

FDA-ASCO: Geriatric Oncology Workshop

Leveraging research designs for real-world patients: Real-world evidence

From RCTs to Observational Research

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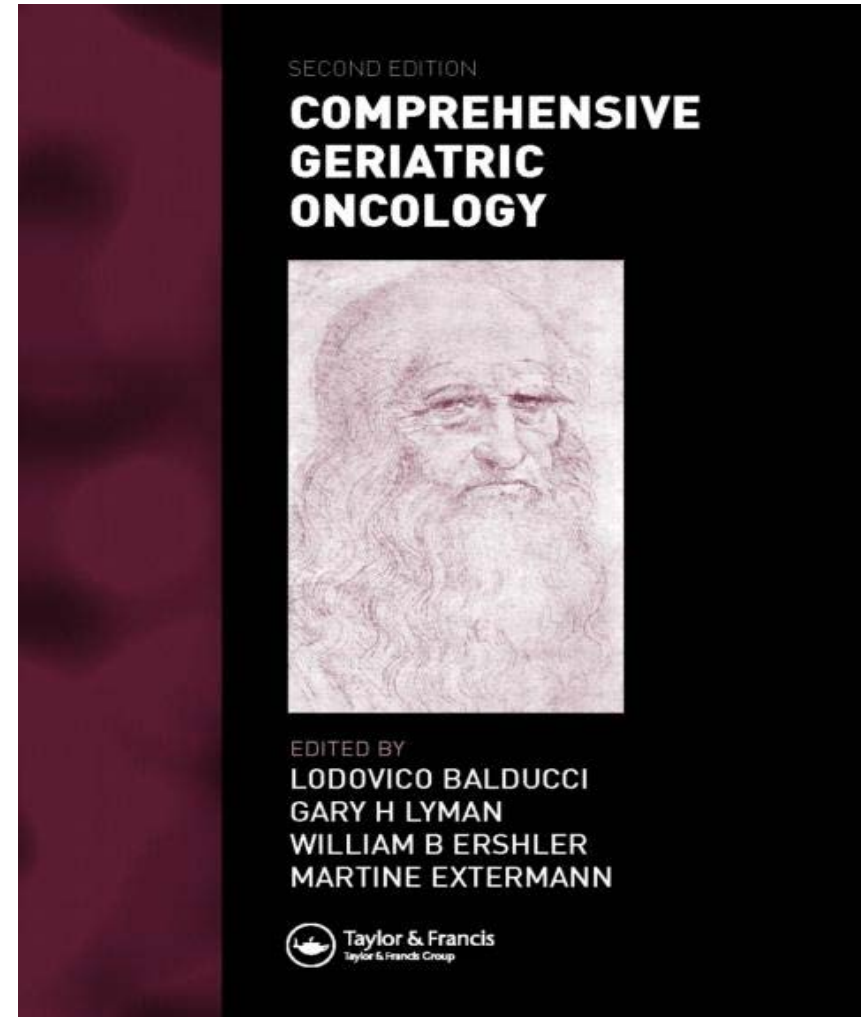
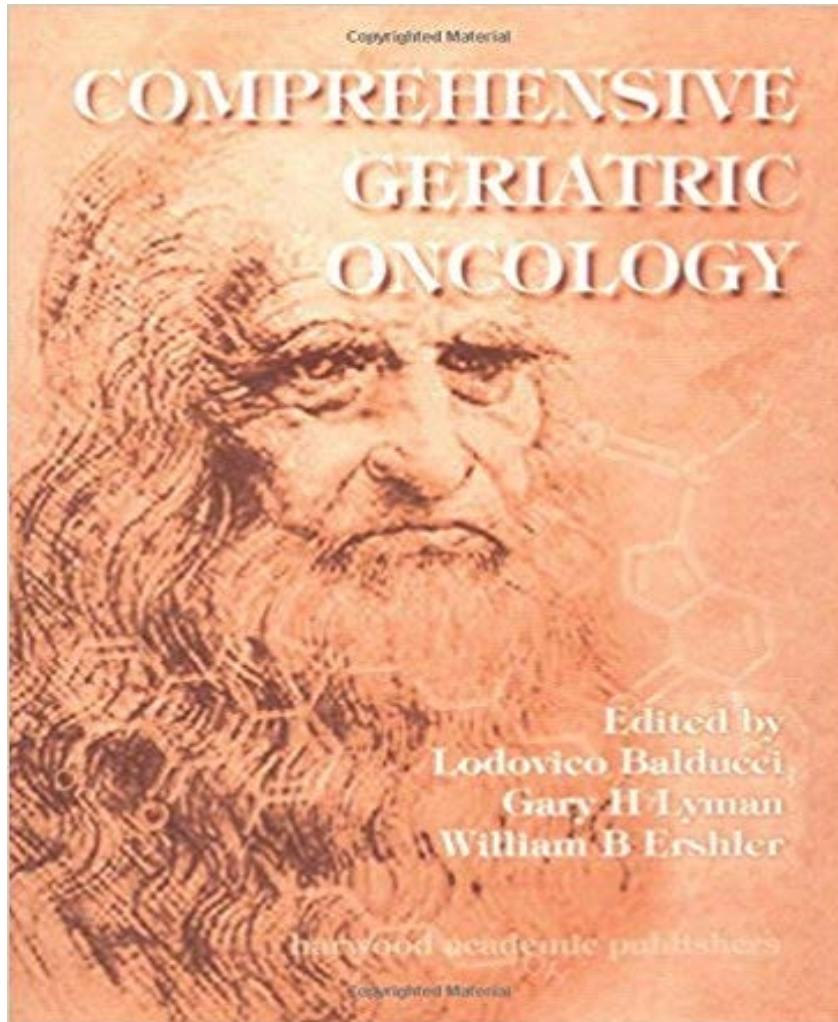
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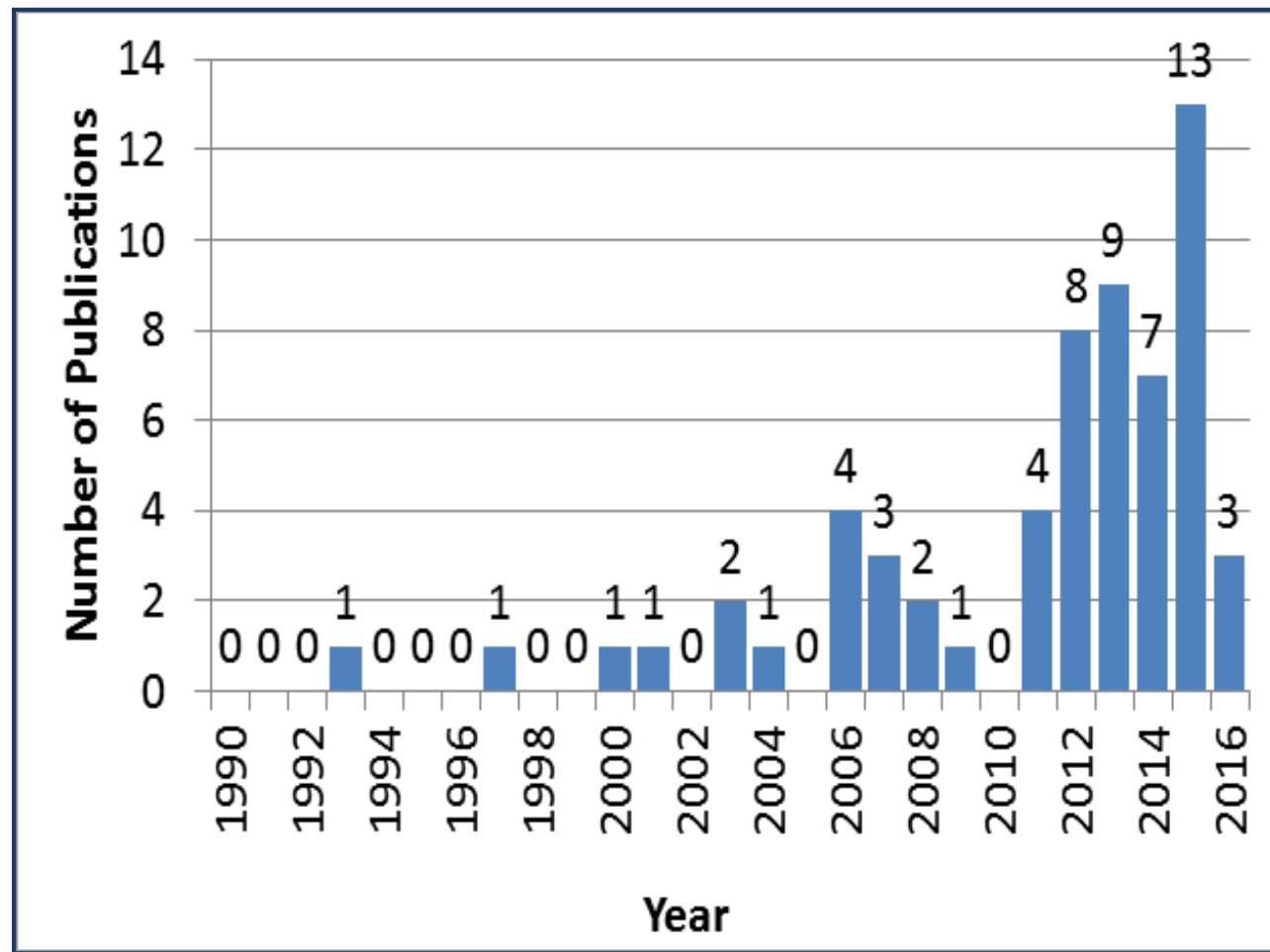
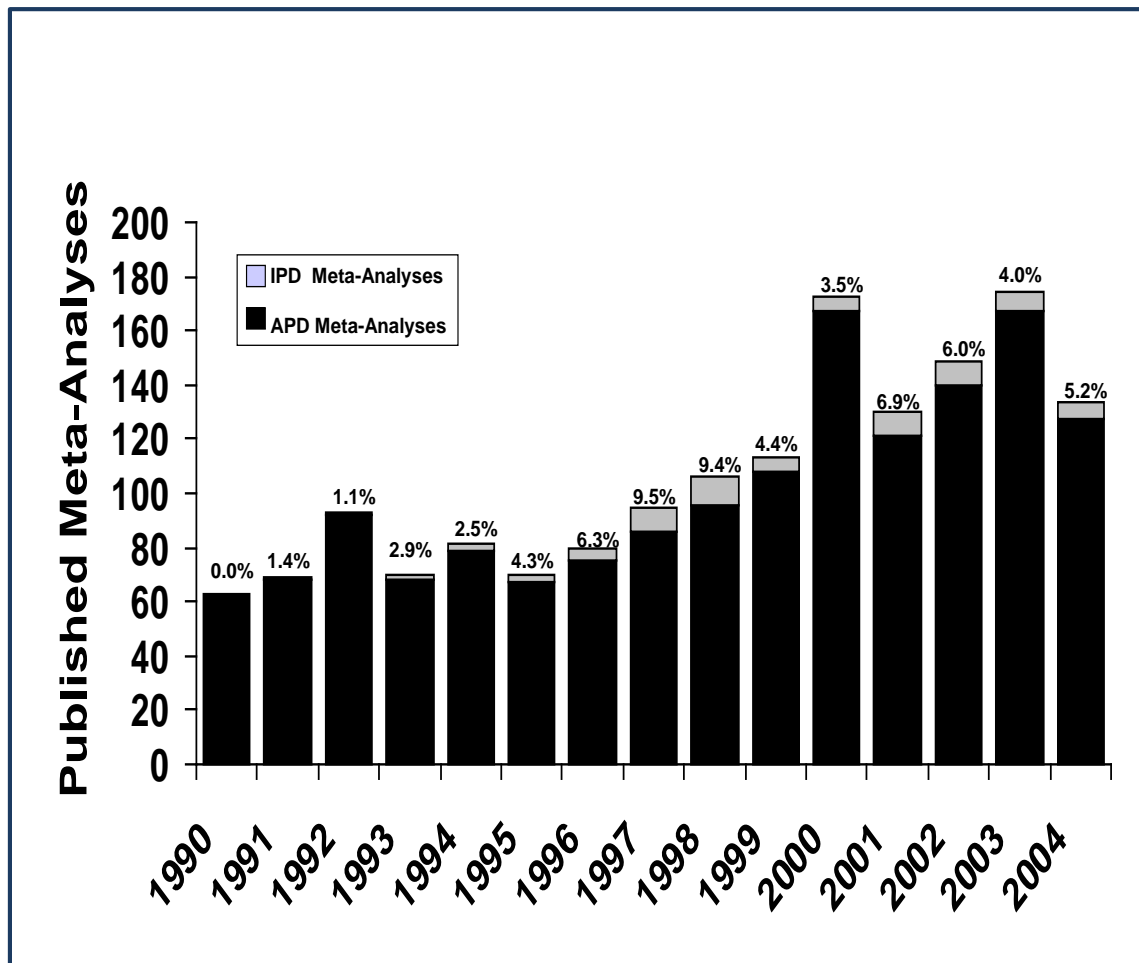
Hutchinson Institute for Cancer Outcomes Research

Geriatric Oncology



Balducci L, Lyman GH, Ershler WB, Extermann M: Comprehensive Geriatric Oncology, 1992 1998, 2004

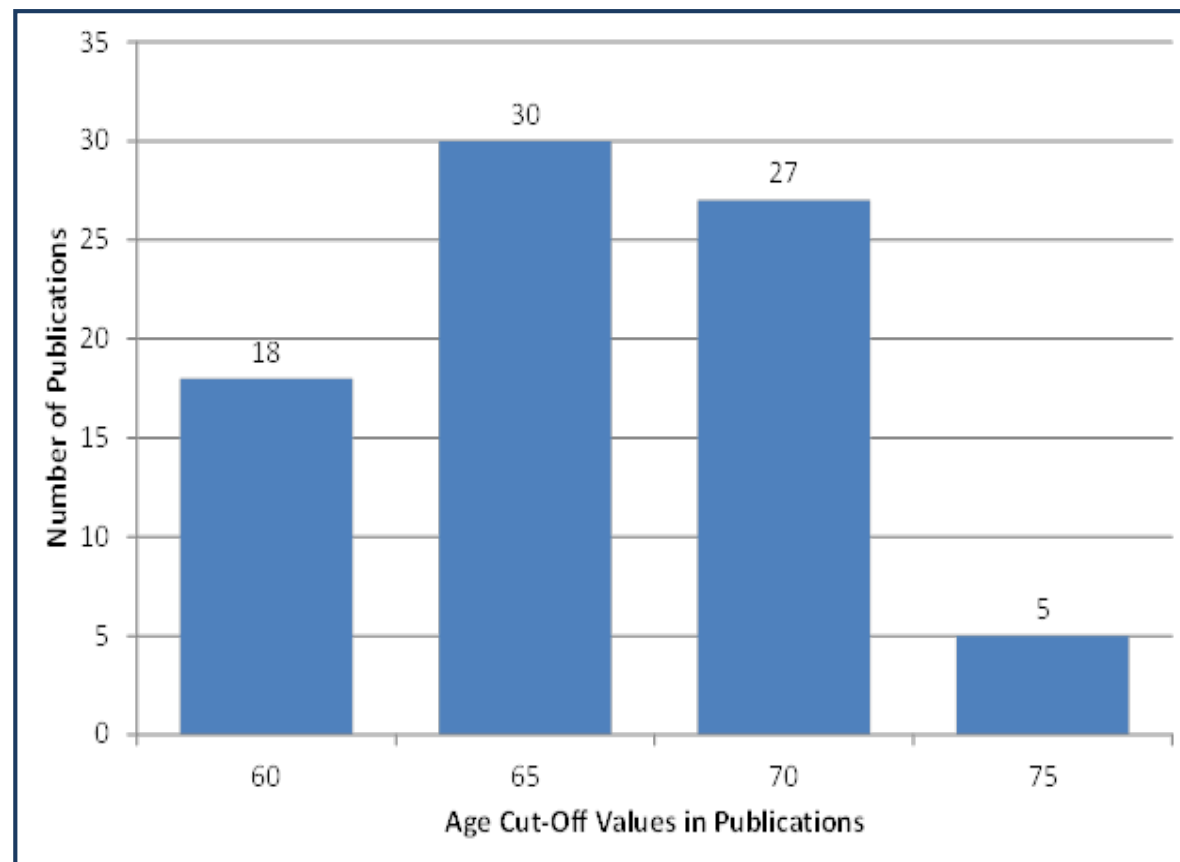
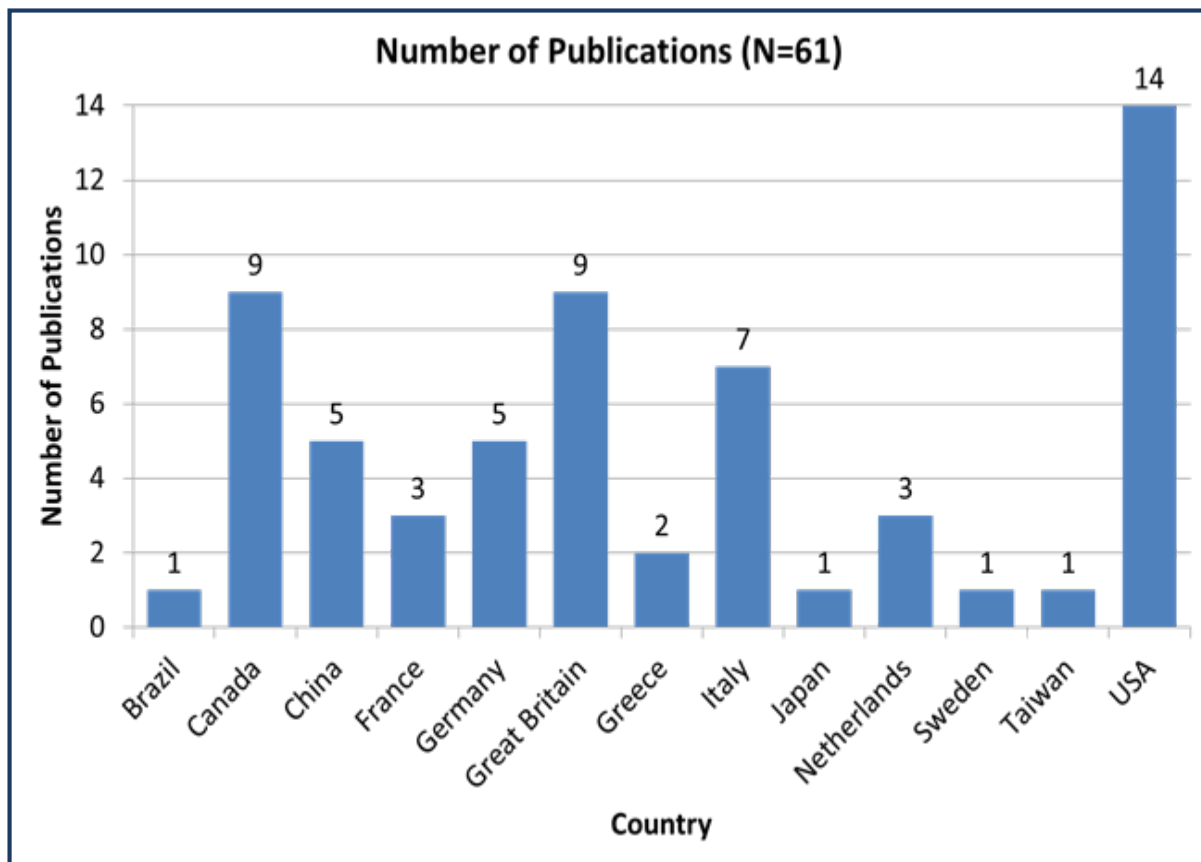
Systematic Reviews and Meta-analysis of RCTs in Geriatric Oncology



Lyman GH, Kuderer NM: BMC Med Res Methodol 2005; 5: 1-7

Lyman GH, Poniewierski MS: in GERIATRIC ONCOLOGY (M Extermann ed), Springer 2017 (in Press)

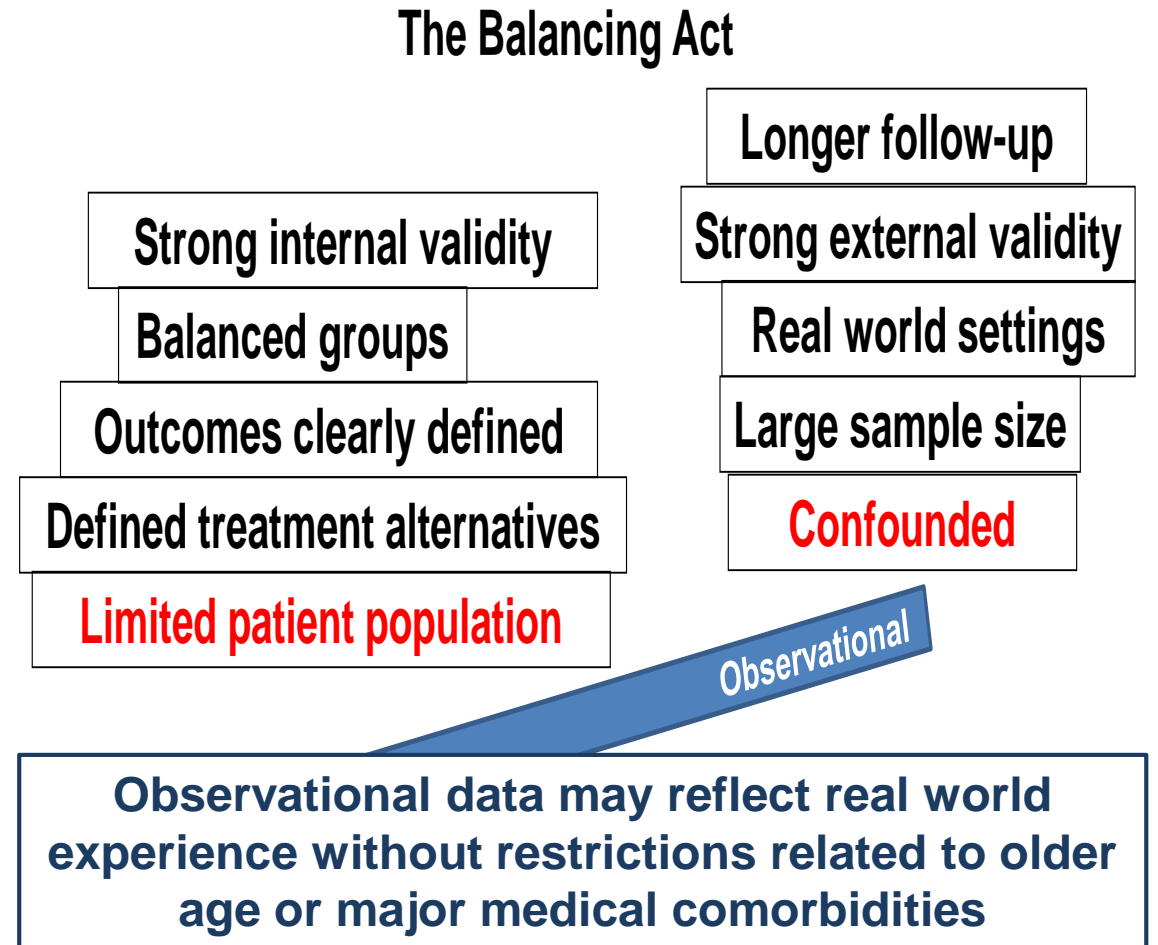
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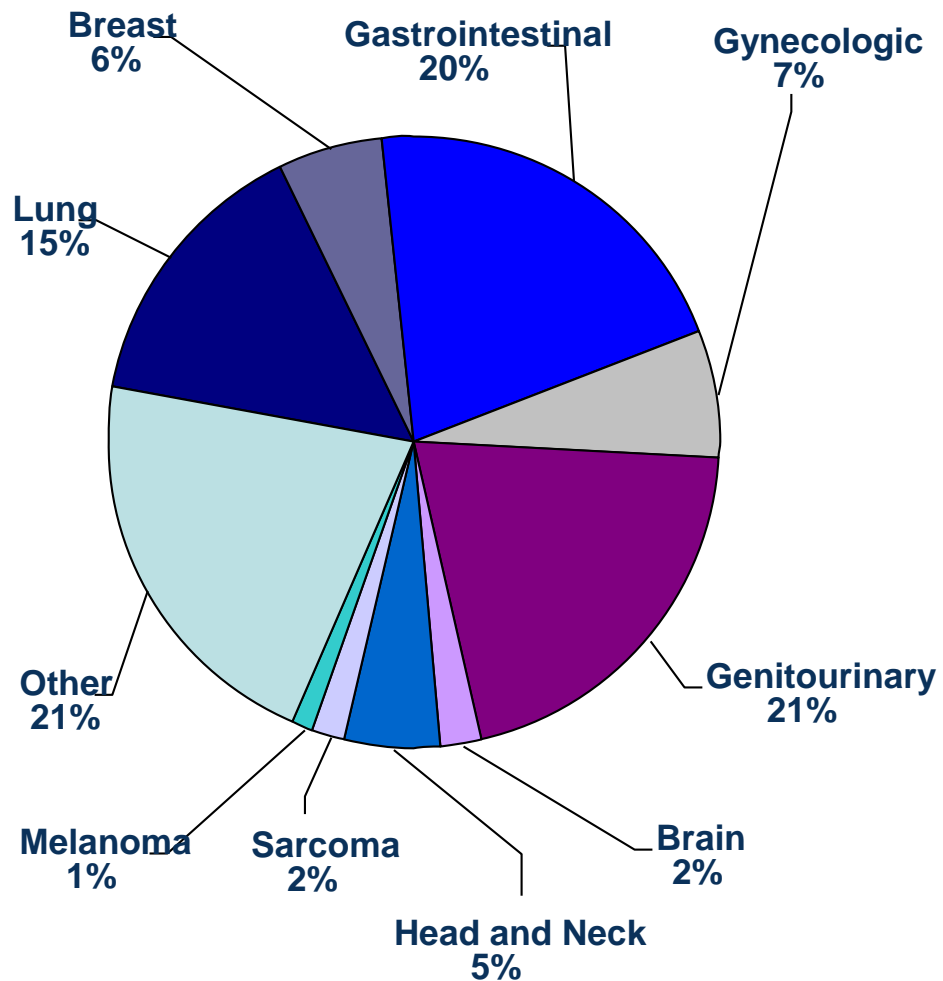
RCTs vs Observational Data: Find the Right Balance

- **Limited external validity:**
 - narrow eligibility criteria (poor generalizability)
- **Limited info on vulnerable subgroups:** elderly; comorbidities
- **Feasibility and ethical issues**
- **Costly:** time and resources
- **Treatment imbalance by chance**
- **Limited attention to toxicities:**
 - especially rare/delayed effects
 - not powered for toxicity events

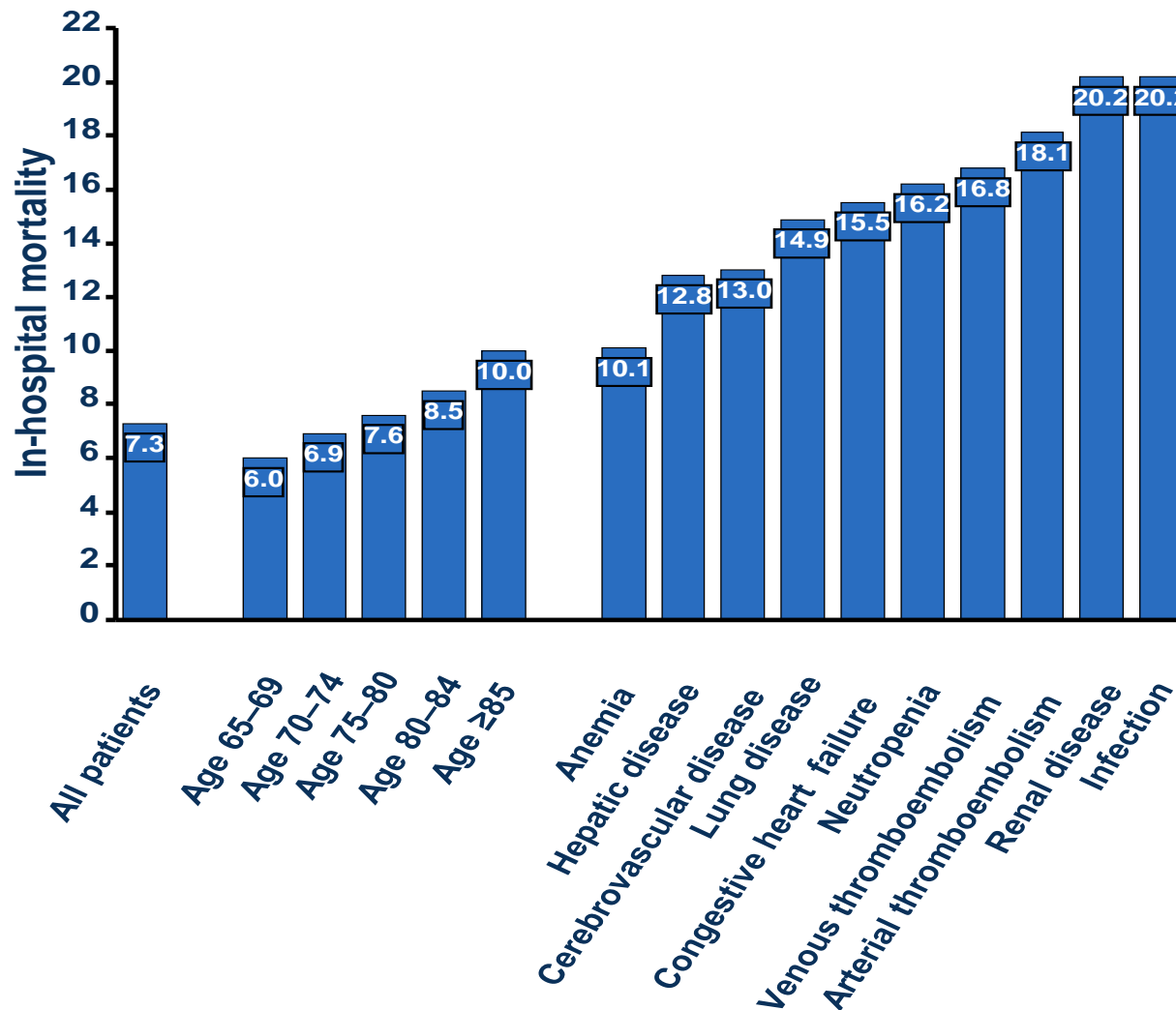


Kuderer N, Wolff A: JCO 2014 32: 1990-1993
Booth CM, Tannock IF: BJC 2014; 110: 551-555

Observational Studies in Geriatric Oncology



n=386,377



Shayne M et al. J Geriatr Oncol 2013; 4: 310-318

The Untapped Potential of Observational Research to Inform Clinical Decision Making

Research Statement from the American Society of Clinical Oncology

Working Group

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The Untapped Potential of Observational Research to Inform Clinical Decision

PRIMARY CONCLUSION: Observational Research is Critical to Informing Health Care Decisions

- Unbiased observational studies that answer research questions of interest are complimentary to RCTs.
 - Hypothesis generating.
 - Develop evidence not answered by RCTs.
- Need to ensure the quality of observational research, as it takes on an expanded role in clinical decision making.

The Untapped Potential of Observational Research to Inform Clinical Decision

Recommendation 1: Establish common data standards & definitions

- National effort to promote existing and emerging standards for the collection, exchange, and reporting of digital healthcare information.
- Oncology providers should support the systematic collection of data in EMRs.
- Vendors and providers ensure products support the capture of meaningful structured data, e.g., stage, recurrence, PRO's.

Recommendation 2: Improve interoperability in electronic health records

- Legislation to ensure widespread interoperability in EMRs.
- Develop true interoperability between electronic clinical data systems.
- Ensure providers are prudent purchasers and users of EMRs.

The Untapped Potential of Observational Research to Inform Clinical Decision

Recommendation 3: Utilize rigorous observational research

- Follow best practices in observational research, as outlined in methodology guides.
- Training programs and professional societies provide training in interpretation and conduct of observational research.

Recommendation 4: Transparent reporting of observational research

- Encourage authors to follow reporting guidelines for observational research,
- Include experts in observational research among peer reviewers of manuscripts.

Recommendation 5: Protect patient privacy

- Identify gaps in privacy protections and ensure responsible use of observational data for research.
- Increase investment in privacy enhancing technologies and incentivize their use.
- Increase transparency about uses of patient data.
- Encourage patients' right to access their health information.

Leveraging Observational Data to Study Novel Cancer Therapies in Older Patients

Observational Studies

The Changing Landscape

Big Data & Rapid Learning Systems

Non-randomized controlled studies

Cohort Studies

Population Studies

Cancer Registries

Administrative & Claims Databases



Challenges/Opportunities

- **Linking molecular data to treatment and outcomes**
- **Capturing toxicities of molecularly targeted agents and immune-related adverse events**
- **Adjustment for major medical comorbidities**
- **Patient centered outcomes**
 - PROs and HRQOL
 - Frailty
 - CGA
- **Resource Utilization & costs**

CANCER LINQ: ASCO's Big Data Initiative into Observational Data



**Unlock, assemble, and analyze
de-identified cancer patient medical
records**



**Uncover patterns that generate
knowledge**



**Provide guidance by identifying the best
evidence-based course of care**



Accelerate Innovation



Thank You