Cancer Drug Development

As a public health matter?

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Disclosures

I have no relationships with the pharmaceutical industry

The privilege of practicing oncology

The essence of oncology



Outline

1. Two Paradoxes

2. The Cancer Value Crisis

3. Common Sense Oncology



The Cancer Medicine Paradox

- 1. There is substantial over-utilization of marginal, toxic and expensive medicines (especially near the end of life).
- 2. At the same time, due to problems with access and affordability most patients in the world do not get the cancer medicines which make a big difference in outcomes.



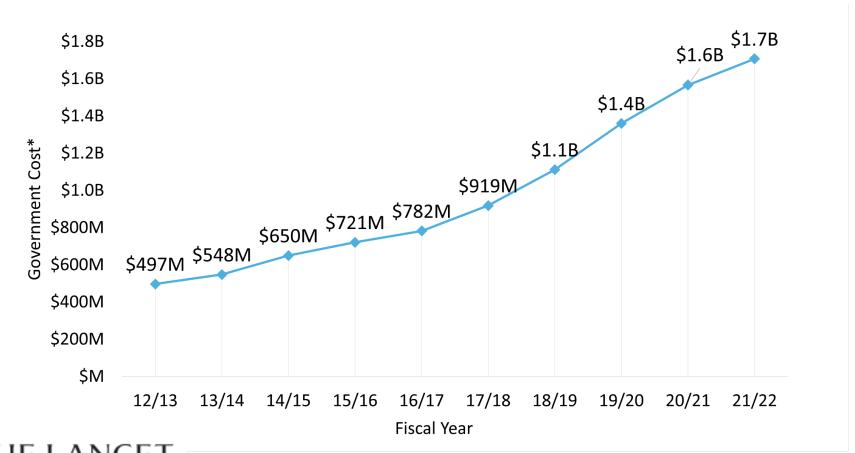
Three Buckets Paradox





Cost and value of cancer medicines in a single-payer public health system in Ontario, Canada: a cross-sectional study

Joseph C Del Paggio, Rohini Naipaul, Scott Gavura, Rebecca E Mercer, Rachel Koven, Bishal Gyawali, Brooke E Wilson, Christopher M Booth



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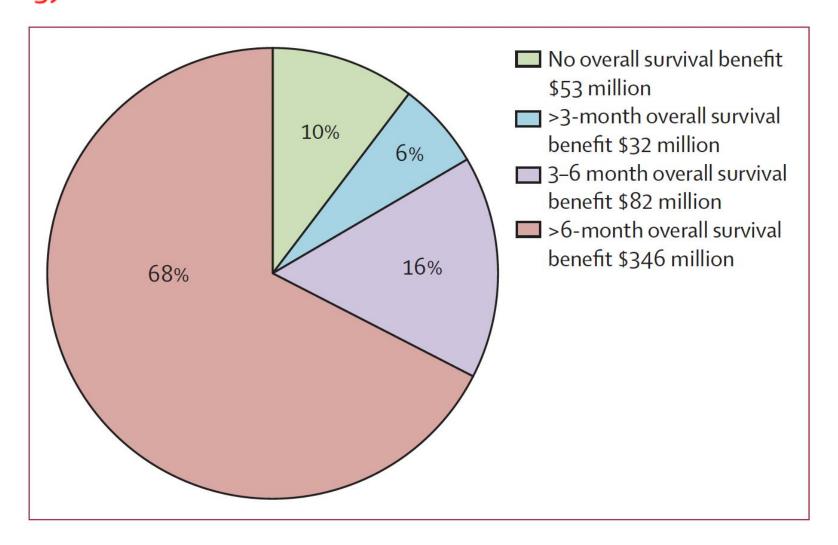
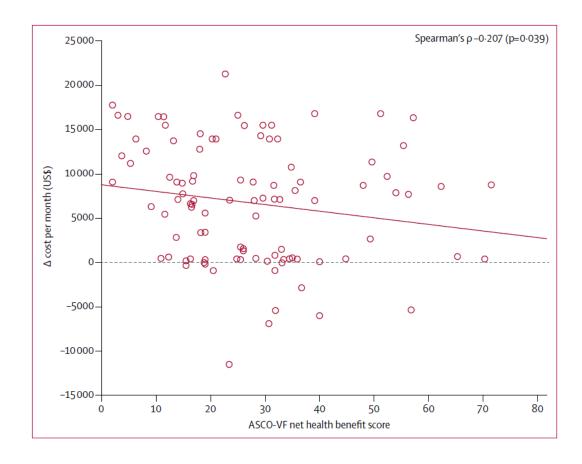


Figure 3: Proportion of expenditure in the Ontario health system on top-ten intravenous cancer medicines (by expenditure) in the 2021–22 fiscal year, stratified by randomised clinical trial evidence for overall survival

Delivery of meaningful cancer care: a retrospective cohort study assessing cost and benefit with the ASCO and ESMO frameworks THE LANCET Oncology

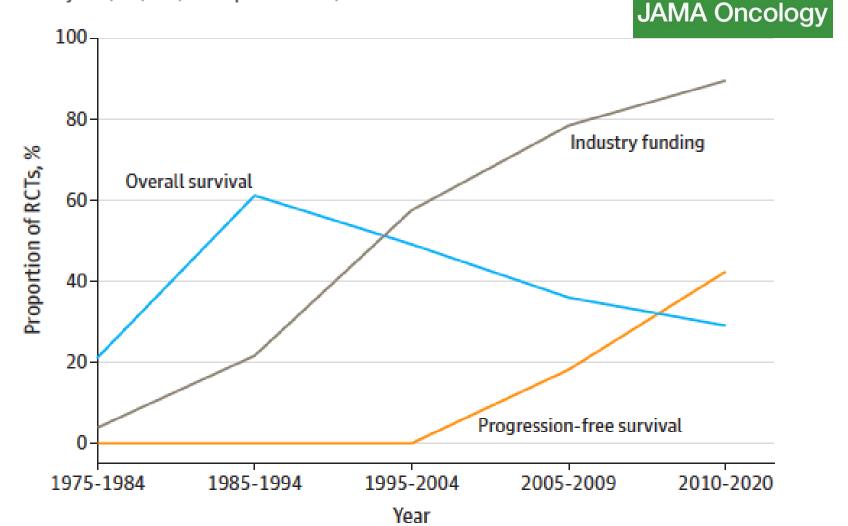
Joseph C Del Paggio, Richard Sullivan, Deborah Schrag, Wilma M Hopman, Biju Azariah, C S Pramesh, Ian F Tannock, Christopher M Booth

 No association between magnitude of benefit and drug price



Evolution of the Randomized Clinical Trial in the Era of Precision Oncology

Joseph C. Del Paggio, MD; John S. Berry; Wilma M. Hopman, MA; Elizabeth A. Eisenhauer, MD; Vinay Prasad, MD; Bishal Gyawali, MD, PhD; Christopher M. Booth, MD



PFS in Context

- 1. PFS may be useful if it is a surrogate for OS/QOL
 - Not the case in most settings for OS
 - Not the case for QOL

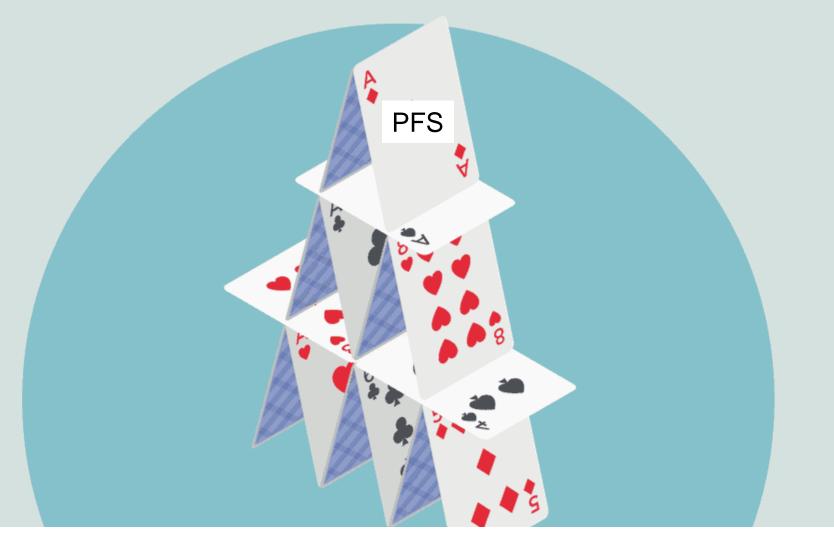
Haslam Eur J Cancer 2019 Gyawali Int J Cancer 2021

- 2. PFS may be useful if it has intrinsic meaning to patients
 - This is far from certain (especially given trade-offs of side effects, time toxicity, and cost)

Brundage JNCI 2023

3. Even if 1 or 2 is true...at least some proportion of "PFS improvement" may be due to artifact

Modern oncology is built on a very precarious endpoint



3 months

Clinical toxicity
Financial toxicity
Time toxicity
Efficacy-effectiveness gap



Fojo JAMA Oto 2014 Davis BMJ 2017 Del Paggio JAMA Onc 2021 Michaeli JCO 2022

Access to cancer medicines deemed essential by oncologists in 82 countries: an international, cross-sectional survey

Adam Fundytus, Manju Sengar, Dorothy Lombe, Wilma Hopman, Matthew Jalink, Bishal Gyawali, Dario Trapani, Felipe Roitberg, Elisabeth G E De Vries, Lorenzo Moja, André Ilbawi, Richard Sullivan, Christopher M Booth

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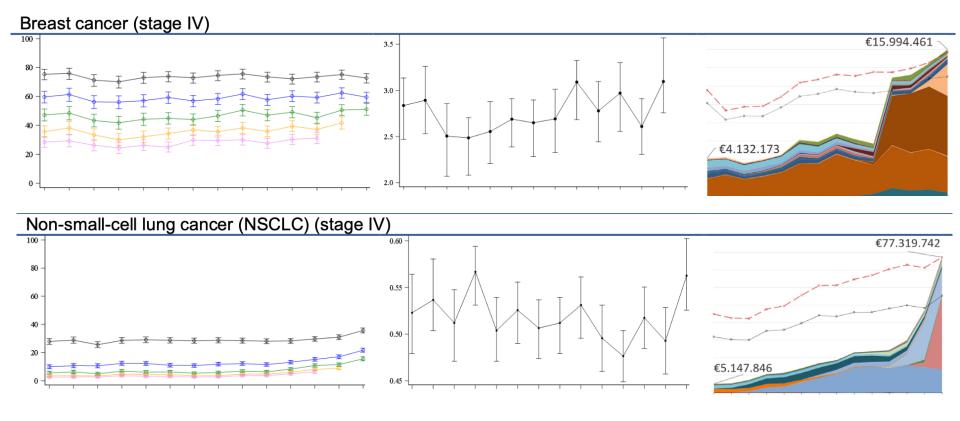
The Desert Island Project

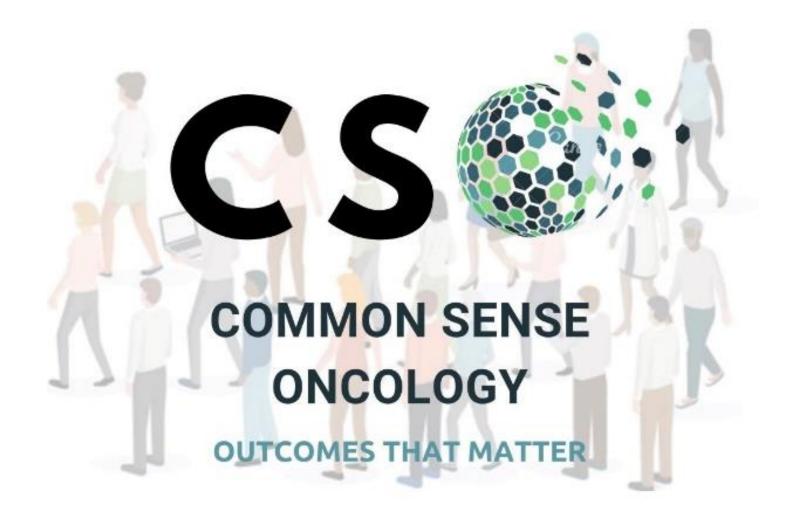
- Which medicines do front-line oncologists consider the most important?
- Are these medicines available to patients in routine clinical practice?

Has investment yielded benefits?

SUMMARY

DO INNOVATIVE MEDICINES AGAINST CANCER ALWAYS HAVE A REAL ADDED VALUE?





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OUTCOMES THAT MATTER

Common Sense Oncology: outcomes that matter

Christopher M Booth ☐ • Manju Sengar • Aaron Goodman • Brooke Wilson • Ajay Aggarwal • Scott Berry •

David Collingridge • Avram Denburg • Elizabeth A Eisenhauer • Ophira Ginsburg • Daniel Goldstein •

Sanjeeva Gunasekera • Nazik Hammad • Kazunori Honda • Christopher Jackson • Deme Karikios • Kevin Knopf •

Rachel Koven • Bernard L Marini • Deborah Maskens • Fabio Y Moraes • Ghulam Rehman Mohyuddin •

Bishesh Sharma Poudyal • C S Pramesh • Felipe Roitberg • Fidel Rubagumya • Sally Schott • Bhawna Sirohi •

Enrique Soto-Perez-de-Celis • Richard Sullivan • Ian F Tannock • Dario Trapani • Michelle Tregear •

Winette van der Graaf • Verna Vanderpuye • Bishal Gyawali • Show less

Mission

To ensure that cancer care focuses on outcomes that matter to patients

Vision

Patients have access to cancer treatments that provide meaningful improvements in outcomes that matter, irrespective of where they live or their health system. To realise this

Booth Lancet Onc 2023

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Guiding principles

- 1 Access to quality cancer care is a basic human right—no patient should be denied access to effective therapy or forced into financial catastrophe to access meaningful cancer care
- 2 Patient and societal needs should drive cancer research and delivery of care
- 3 Patient and public involvement is essential when making policy decisions
- 4 Patients should expect that recommended cancer treatments meaningfully improve their survival or quality of life
- 5 Shared decision making between patients and oncologists should be based on patient values and grounded in evidence-based medicine and critical appraisal
- 6 Cancer treatments should be fairly priced for the context in which they are used
- 7 Equity in access to high quality care should be prioritised as much as innovation and new treatments
- 8 Comprehensive patient-centred cancer care includes timely integration of psychosocial oncology, survivorship, and palliative care

CSO: Core Pillars of Work

Evidence Generation

ensure clinical trials use and report outcomes that matter

Evidence Interpretation

foster critical thinking in oncology

Evidence Communication

improve patient, public, policy-maker understanding of cancer treatment options



CSO will promote interventions that measurably improve the lives of patients. We will celebrate well conducted trials and promote effective treatments but we will also speak up about and challenge interventions that might cause more harm than good.



We will seek to decrease global and regional inequities in access to affordable high-quality care. Improvements in the generation, interpretation, and communication of evidence will help close these gaps and move our field closer to a future in which a patient's outcome is not determined by where they live, what they can afford, or the strength of a marketing campaign.

Public Health Perspective?

• Cancer medicines should help people live longer and better lives

- Oncologists treat people, not tumours
- The current economic model for cancer medicine delivery and research is broken
- Patients and health systems deserve better

