

CADTH

APRIL 23 TO 25, 2017 | OTTAWA, ON

2017 CADTH SYMPOSIUM
MEASURING VALUE IN THEORY AND THE REAL WORLD



VALUE FRAMEWORKS IN HTA

Are Value Frameworks Useful in Helping Canadian Cancer Systems Determine Funding for New Therapies?

April 24, 2017

Association canadienne
du cancer colorectal

Colorectal Cancer
Association of Canada

Background

- **Therapeutic innovation continues to grow** as new therapies enter clinical practice.
- **Increased survivorship** is transforming the approach from acute treatment to chronic disease management.
- This has resulted in a **change of focus to total patient care encompassing QoL issues and patient preferences or Values.**
- **There is pressure for better access to affordable & effective cancer treatments** and a growing call for *Value* in cancer drugs.

CAPCA Funding Sustainability

January 19, 2017 Webinar - Canadian Association of Provincial Cancer Agencies

... And Cancer Drugs Present 3 Specific Sustainability Challenges



- 1. Growth in the cancer drug pipeline.** As many as 150 new cancer therapies and 300 new drug indication pairs currently under investigation in clinical trials.
- 2. Increase in the number of new cancer cases.** By 2030 number of new cancer cases expected to grow by 40%. Survival rates continue to improve, increasing rate of recurrent and new primary cancer diagnoses.
- 3. Rising Provincial Oncology Drug Budgets:** Oncology drug budgets tripled in less than a decade and accelerated rate of growth – now approaching 12-15% annually and expected to continue. The increasing aggregate cost of cancer drugs is being caused by expanding lines of treatment, simultaneous use of multiple cancer drugs, and availability of immunotherapy and other forms of targeted therapy for growing number of cancer-specific indications.

Clinical Algorithms, Clinical Pathways, Affordability, RWE

Over the next 12-18 months, CAPCA and its partners will lead collaborative efforts to:

Harmonization

1. Implement a mechanism for provinces to work together on how new drugs will be integrated into treatment plans – including clinical algorithms – to help provinces identify and fund the best cancer treatments.

Intent is to increase consistency in drug funding decisions, inform pan-Canadian price negotiations and enhance the information available for provincial cancer drug funding decisions.

CAPCA STAKEHOLDER ENGAGEMENT

21

Over the next 12-18 months, CAPCA and its partners will lead collaborative efforts to:

Optimization

2. Develop implementation considerations for the selection and use of cancer drugs and corresponding clinical pathways to support provincial funding decisions. This work include consideration of cancer drugs that may no longer be justified.

Over the next 12-18 months, CAPCA and its partners will lead collaborative efforts to:

Affordability

3. Proposing criteria and a process to assess a drug's affordability to better inform provincial drug funding decisions.

Over the next 12-18 months, CAPCA and its partners will lead collaborative efforts to:

RWE

4. Create process to gather Real World Evidence (RWE). There is currently no mechanism to confirm if what is seen in clinical trials is what will be the case when the drug is made available to a larger population. Consistent use of RWE across Canada is essential for ongoing assessment and reassessment of cancer drugs.



CAPCA STAKEHOLDER ENGAGEMENT

23

CAPCA STAKEHOLDER ENGAGEMENT

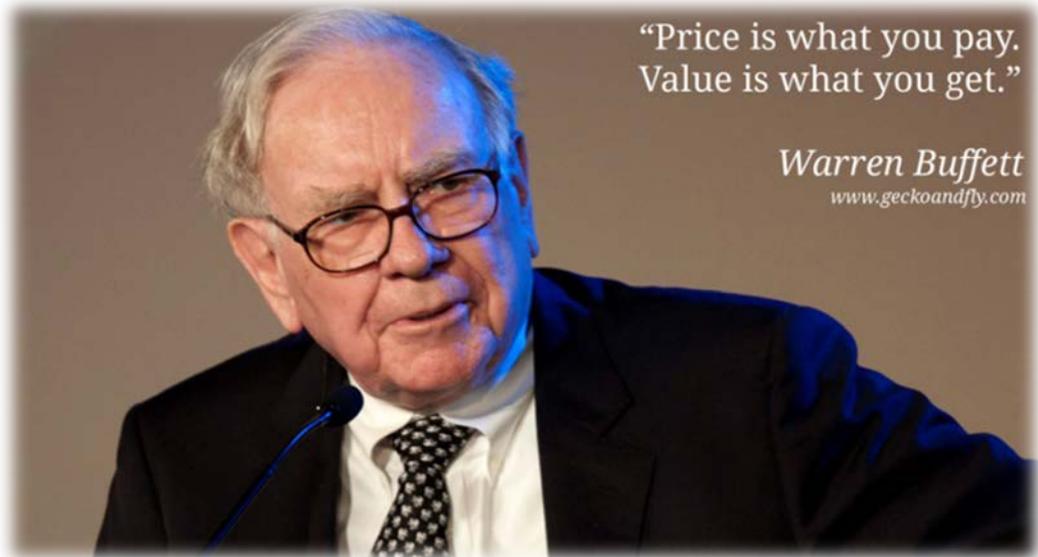
24

The Challenge

Defining, Measuring & Weighing “Values” as they apply to new oncology drugs is a challenge faced by many stakeholders with different interests.

- **Patients:** Have the most vested interest.
- **Advocacy groups:** Equal and timely access to effective treatments to improve patient outcomes.
- **Physicians:** Treatment options for best outcome.
- **Healthcare providers:** Best treatments within their system and budget.
- **Regulators:** Fairness and diligence in assessing the risk/benefit ratio according to their methods of appraisal.
- **Government agencies:** Policy and direction on best spending practices to ensure overall population good health.
- **Third-party payers:** Best value from available funds when covering prescription choices.

What is Value?



“Price is what you pay.
Value is what you get.”

Warren Buffett
www.geckoandfly.com

- **VBM** is more than managing drug costs, it is part of a broader debate on access to treatment that includes differing stakeholder expectations on **value in cancer care** in general.
- There is also a shift from a product-oriented approach, rooted in science and efficacy, to a broader assessment that includes **pharmacoeconomics, therapy management, compliance issues and patient QoL.**
- **New approaches are developing to address this issue, but do they truly account for what patient’s value?**

Cancer Patient Group Input

In Canada, cancer PATIENT GROUP INPUT is reviewed by pERC (pCODR Expert Review Committee) as part of the deliberative framework when assessing a cancer drug for reimbursement.

**CLINICAL
BENEFIT**

**ECONOMIC
EVALUATION**

pERC's Deliberative Framework
for drug funding
recommendations focuses on 4
main criteria:

**ADOPTION
FEASIBILITY**

***PATIENT-
BASED VALUES***

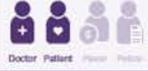
Review Identified 7 value frameworks

- **All frameworks aim to provide an objective assessment of the value of anti-cancer therapies, but vary in their definition of value, methodology, target audience, and stage of development.**
- **Similarities of the frameworks include:**
 - assessing treatment value in a transparent and objective manner,
 - safety and clinical efficacy being vital elements,
 - **patient preference and quality of life not being included or given much weight.**

Differences include: stage of development, target audience, methodology, and value dimensions and items.

Comparison of 7 Value Frameworks; target audiences, key points of critique

Table 1. Comparison of the seven frameworks assessing the value of cancer treatments

Organization/framework	Country/region of origin	Year established	Target audience	Main points of criticism (from a review of the literature and opinion papers)
Dutch Association for Medical Oncology, PASKWIL ⁵	Netherlands	1999	 Doctor Patient Payer Policy-maker	<ul style="list-style-type: none"> • Thresholds to evaluate safety and efficacy are somewhat arbitrary • Focuses on drug acquisition costs only, excluding the majority of health care costs from the evaluation^{21,24}
National Health Service, CDF Prioritisation Tool ⁶	England	2011	 Doctor Patient Payer Policy-maker	<ul style="list-style-type: none"> • Decision-making about which drugs should be included in the CDF is not transparent²⁵ • Bypasses and undermines NICE. Inefficient to assess the same drugs twice²⁵ • May be seen as prioritizing cancer at the expense of other therapeutic areas²⁶ • Arbitrarily derived values associated with different dimensions do not necessarily produce a coherent overall score²²
ESMO Magnitude of Clinical Benefit Scale ⁷	Europe	2015	 Doctor Patient Payer Policy-maker	<ul style="list-style-type: none"> • Can only be applied to comparative outcomes from RCTs; not applicable when evidence derives from single-arm or real-world evidence studies^{7,16} • Validity of results may be influenced by quality and design of the study; e.g. a relatively weak control arm may generate exaggerated benefit of the experimental drug⁷ • Relative weighting of evidence and thresholds for hazard ratio and absolute gains involves judgments and subjective considerations which are open to dispute and challenge⁷ • Rankings are based on expert opinion only. Preferences of patients, family, or general public are not considered. Explicit measures of QoL from patient perspective are lacking^{19,24} • Developed only for solid tumors⁷ • Does not consider any cost-related information as part of the evaluation¹⁶
ASCO Conceptual Framework ⁸	USA	2015	 Doctor Patient Payer Policy-maker	<ul style="list-style-type: none"> • Scoring categories for OS, PFS, and response rate are somewhat arbitrary and weights are based largely on expert clinical opinions^{8,21} • Summing arbitrarily derived values associated with different dimensions does not necessarily produce a coherent overall score²² • Derived from RCTs directly comparing two or more regimens and, hence, does not permit cross-trial comparisons^{8,15,27} • QoL outcomes are excluded and patient preferences are not accounted for^{16,18} • NHB scores may be inaccurate due to a lack of peer-reviewed publications, non-randomized trial designs, median OS or PFS not reached at the time of final analysis, and palliation or treatment-free interval data that are not available²⁹ • Drug pricing is set at launch, whereas demonstrated value will change over the product's life cycle^{16,22}
NCCN Evidence Blocks ²⁹	USA	2015	 Doctor Patient Payer Policy-maker	<ul style="list-style-type: none"> • Evidence Blocks use OS as primary end point in determining efficacy, when PFS and improved QoL are important measures not considered by the tool³⁰ • Scores reflect a standardized view based on the "average patient" even though the circumstances for each cancer patient may differ significantly³⁰ • A patient's insurance policy and ability to pay for a specific treatment can be unique, and it is unclear how affordability based on average costs of various clinical items can be made relevant to an individual patient^{21,27,30} • Payers may use the value assessment provided in the Evidence Blocks to justify coverage decisions³⁰
MSKCC DrugAbacus ¹¹	USA	2015	 Doctor Patient Payer Policy-maker	<ul style="list-style-type: none"> • Lacks some potentially important information, such as how patients describe their experience with the treatment¹⁰ • A strategy setting prices to reflect research, development, and production costs for drugs does not reflect value or patient preferences²² • It remains unclear how the various domain measurements have been derived from clinical trial data. For instance, the data do not contain any information about the probability of discontinuing drug use which is used for the toxicity attribute²¹ • Drug manufacturers might consider increasing the prices of drugs considered by DrugAbacus to be excellent value for money²¹ • Currently, only shows information for the first FDA-approved indication²⁷
ICER Value Assessment Project ¹³	USA	2015	 Doctor Patient Payer Policy-maker	<ul style="list-style-type: none"> • Limits each drug's budget impact to an arbitrary value of \$904 million annually. The budget criterion used has little to do with patient benefit and may discourage companies from developing drugs designed to help significant numbers of patients^{20,22} • A 5-year time horizon is considered in the Value Framework's "Health System Value" metric, so long-term benefits and cost off-sets of some therapies may be undervalued^{20,22} • Methods used to assess the value of a therapy are based on a QALY benchmark, the disadvantages of which are well documented²⁰ • The data on which the "Health System Value" metric is based is not evidence-based, nor does it accurately reflect the realities of the marketplace²⁰ • Economic benefits such as improvements in worker productivity or reductions in caregiver burden are not taken into consideration²⁰

* Critiques were derived from the literature and are those of the authors but not necessarily those of the companies they work for.

ASCO = American Society of Clinical Oncology; CDF = Cancer Drugs Fund; ESMO = European Society for Medical Oncology; FDA = United States Food and Drug Administration; ICER = Institute for Clinical and Economic Review; MSKCC = Memorial Sloan Kettering Cancer Center; NCCN = National Comprehensive Cancer Network; NHB = net health benefit; NICE = National Institute for Health and Care Excellence; OS = overall survival; PASKWIL = Palliative/Adjuvant effectiveness, Specific adverse events, (Kw) quality of life, Impact of treatment, and Level of evidence; PFS = progression-free survival; QALY = quality-adjusted life-year; QoL = quality of life; RCTs = randomized controlled trials.

Framework Tool and Value Items

✓ = included in algorithm/calculation

✓ = shown for information or comparison purposes

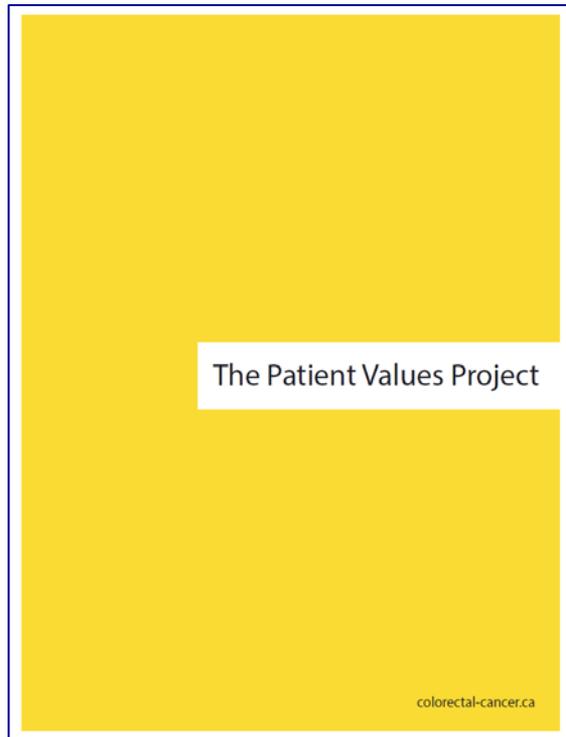
x = not included or shown

Value item	PASKWIL ⁵	CDF Prioritisation Tool ⁶	ESMO Magnitude of Clinical Benefit Scale ⁷	ASCO Conceptual Framework ⁸	NCCN Evidence Blocks ²⁹	MSKCC DrugAbacus ¹¹	ICER Value Assessment Project ¹²
Efficacy (OS, PFS, response rate)	✓	✓	✓	✓	✓	✓	✓
Toxicity	✓	✓	✓	✓	✓	✓	✓
QoL/palliation	✓	✓	✓	✓	x	x	✓
Treatment-free interval	x	x	x	✓	x	x	x
Level of evidence	✓	✓	x	x	✓	✓	x
Patient preferences	x	x	x	x	x	x	x
Disease burden/ incidence	x	x	x	x	x	✓	✓
Unmet need	x	✓	x	x	x	✓	x
Novelty	x	x	x	x	x	✓	x
Research cost	x	x	x	x	x	✓	x
Drug costs	✓	✓	x	✓	✓	✓	x
Cost- effectiveness/ offsets	x	✓	x	x	✓	x	✓
Budget impact	x	x	x	x	x	✓	✓

✓ = included in algorithm/calculation ✓ = shown for information or comparison purposes x = not included or shown.
 ASCO = American Society of Clinical Oncology; CDF = Cancer Drugs Fund; ESMO = European Society for Medical Oncology; ICER = Institute for Clinical and Economic Review; MSKCC = Memorial Sloan Kettering Cancer Center; NCCN = National Comprehensive Cancer Network; OS = overall survival; PASKWIL = Palliative/Adjuvant effectiveness, Specific adverse events, (Kw) quality of life, Impact of treatment, and Level of evidence; PFS = progression-free survival; QoL = quality of life.

The Patient Values Project

The CCAC is embarking on a Patient Values Project (“PVP”) to better define, measure and weigh patient Values in cancer treatment.



A Question of Values

The Colorectal Cancer Association of Canada’s (“CCAC”) Patient Values Project (“PVP”) aims to define, measure and weigh Patient Values in cancer treatment.

Therapeutic innovation continues to grow as new therapies enter clinical practice. Increased survivorship is transforming the approach from acute treatment to chronic disease management.

This has resulted in a change of focus to include total patient care encompassing Quality of Life (“QoL”) issues and patient preferences.

There is also pressure for better access to affordable and effective cancer treatments, but costs have dramatically increased and there is a growing call for Value in cancer therapies. The UK, France, Germany, and Australia rely on government

Health Technology Assessment (“HTA”) bodies to recommend the value of new therapeutic options and to help determine whether or not the public system will cover the cost of a new therapy. In Canada, and increasingly elsewhere, patient group input plays an important role in this process.



Description and Purpose

The Patient Value Project aims to better define, measure and incorporate patient preferences in CRC into the evaluative HTA framework for cancer drug approval and then apply it to other disease sites both in Canada and in other countries.

The Patient Values Project will be conducted in three phases:

Phase 1 Patient preferences and values survey.

Phase 2 Analysis of the Preferences captured in the survey to identify key metrics and assign a weight to different attributes and levels.

Phase 3 Framework design and validation to become part of the pan-Canadian Oncology Drug Review (pCODR) decision making and health technology assessment process.*

Colorectal Cancer
Association of Canada

*pCODR is a pan Canadian oncology drug health technology assessment (HTA) review division of the Canadian Agency for Drugs and Technologies in Health (CADTH) that evaluates oncology drugs exclusively. The pERC is responsible for assessing the clinical value and cost-effectiveness of cancer drugs submitted for reimbursement.

Thank you to our Partners!



Elisabeth Baugh,
Ovarian Cancer
Canada



Barry Stein,
Colorectal Cancer
Assoc. of Canada



Šarūnas Narbutas,
Lithuanian Cancer
Patient Coalition



Stefania Vallone, Women
Against Lung Cancer in
Europe, Italy



Carolyn Aldigé
Prevent Cancer
Foundation, USA



Oscar Prieto Martínez,
Assoc. of Patients with
Brain Tumours, Spain



Yoshiyuki Majima,
NPO Pam CAN,
Japan



Kara Magsanoc-Alikpala,
ICANSERVE, Philippines



Luciana Holtz
Instituto Oncoguaia,
Brasil



Ignatio Zervino
Fundacion ACIAPO,
Argentina

Thank You! Merci!



ENDANGEREDBUTTS.CA

Colorectal Cancer
Association of Canada

Colorectal Cancer
Association of Canada