 1000	<b>673 per 1000</b> (591 to 770)	<b>RR 1.31</b> (1.15 to 1.50)	2552 (9 RCTs)	⊕⊕⊕⊙ Low <sup>b,i</sup>	Matched targeted therapy may result in little to no difference in severe adverse events.
Quality of life (QoL) assessed with: FACT-G/ FACT-O follow-up: range 2 months to 6.2 months <sup>j</sup>	-	<b>SMD 0.06 SD higher</b> (0.08 lower to 0.19 higher) <sup>2,k</sup>	-	944 (3 RCTs)	⊕⊕⊕⊙ Very low <sup>b,l</sup>	Matched targeted therapy may result in little to no difference in severe adverse events.



# Innovation from Trials might be a Vision in Real World....

## Vision



Leonardo da Vinci  
1452-1519

There is a real progress only when the benefits of a new technology become available to everyone

*Henry Ford*

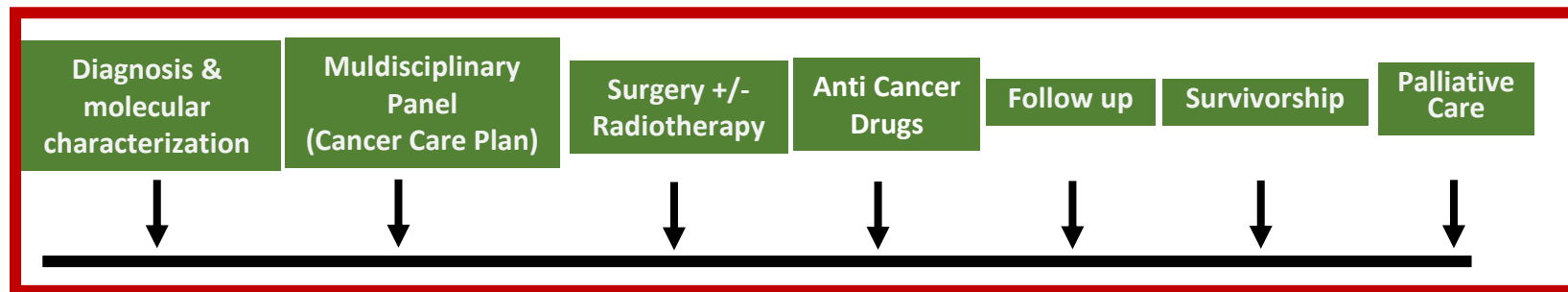
## Innovation



Wilbur Wright  
1867-1912



Orville Wright  
1871-1948



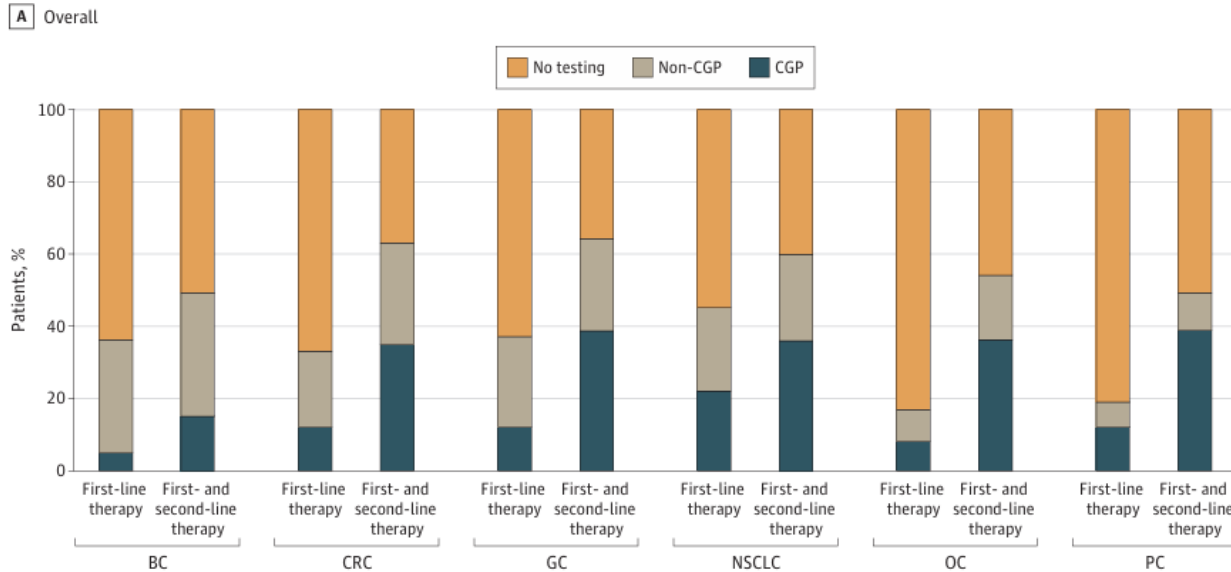
## Biomarker Testing Approaches, Treatment Selection, and Cost of Care Among Adults With Advanced Cancer

Stacey DaCosta Byfield, PhD, MPH; Bela Bapat, MS; Laura Becker, MS; Carolina Reyes, PhD; Ismini Chatzitheofilou, MS; Brock E. Schroeder, PhD; Damon Hostin, MA; John Fox, MD, MHA

Jama Network Open 2025

**Optum Labs Data Warehouse**  
**26,311 patients with advanced cancers**  
**diagnosed from jan 2018 to jan 2022**

Biomarker Testing Rates Before First- and Second-Line Therapy



**A-BRAVE TRIAL: A PHASE III RANDOMIZED TRIAL WITH AVELUMAB IN EARLY TRIPLE NEGATIVE BREAST CANCER WITH RESIDUAL DISEASE AFTER NEOADJUVANT CHEMOTHERAPY OR AT HIGH RISK AFTER PRIMARY SURGERY AND ADJUVANT CHEMOTHERAPY**

**477 patients from 67 institutions**  
**Accrual from june 2016 to october 2020**  
**BRCA status known in 284 patients (59.5%)**

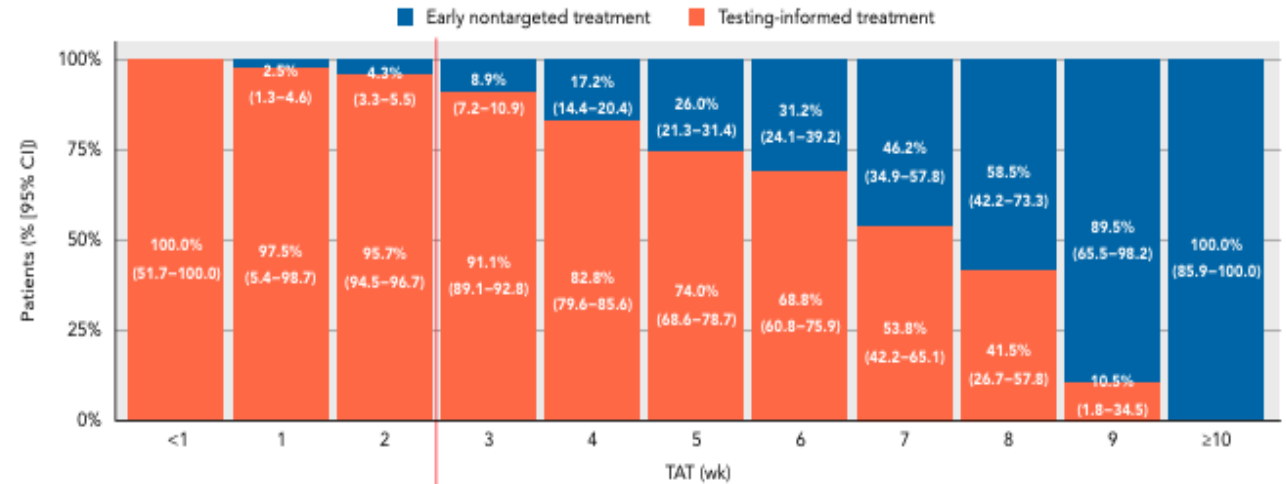
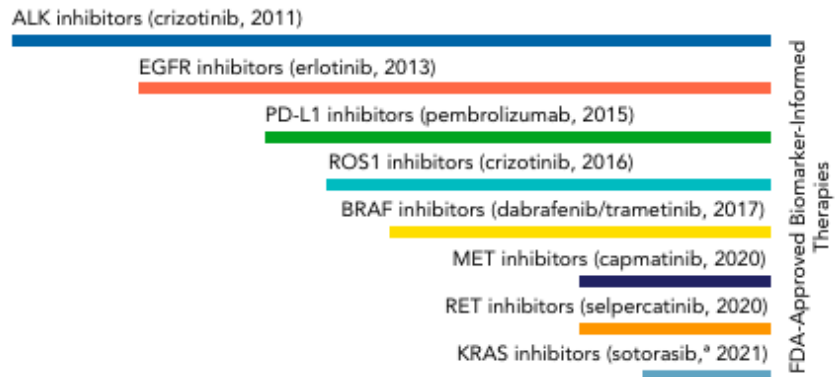
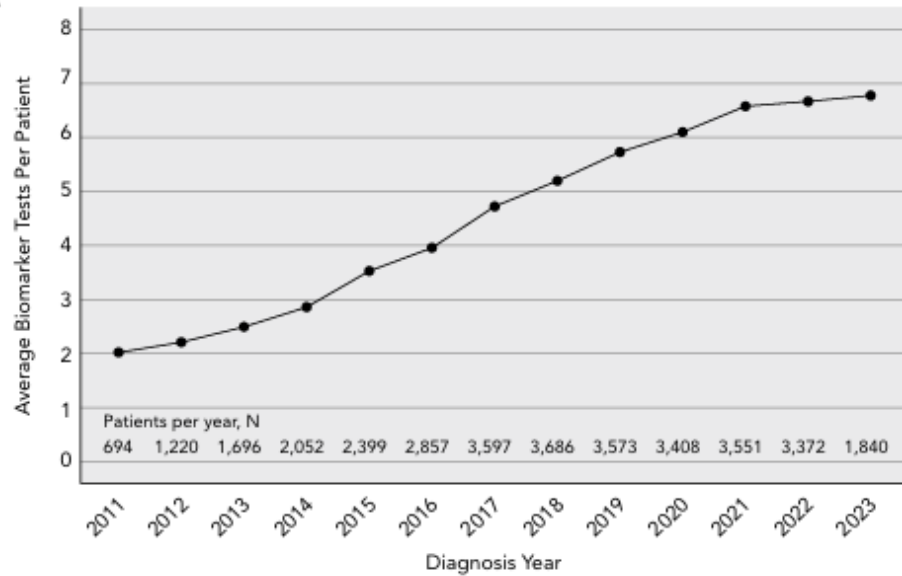
Conte P et al, Ann Oncol 2025

# Molecular Biomarker Testing Patterns and Turnaround Time in US Patients With Advanced Non-Small Cell Lung Cancer

Xiao Wang, MD<sup>1,2</sup>; Do H. Lee, MPH<sup>2</sup>; Szu-Chun Yang, MD, PhD<sup>3,4</sup>; Yimeng Li, MPH<sup>3</sup>; Pamela R. Soulos, MPH<sup>2</sup>; Cary P. Gross, MD<sup>2,3,5</sup>; Shi-Yi Wang, MD, PhD<sup>2,3</sup>; and Anne C. Chiang, MD, PhD<sup>1</sup>

J Natl Compr Canc Netw 2025;23(11):461-467  
doi:10.6004/jnccn.2025.7063

**33,945 aNSCLC patients from 2011 to 2023 in Flatiron Data Base**  
Recommended turnaround time (TAT) for ALK/EGF testing: 2 weeks.



	<1	1	2	3	4	5	6	7	8	9	≥10
<b>All ALK+/EGFR+ aNSCLC</b> N=4,020		436	1,376	935	634	311	154	78	41	*	*
Row %		10.8	34.2	23.3	15.8	7.7	3.8	1.9	1.0	*	*
<b>Early nontargeted treatment</b> n=498		11	59	83	109	81	48	36	24	*	*
Row %		2.2	11.8	16.7	21.9	16.3	9.6	7.2	4.8	*	*
<b>Testing-informed treatment</b> n=3,522		425	1,317	852	525	230	106	42	17	*	*
Row %		12.1	37.4	24.2	14.9	6.5	3.0	1.2	0.5	*	*

← 45.2% (at TAT=2)      54.8% → (at TAT=3)  
← 14.1% (at TAT=2)      85.9% → (at TAT=3)  
← 49.6% (at TAT=2)      50.4% → (at TAT=3)

# Timeliness of Newer Targeted Therapy and Survival in Lung Cancer: A Population-Based Analysis

Tawee Tanvetyanon, MD, MPH<sup>1</sup> ; Utsav Joshi, MD<sup>1</sup> ; Dung-Tsa Chen, PhD<sup>1</sup>; and Jhanelle E. Gray, MD<sup>1</sup>

JCO Oncol Pract 2025

EHR-derived data from 05/2014 to 03/2023  
857 Advanced NSCLC with BRAF/MET/NTRK/ROS1/RET alterations  
Independent prognostic factor for OS: TTI, PS, squamous histology

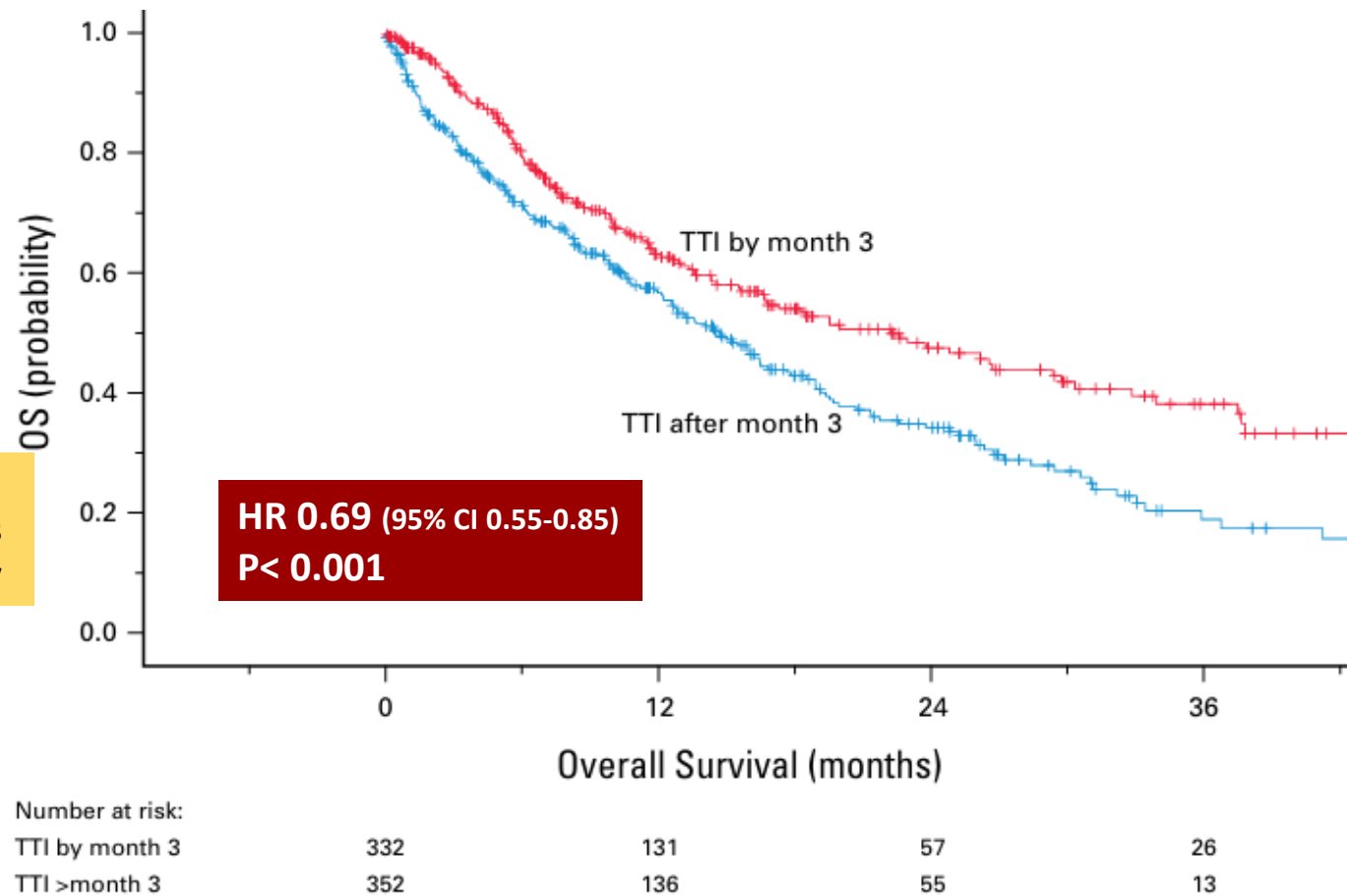


FIG 1. Overall survival from 3-month landmark time point. TTI, time to treatment initiation.

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- More molecular biomarkers to be tested, more prolonged TAT, more patients treated with less effective therapy

- Real World Data: not an easy task.....

# Real World Data: mission impossible?

## The Italian health data system is broken

A major weakness of the health-care system in Italy is the fragmented health data infrastructure: there is no unified, centralised system for documenting and sharing electronic health records (EHRs), hospital data, and general practitioner records.

The Lancet Regional  
Health - Europe  
2025;48: 101206  
<https://doi.org/10.1016/j.lanepe.2024.101206>

## The Promise of Real-World Data for Research — What Are We Missing?

Ali B. Abbasi, M.D.,<sup>1</sup> Lesley H. Curtis, Ph.D.,<sup>2</sup> and Robert M. Califf, M.D.<sup>2</sup>

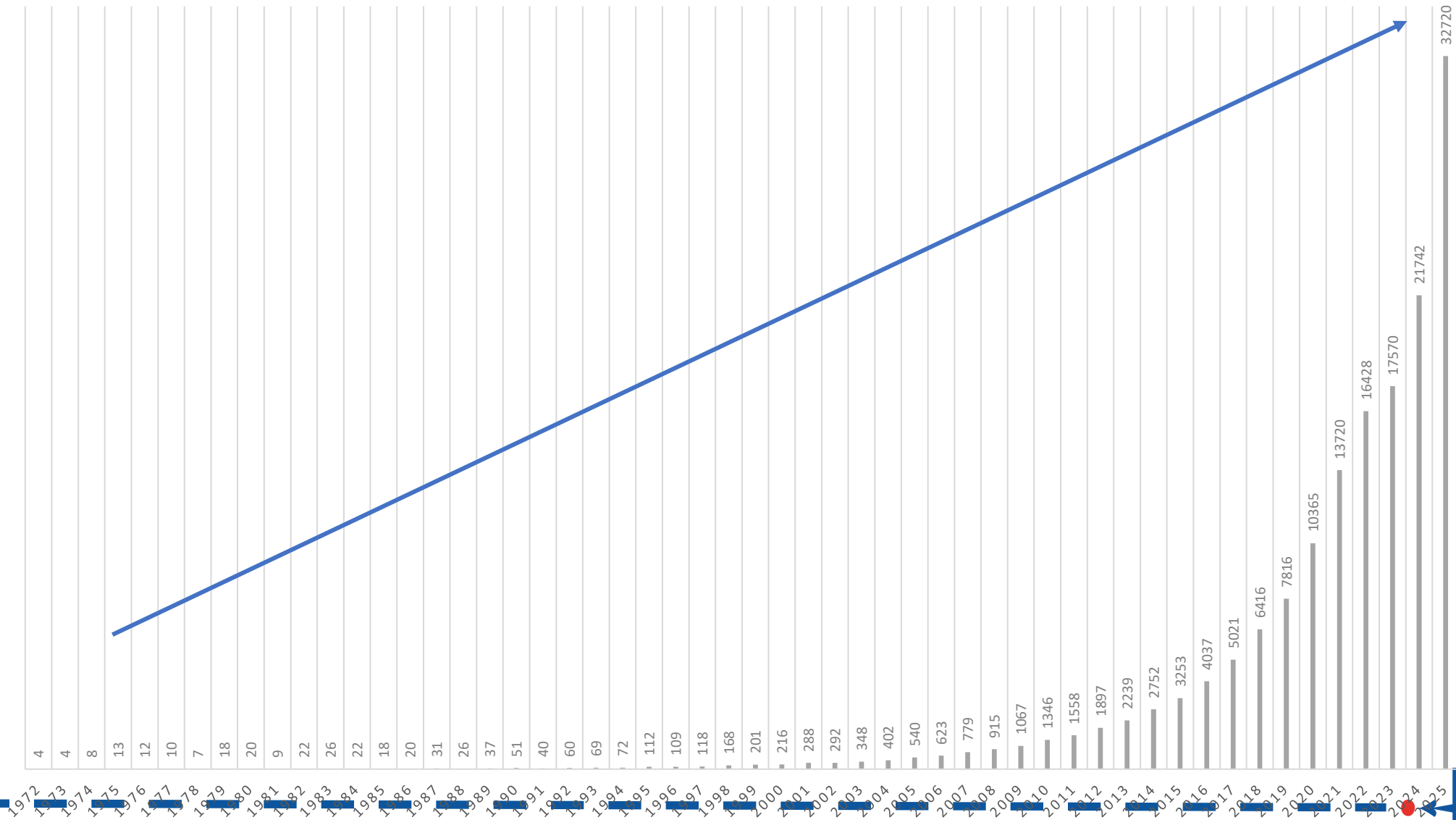
«But although 96% of US hospitals have adopted EHRs, according to federal data, these systems were designed to document individual episodes of care and facilitate the highest possible payments for that care, not to support clinical research.»

NEJM 393: 318-321, 2025

### BARRIERS to good quality Real World Data

- Access to regional/national Data Bases
- Data availability (i.e. molecular characteristics/mutational profiles; staging)
- Set of Data (i.e. proportion of patients; attrition rate)
- Quality of Data (EHR, source of data and data verification)
- Data interpretation (comparative effectiveness)
- Funding

# PUBMED: "REAL-WORLD" OR "REAL WORLD"



# Milestones in the FDA's Real-World Evidence Activities



**Real-world data (RWD)** are data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources. Examples of RWD include data derived from electronic health records, medical claims data, data from product or disease registries, and data gathered from other sources (such as digital health technologies) that can inform on health status.

**Real-world evidence (RWE)** is the clinical evidence about the usage and potential benefits or risks of a medical product derived from analysis of RWD.



<https://www.evidencebaseonline.com/>

As announced in the Federal Register notice published on October 20, 2022, FDA is conducting an **Advancing Real-World Evidence (RWE) Program**, which seeks to improve the quality and acceptability of RWE-based approaches in support of new intended labeling claims, including approval of new indications of approved medical products, or to satisfy post-approval study requirements.

“It’s almost as if things that were dreamed about 30 years ago are now gradually coming true and may hit a tipping point soon where things will really take off.”

R Califf, FDA Commissioner



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

17 March 2025  
EMA/99865/2025  
Committee for Human Medicine Products/  
Methodology Working Party (CHMP/MWP)

Reflection paper on use of real-world data in non-interventional studies to generate real-world evidence for regulatory purposes

Draft agreed by Methodology Working Party (MWP)	October 2023
Adopted by CHMP PROM for release for consultation	15 April 2024
Start of public consultation	3 May 2024
End of consultation (deadline for comments)	31 August 2024
Agreed by Methodology Working Party (MWP)	March 2025
Adopted by CHMP PROM	17 March 2025

Keywords	Non-interventional study, real-world data, real-world evidence, feasibility assessment, bias, confounding, data quality
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## 2022 ASCO Annual Meeting Education Session “Point/Counterpoint: Real-World Data—Do We Even Need Clinical Trials Anymore?”.

Faculty include moderator Safiya Karim, MD, MSc, of Tom Baker Cancer Centre at the University of Calgary, in Canada; presenter Paul G. Kluetz, MD, of the U.S. Food and Drug Administration (FDA); and presenter Lawrence H. Kushi, ScD, of Kaiser Permanente Northern California Division of Research.



Dear Dr. Conte,

I am writing on behalf of the 2026 Program Committee Chairs, Drs. Paul S. Mischel and Alice T. Shaw, and the American Association for Cancer Research (AACR) President, Dr. Lillian L. Siu to invite you to serve as **Chair** of the Real-World Data and Real-World Evidence Section of the CL09: **Real-World Data (RWD) and Real-World Evidence (RWE) Subcommittee of the Program Committee for the AACR Annual Meeting 2026** taking place in San Diego, CA, from April 17-22, 2026.

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- **Real World Data: not an easy task.....BUT....**

- It can be done!

- Growing interest from scientific journals, scientific societies and regulatory agencies

- **Real World Data: Periplo vision and mission**

# Main sources of Real World Data in Oncology Publications

**SEER:** the registries cover 45.9% of US population and routinely collect data on patient demographics, primary tumor site, tumor morphology and stage at diagnosis, first course of treatment and follow-up for vital status.

**CancerLinQ:** the ASCO community comprises more than 100 oncology practices, hospitals and health systems, representing more than 2,000 oncologists nationwide. When a practice participates in CancerLinQ, treatment information about their patients is securely transferred from the practice's electronic health record (EHR) and other data systems into the secure CancerLinQ platform.

**Private Health Insurance Companies:** insurance companies (Blue Cross&Blue Shield Health Insurance, Kaiser Permanente, United Health Care, Cigna) provide RWD on their insured patients

**Flatiron Health:** Roche-owned company; collect disease specific real world data from EHRs for 22 diseases from 280 oncology practices (75% community, 25% academic oncology centers)

**These data, although generally of good quality, are subject to biases:**

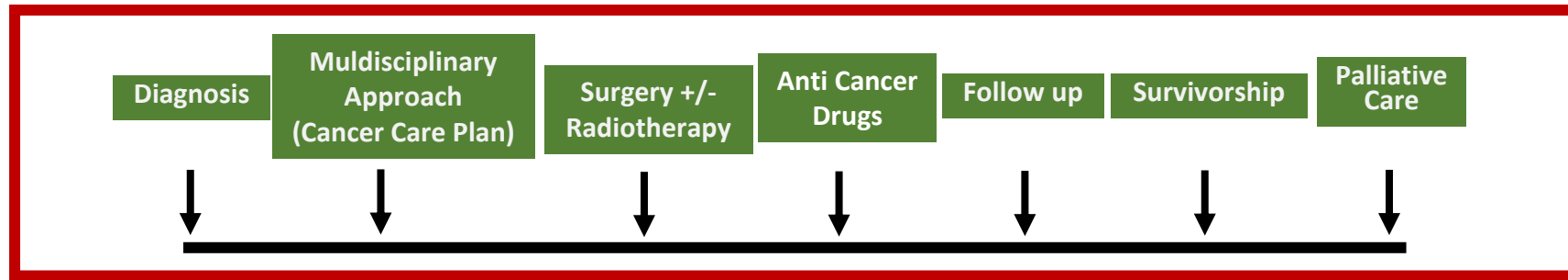
- only part of patients' journey is captured (SEER)
- patients from participants oncologists only (CancerLinQ)
- patients covered by private insurance plans only (Insurance companies)
- largely based on US patients (Flatiron Health); recent agreements in UK, Germany, Japan
- differences in baseline patients characteristics between cancer registries and Flatiron Health
- (X Ma et al, *medRxiv*. 2023. doi: <https://doi.org/10.1101/2020.03.16.20037143>)

# The NHS: a key player in innovation & sustainability

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- **Silo-based health care funding**
- Innovation & diagnostic-therapeutic pathways

# A unique opportunity for the NHS: tracking indicators of the entire patients' journey



## Sources of Data

### Pathology Reports

Histopathology and cytology reports

### Additional Administrative Data

Hos

**Data links and interpretation**  
a multidisciplinary team including clinicians, pathologists, pharmacists, epidemiologists, statisticians, health economists

Death Certificates

Emergency departments

# The Budget Impact Conundrum

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- **Budget Impact (BI) is a key element of the reimbursement process**
- **Determinants of BI are:**
  - negotiated price of drug
  - size of eligible patient population
  - treatment duration

# Analysis of Italian BRCA1/2 Pathogenic Variants Identifies a Private Spectrum in the Population from the Bergamo Province in Northern Italy

Gisella Figlioli<sup>1</sup>, Arcangelo De Nicolo<sup>2</sup>, Irene Catucci<sup>1</sup>, Siranoush Manoukian<sup>3</sup>, Bernard Peissel<sup>3</sup>,  
 Jacopo Azzollini<sup>3</sup>, Benedetta Beltrami<sup>3</sup>, Bernardo Bonanni<sup>4</sup>, Mariarosaria Calvello<sup>4</sup>, Davide Bondavalli<sup>4</sup>,  
 Barbara Pasini<sup>5,6</sup>, Francesca Vignolo Lutati<sup>5</sup>, Paola Ogliastra<sup>6</sup>, Monica Zuradelli<sup>7</sup>, Valeria Pensotti<sup>8</sup>,  
 Giovanna De Vecchi<sup>8</sup>, Sara Volorio<sup>8</sup>, Paolo Verderio<sup>9</sup>, Sara Pizzamiglio<sup>9</sup>, Giuseppe Matullo<sup>5,6</sup>,  
 Serena Anelli<sup>5</sup>, Giovanni Birolo<sup>5</sup>, Federica Zanardi<sup>10</sup>, Carlo Tondini<sup>11</sup>, Alberto Zambelli<sup>11</sup>,  
 Luca Livraghi<sup>11,12</sup>, Michela Franchi<sup>11</sup>, Paolo Radice<sup>13</sup> and Paolo Peterlongo<sup>1,\*,†</sup>

Cancers 13:532, 2021

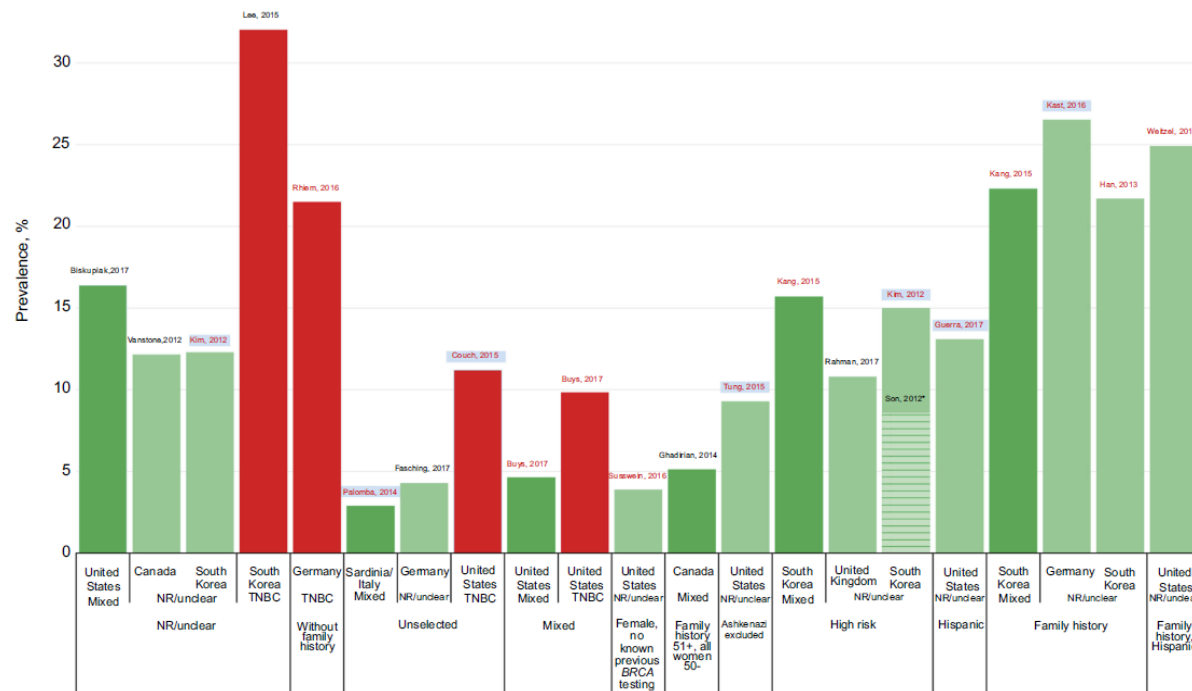
Region	Region of Birth of Carrier Ascertained in				Number of Residents #	Rate * (1:100,000)
	All Cities	Milan (INT, IEO, ICH)	Turin (ACSS)	Bergamo (OPG)		
Lombardy (Non-BGP)	293	281	3	9	8,987,585	3.3
BGP	100	29	0	71	1,116,384	9.0
Lazio	20	18	2	0	5,865,544	0.3
Campania	40	32	7	1	5,785,861	0.7
Sicily	63	50	9	4	4,968,410	1.3
Veneto	24	19	4	1	4,907,704	0.5
Emilia Romagna	41	35	5	1	4,467,118	0.9
Piedmont	167	43	124	0	4,341,375	3.9
Apulia	103	85	17	1	4,008,296	2.6
Tuscany	23	22	1	0	3,722,729	0.6
Calabria	39	27	12	0	1,924,701	2.0
Sardinia	22	17	3	2	1,630,474	1.4
Liguria	16	13	3	0	1,543,127	1.0
Marche	12	11	1	0	1,518,400	0.8
Abruzzo	13	10	3	0	1,305,770	1.0
Friuli Venezia Giulia	14	8	6	0	1,211,357	1.2
Trentino Alto Adige	9	9	0	0	1,074,819	0.8
Umbria	3	3	0	0	880,285	0.3
Basilicata	9	6	3	0	556,934	1.6
Molise	1	1	0	0	302,265	0.3
Aosta Valley	7	4	3	0	125,501	5.6
All regions (non-BGP)	919	694	206	19	59,128,255	1.6
All regions	1019	723	206	90	60,244,639	1.7

# Derived from <https://www.tuttitalia.it/regioni/popolazione/> on 31 December 2019. \* Rates were derived considering the number of BRCA1/2 PV carriers by region of birth and the number of residents in each region.

# A systematic review of the international prevalence of BRCA mutation in breast cancer

This article was published in the following Dove Press journal:  
 Clinical Epidemiology

Armstrong N et al, Clinical Epidemiology 11: 543-561, 2019

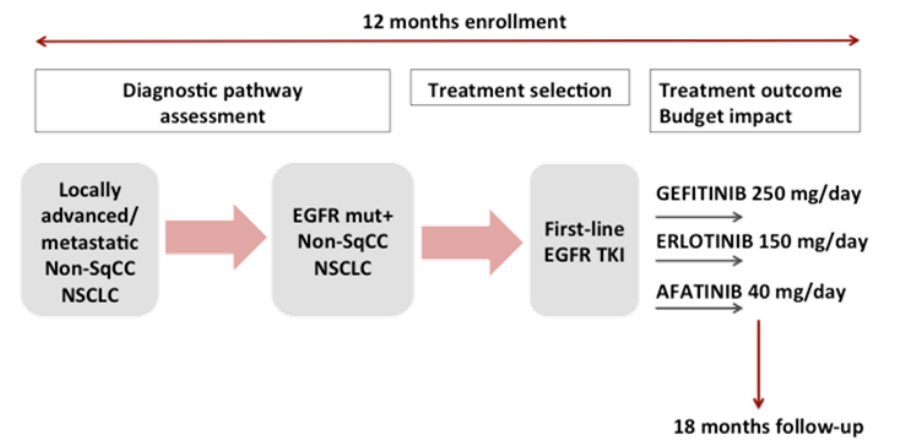


# From Diagnostic-Therapeutic Pathways to Real-World Data: A Multicenter Prospective Study on Upfront Treatment for EGFR-Positive Non-Small Cell Lung Cancer (MOST Study)

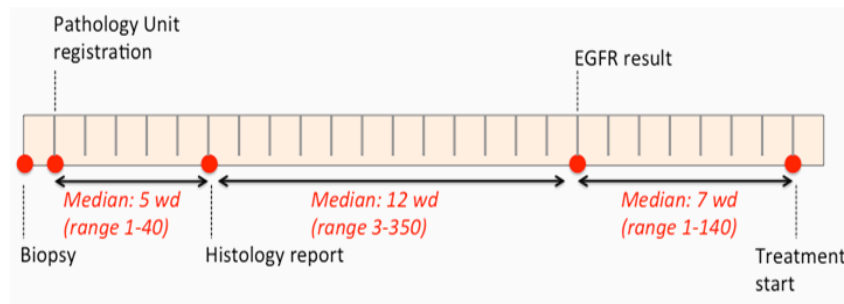
GIULIA PASELLO,<sup>a</sup> GIOVANNI VICARIO,<sup>b</sup> FABLE ZUSTOVICH,<sup>c</sup> FRANCESCO ONIGA,<sup>d</sup> STEFANIA GORI,<sup>e</sup> FRANCESCO MOETI,<sup>f</sup> ANDREA BONETTI,<sup>g</sup> ADOLFO FAVARETTO,<sup>h</sup> SILVIA TOSO,<sup>i</sup> ROBERTA REDELOTTI,<sup>j</sup> ANTONIO SANTO,<sup>k</sup> DANIELE BERNARDI,<sup>l</sup> PETROS GIOVANIS,<sup>m</sup> CRISTINA OLIANI,<sup>n</sup> LORENZO CALVETTI,<sup>o</sup> CARLO GATTI,<sup>p</sup> GIOVANNI PALAZZOLO,<sup>q</sup> ZORA BARETTA,<sup>r</sup> ALBERTO BORTOLAMI,<sup>a</sup> LAURA BONANNO,<sup>a</sup> MARCO PASSO,<sup>b</sup> JESSICA MENIS,<sup>a,r</sup> DONATELLA DA CORTE,<sup>c</sup> STEFANO FREGA,<sup>a</sup> VALENTINA GUARNERI,<sup>a,r</sup> PIERFRANCO CONTE,<sup>a,r</sup> ON BEHALF OF VENETO ONCOLOGY NETWORK

The Oncologist 2019)

Practice-Changing Article



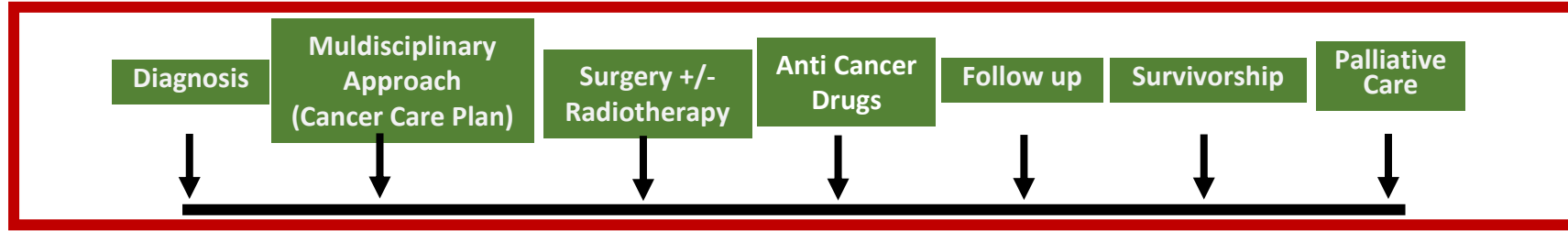
### Results: diagnostic pathway (benchmark 10 wd)



DRUG	MOST treatment interruption %	Pivotal trials treatment interruption %	MOST mTTF (mo.)	Pivotal trials mPFS (mo.)	MOST BI €	Pivotal trials BI €	BI Gap €
Gefitinib	9	14	14.6	9.5	2,000,949	1,234,754	+ 773,195
Erlotinib	NA	NA	22.9	9.7	735,013	291,991	+ 443,021
Afatinib	18	34	15.3	11.0	495,638	286,811	+ 208,827
TOTAL			15.3		3,238,602		+ 1,425,044

Patients with EGFRm NSCLC treated with TKIs in Veneto had fewer treatment interruptions and more prolonged duration of treatment than patients on trials. This resulted in a 78% higher BI than estimate by AIFA

# A unique opportunity for the NHS: tracking indicators of the entire patients' journey



## Thoracic Cancer

Thoracic Cancer ISSN 1759-7706

ORIGINAL ARTICLE

### Estimated direct costs of non-small cell lung cancer by stage at diagnosis and disease management phase: A whole-disease model

Alessandra Buja<sup>1</sup>, Michele Rivera<sup>1</sup>, Anna De Polo<sup>1</sup>, Eugenio di Brino<sup>5</sup>, Marco Marchetti<sup>6</sup>, Manuela Scioni<sup>2</sup>, Giulia Pasello<sup>4</sup>, Alberto Bortolami<sup>7</sup>, Vincenzo Rebba<sup>3</sup>, Marco Schiavon<sup>1</sup>, Fiorella Calabrese<sup>1</sup>, Giovanni Mandoliti<sup>5</sup>, Vincenzo Baldo<sup>1</sup> & PierFranco Conte<sup>4,8</sup>

**Table 3** Estimates of average (and confidence interval) per-patient costs of care for NSCLC by disease stage at diagnosis

Stage	Average total cost (€)	95% CI
Stage I	16 291	(95% CI: 15 291–17 291)
Stage II	19 530	(95% CI: 18 530–20 530)
Stage III	21 938	(95% CI: 20 938–22 938)
Stage IV	22 175	(95% CI: 22 127–22 190)
Pancoast	28 711	(95% CI: 27 711–29 890)
TOTAL	21 328	(95% CI: -20 897–22 322)

Total costs adjusted for age, stage at diagnosis, sex, cohort, at 2 yrs after cancer diagnosis

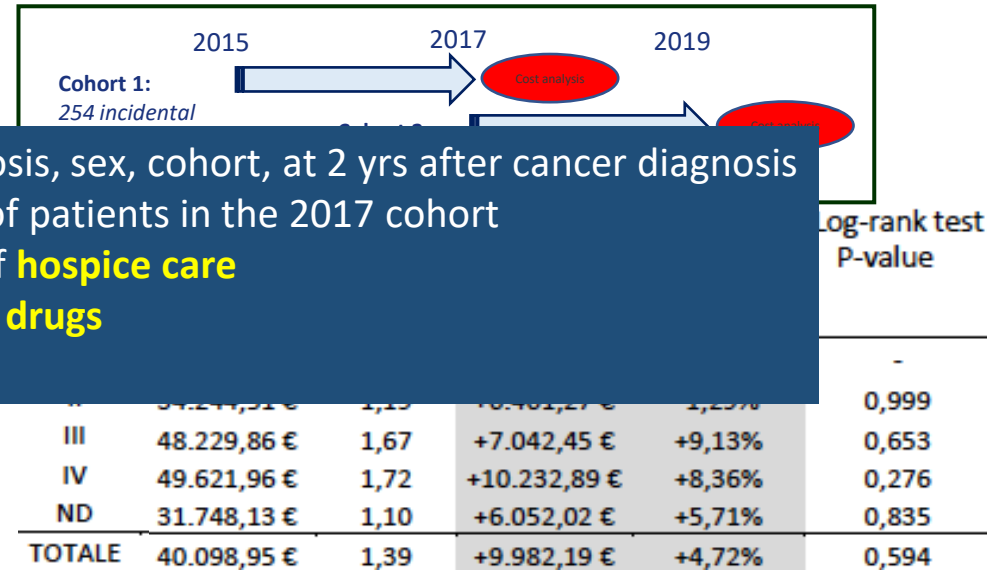
- significant **increase in the average costs** of patients in the 2017 cohort
- significant **decrease** in the average cost of **hospice care**
- significant **increase** in the average cost of **drugs**
- **Significant OS improvement at 2 yrs**

VALUE IN CANCER CARE ReCAP

## Non-Small-Cell Lung Cancer: Real-World Cost Consequence Analysis

Alessandra Buja, MD, PhD<sup>1</sup>; Giulia Pasello, MD<sup>2</sup>; Giuseppe De Luca, MD<sup>1</sup>; Alberto Bortolami, PharmD<sup>3</sup>; Manuel Zorzi, MD<sup>4</sup>; Federico Rea, MD<sup>1</sup>; Carlo Pinato, MStat<sup>2</sup>; Antonella Dal Cin, BS<sup>4</sup>; Anna De Polo, MD<sup>1</sup>; Marco Schiavon, MD<sup>1</sup>; Andrea Zuin, MD<sup>1</sup>; Marco Marchetti, MD<sup>4</sup>; Giovanna Scroccaro, PharmD<sup>5</sup>; Vincenzo Baldo, MD<sup>1</sup>; Massimo Rugge, MD<sup>4</sup>; Valentina Guarneri, MD, PhD<sup>2,6</sup>; and PierFranco Conte, MD<sup>2,6</sup>; on behalf of Rete Oncologica Veneta

Buja A et al, JOP 2021



# Periplo Foundation

## Outcome-COsts-CAnCER Programme



Registro  
Tumori  
Veneto



Rete Oncologica Veneta  
FONDAZIONE PERIPLO



UNIVERSITÀ  
DEGLI STUDI  
DI PADOVA



Observational cohort study collecting all cases of NSCLC diagnosed in Veneto Region over three years (2017,2019,2021) to evaluate:

- 1) Quality of care
- 2) Global care costs
- 3) Survival outcomes

### NON-SMALL-CELL LUNG CANCER: Real-World Population-Based Cohorts' Study

Alessandra Buja <sup>1,\*</sup>, Massimo Rugge <sup>2</sup>, Alberto Bortolami <sup>3</sup>, Manuel Zorzi <sup>4</sup>, Federico Rea <sup>1</sup>, Anna Zanovello <sup>1</sup>  
Giovanna Scroccaro <sup>3</sup>, Pierfranco Conte <sup>5,6</sup>, Giulia Pasello <sup>7,8</sup>, Valentina Guarneri <sup>7,8</sup>,  
on behalf of Rete Oncologica Veneta and Periplo Foundation

Cancers, 2025

#### Mean costs into three years from diagnosis by year and difference in costs

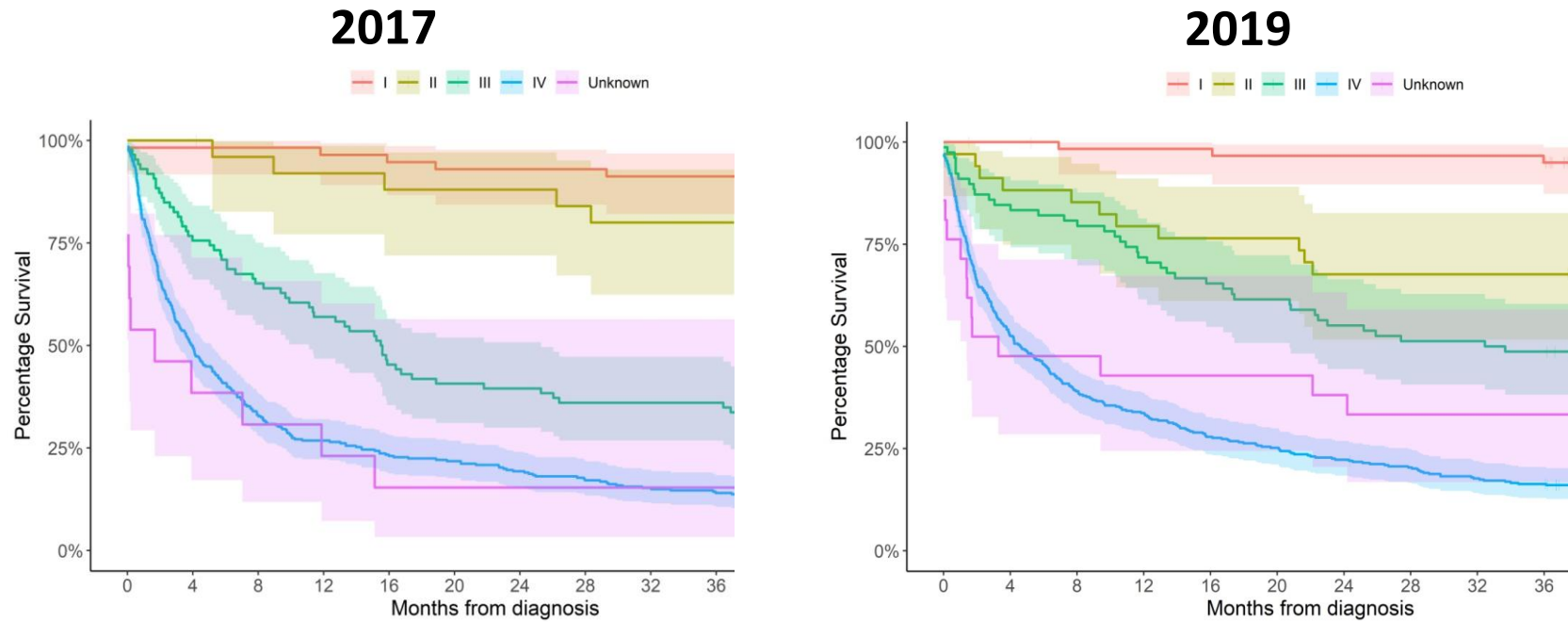
	Total	Inpatient drugs	Outpatient services	Hospitalisation	Hospice	Emergency Dept visits	Outpatient drugs	Medical devices
2017	45590.46	20718.6	9161	11469.2	1221.97	623	1581.8	814.5
2019	47846.74	2226.6	10805	10968.7	603.13	683.4	1455.7	1065
Δ	<b>+2256.28</b>	<b>+1547.96</b>	<b>+1643</b>	<b>- 500.54</b>	<b>- 618.84</b>	<b>+60.44</b>	<b>- 126.08</b>	<b>+250.21</b>

2021 cohort: work in progress



# OCOCA- Lung

## NSCLC: OS by stage and year of diagnosis



**1,050 NSCLC patients diagnosed in 2017 and 2019.**  
**Significant overall OS improvement in 2019 cohort: HR 0.84 (95%CI 0.72-0.98); p value 0.024.**  
**Significant LCS-OS improvement in 2019 cohort for stage III disease: HR 0.61 (95% CI 0.41-0.91); p value 0.025**

A Buja et al, Cancers 2025

# Periplo Foundation

## OCOCA Lung Cancer – Key Findings

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### 2019 compared to 2017:

- Significantly increased costs of inpatient drugs (doubled use of ICPs in 2019 over 2017)
- 55% of increased inpatient drug cost compensated by decreased costs in hospitalisation, hospice, outpatient drugs
- 73% of total cost increase due to outpatient services (laboratory tests, imaging, specialist visits)
- 16% reduction in risk of death (p 0.024)

# The NHS: a key player in innovation & sustainability

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- **Silo-based health care funding**

Trial based BI does not take into account the country-specific prevalence of molecular dysregulations and clinical practice

Innovation may be sustainable

**NHS funding system certainly NOT!**

- Diagnostic-therapeutic pathways based on real world clinical data

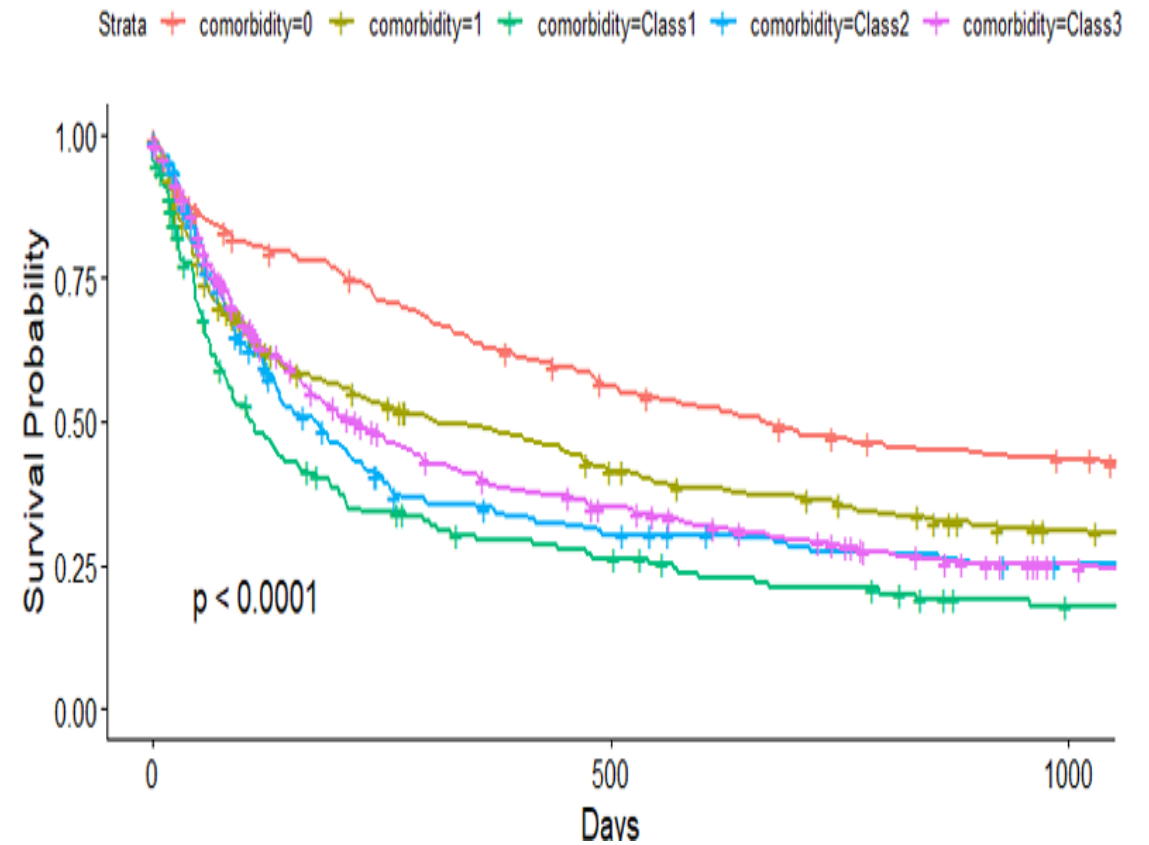
Article  
**Patterns of Comorbidities in Lung Cancer Patients and Survival**

Alessandra Bujia <sup>1,\*</sup>, Marcello Di Pumo <sup>2,3</sup>, Massimo Rugge <sup>4</sup>, Manuel Zorzi <sup>5</sup>, Federico Rea <sup>1</sup>,  
 Ilaria Pantaleo <sup>1</sup>, Giovanna Scroccaro <sup>6</sup>, Pierfranco Conte <sup>7</sup>, Leonardo Rigon <sup>7,8</sup>, Giorgio Arcara <sup>7,9</sup>,  
 Giulia Pasello <sup>10,11</sup> and Valentina Guarneri <sup>10,11</sup> on behalf of Rete Oncologica Veneta and Periplo Foundation

Cancers 2025

NSCLC by gender, age and number of comorbidities		
	Number (%)	Mean Comorbidities
Females	611 (36.5)	1.93
Males	1063 (63.5)	2.18
Age		
< 65 yrs	316 (18.9)	1.89
65-69	207 (12.4)	1.99
70-74	328 (19.6)	1.90
75-79	309 (18.4)	2.11
> 80	514 (30.6)	2.36
Nb of comorbidities		
0	283 (16.9)	
1	391 (23.4)	
2	309 (18.4)	
≥ 3	557 (33.3)	
Missing	134 (8.0)	

## OCOCA- Lung Co-morbidities and Lung Cancer Specific Survival



Article

# Healthcare Costs by Comorbidity Patterns in Lung Cancer Patients

Alessandra Buja <sup>1,\*</sup>, Massimo Rugge <sup>2</sup>, Marcello Di Pumpo <sup>3,4</sup>, Manuel Zorzi <sup>5</sup>, Federico Rea <sup>1</sup>, Ilaria Pantaleo <sup>1</sup>, Giovanna Scroccaro <sup>6</sup>, Pierfranco Conte <sup>7</sup>, Leonardo Rigon <sup>7,8</sup>, Giorgio Arcara <sup>7,9</sup>, Giulia Pasello <sup>10,11</sup> and Valentina Guarneri <sup>10,11</sup>

Cancers 2025

**Table 4.** Three-year generic healthcare costs (all cause) by comorbidity category (€: mean per patient).

	No Comorbidity	1 Comorbidity	Class1 Cardiovascular-Respiratory and Endocrine Diseases	Class 2 Multiorgan Diseases	Class 3 Socio-Multifactorial Neuro Conditions	Overall	p-Value
Hospitalizations	13,028.54	13,666.04	13,246.98	15,539.10	17,719.42	15,000.21	<0.001
Hospital drugs	22,996.38	24,825.12	15,651.43	13,483.54	15,798.49	19,627.14	0.077
Community drugs	1117.88	1245.28	1455.04	1451.11	1713.92	1370.89	0.001
Outpatient	8352.54	9207.73	5479.90	6843.54	8531.85	8162.09	<0.001
Emergency room	497.02	517.20	676.61	749.27	761.72	630.56	<0.001
Hospice	701.76	908.00	1208.46	761.19	1156.79	929.63	0.503
Medical devices	995.93	1670.48	728.69	1433.49	1701.26	1434.79	0.132
<b>Total costs</b>	<b>47,690.06</b>	<b>52,039.85</b>	<b>38,447.09</b>	<b>40,261.25</b>	<b>47,383.45</b>	<b>47,155.30</b>	<b>&lt;0.001</b>

# Periplo Foundation

## OCOCA Lung Cancer – Key Findings

---

### 2019 compared to 2017:

- Significantly increased costs of inpatient drugs (doubled use of ICPs in 2019 over 2017)
- 55% of increased inpatient drug cost compensated by decreased costs in hospitalisation, hospice, outpatient drugs
- 73% of total cost increase due to outpatient services (laboratory tests, imaging, specialist visits)
- 16% reduction in risk of death (p 0.024)

### Overall Data:

- Patients aged 80+ years at diagnosis represent 30.6% of lung cancer patients in Veneto
- 51.7% of lung cancer patients have  $\geq 2$  comorbidities
- Presence of at least 1 comorbidity is associated to a significantly higher risk of death
- High overall health care costs for patients with comorbidities (in spite of reduced costs of hospital drugs)

### Ongoing:

- Use of innovative oncology drugs in patients over 80 years old and/or with  $\geq 2$  comorbidities

# The NHS: a key player in innovation & sustainability

---

- **Silo-based health care funding**

Trial based BI does not take into account the country-specific prevalence of molecular dysregulations and clinical practice

Innovation may be sustainable

**NHS funding system certainly NOT!**

- **Diagnostic-therapeutic pathways based on real world clinical data**

Elderly & comorbid cancer patients require dedicated care pathways

KPIs (not only drugs!) are crucial to assess appropriateness and efficacy of care pathways

# RWD and a sustainable innovation

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## Periplo Foundation

### ▪ Board of Directors:

Pierfranco Conte (chair)

Paolo Pronzato (vice chair)

Sandro Pignata

### ▪ Scientific Committee:

Gianni Amunni

Mariangela Ciccarese

Pierfranco Conte (chair)

Valentina Guarneri

Carmine Pinto

### ▪ Organizational Secretariat:

Over srl

## Periplo Foundation ongoing & planned projects:

- Perseo Study
- Real World Data Task Force
- National Registry on MTB (G Pruneri)

# PERIPLO OCOCA – PERSEO Study

PROMOTORE: IRST IRCCS

STEERING COMMITTEE: Gianni Amunni, Fabrizio Gemmi, Daniele Generali, Ilaria Massa, Antonino Musolino, Manuela Roncella

Chief Steering Committee Pier Franco Conte per Fondazione Periplo

STATISTICAL COMMITTEE: William Balzi, Alessandra Buja, Claudia Szazs



**ISPRO**  
Istituto per lo studio, la prevenzione  
e la rete oncologica

ISTITUTO  
ROMAGNOLO  
PER LO STUDIO  
DEI TUMORI  
DINO AMADORI

- **Type of Study:**

Retrospective/prospective, observational, multicentric non interventional study

- **Study Population:**

Patients with operable breast cancer treated with neo/adjuvant therapy from 2014 to 2024 (~ 45,000 patients)

- **Eligibility Criteria:**

Access to electronic administrative databases

Demographic data

Specialist Outpatient Care (SPS)

Hospital Discharge Records (SDO)

Drug Consumptions (DDF and Cineca)

Death Certificates

- **Participating Centers:**

Agenzia Regionale di Sanità (ARS), Tuscany Region

Istituto Romagnolo per lo studio e la cura dei Tumori «Dino Amadori» – IRST IRCCS

Istituto per lo Studio e Prevenzione Oncologica – ISPRO

Words can be stones...  
Data are bricks!

fondazione  
Periplo

