Patient Values In HTA

CADTH
Barry D. Stein
April 11 2016
About The CCAC

- **Colorectal Cancer Association of Canada** (“CCAC”) commenced in 1998 and incorporated in 1999 and continued under the Canada Not For Profit Act.

- First national patient association dedicated to supporting Canadians with colorectal cancer (“CRC”).

- National Board of Directors & Medical Advisory Board provide counsel ensuring members are up to date with latest medical advances in the prevention, diagnosis and treatment.

**Awareness & Education, Support, Advocacy**
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.

- **There is pressure for better access to affordable and effective cancer treatments**, but costs have dramatically increased and there has been a growing call for Value in cancer drugs.

- The UK, France, Germany, Canada, Australia rely on **government HTA bodies to determine the value of new therapeutic options** and use this assessment to help determine whether or not to cover the costs of a new therapy….others will follow.

- **Patient group input must play an important role in this process.**
The Challenge

Defining, Measuring & Weighing “Values” as they apply to new oncology treatments and drugs in general 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.
The Role of Patient Advocacy Groups In Providing Input on Value

- “Patient Values” go to the heart of quality of care and they are of paramount importance to groups providing input in HTA assessments.

- It is essential that the patient’s voice on values be clear and impactful based on well-planned and structured research showing sources and the methods used that lead to the findings.

- However, it is costly for patient groups and includes the development and carrying out of surveys, analysis of Patient Reported Outcomes (PROs), research and review of literature and drafting a submission in the context of changing rules of regulatory authorities.

- It also may involve contacting and researching patients on an international level when clinical trials are not carried out in your country.

- It is a skill that is developed over time and evolves based on the decisions made by HTA authorities.
Cancer Patient’s Input

- Individual patients and their caregivers can provide experiential evidence to expert committees about:
  - living with the illness, its nature
  - the limitations that the illness imposes on them, their families & caregivers
  - their needs and preferences in managing the symptoms and side effects of treatment.

- Unfortunately, patient views are often interpreted subjectively and may only be used to add qualitative information if cost effectiveness is confirmed.
ASKING FOR PATIENT INPUT?

“I’m writing you a prescription. Do you want a longer life with less quality or vice versa?”

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Colorectal Cancer Association of Canada
What is Value?

- There is a shift to enhancing Evidenced-Based Medicine (“EBM”) with Value-Based Medicine (“VBM”).

- 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?
ASCO has developed an algorithm that includes a definition of “value” specific to oncology in order to determine the value of different cancer treatments.

Within this framework, “value” will be defined by:

- **Clinical Benefit**: The treatment demonstrates an improvement in survival, compared with no therapy (when appropriate) or a known effective therapy, although this can sometimes differ (e.g., improvement in time to disease progression).

- **Toxicity**: The degree of treatment-associated adverse events, particularly those that affect QoL or the ability to complete usual activities of daily living. Many of which can be managed with supportive or additional treatments.

- **Cost**: Expenses incurred by patients, society, and insurers.
ASCO’s Conceptual Framework to Assess Value of Cancer Treatment Options- A Point System

• The ASCO value framework includes a **physician-guided tool** to assist the physician and patient in shared decision making.

• **Enables comparisons** of a new treatment regimen with the prevailing standard of care for a specific clinical cancer indication using data derived from a prospective randomized trial.

• **Two versions** have been developed:
  – one for metastatic or advanced cancer.
  – another for potentially curative treatment (adjuvant or neoadjuvant therapy)

• In both the frameworks, **points are awarded (or subtracted) in the categories of clinical benefit and toxicity.** In the Advanced Disease Framework, **bonus points** can be earned if a regimen shows statistically significant improvement in palliation of symptoms and/or treatment-free interval compared with the control treatment in a clinical trial.
Points accumulated on Clinical Benefit and Toxicity (and bonus points, in the advanced disease framework) are combined to generate a net health benefit (NHB) score, which is then juxtaposed against the direct cost of the treatment, to provide an overall summary assessment.
ASCO Value Framework
Advanced Disease

**THE ASCO VALUE FRAMEWORK: ADVANCED DISEASE**

**Step 1: Determine the regimen:** CLINICAL BENEFIT

<table>
<thead>
<tr>
<th>Step</th>
<th>Description</th>
<th>Formula/Calculation</th>
<th>Example</th>
<th>OS Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>1A.</td>
<td>Is the overall survival (OS) reported?</td>
<td>Yes, assign an OS Score (1 through 5 as shown below) and multiply by 15. Write this number in the box labeled “OS Score.” Proceed to I.B.</td>
<td></td>
<td></td>
</tr>
<tr>
<td>1B.</td>
<td>OS Score</td>
<td>3</td>
<td></td>
<td></td>
</tr>
<tr>
<td>1C.</td>
<td>Improvement in median OS (% change in median OS)</td>
<td>+6% +24% +54% +80% +100%</td>
<td>At double the median OS of new regimen, there is a 50% improvement in the fraction of patients surviving</td>
<td></td>
</tr>
<tr>
<td>1D.</td>
<td>OS Score</td>
<td>3</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Step 2: Determine the regimen:** CLINICAL BENEFIT - TTx/FxTx

<table>
<thead>
<tr>
<th>Step</th>
<th>Description</th>
<th>Formula/Calculation</th>
<th>Example</th>
<th>Fx Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>2A.</td>
<td>Is the toxicity of the new regimen greater than the sum of the toxicities of the old regimen?</td>
<td>Yes, assign an Fx Score (1 through 5 as shown below) and multiply by 6. Write this number in the box labeled “Fx Score.” Proceed to I.E.</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2B.</td>
<td>Fx Score</td>
<td>3</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2C.</td>
<td>toxicity score</td>
<td>≤ 120</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2D.</td>
<td>Does the new regimen represent an improvement in toxicity over the standard of care?</td>
<td>Yes, assign an Fx Score (1 through 5 as shown below) and multiply by 6. Write this number in the box labeled “Fx Score.” The maximum allowable toxicity points are 20. Proceed to I.F.</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2E.</td>
<td>Fx Score</td>
<td>3</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Step 3: Determine Bonus Points:****

<table>
<thead>
<tr>
<th>Bonus Points</th>
<th>Description</th>
<th>Formula/Calculation</th>
<th>Example</th>
<th>Bonuses</th>
</tr>
</thead>
<tbody>
<tr>
<td>3A. FALLOUT</td>
<td>Yes, if a statistically significant improvement in cancer-related symptoms is reported, award points and place this in the box labeled “Falloout Bonus Points.” Proceed to Step 3.B.</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3B. FALLOUT</td>
<td>No, no bonuses are awarded. Proceed to Step 3.B.</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3C. TREATMENT</td>
<td>Yes, if a statistically significant improvement in treatment-free interval is reported, award points and place this in the box labeled “Treatment-Free Interval Bonus Points.” Proceed to Step 3.C.</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3D. TREATMENT</td>
<td>No, no bonuses are awarded. Proceed to Step 3.C.</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3E. TOTAL BONUS POINTS</td>
<td>Add the Falloout Bonus Points (Step 3.A) and the Treatment-Free Interval Bonus Points (Step 3.B) and place this in the box labeled “Total Bonus Points.” The maximum point available for Bonus Points is 20. Proceed to Step 4.</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3F. TOTAL BONUS POINTS</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Step 4: Determine the regimen’s NET HEALTH BENEFIT:****

<table>
<thead>
<tr>
<th>Step</th>
<th>Description</th>
<th>Formula/Calculation</th>
<th>Example</th>
<th>Net Health Benefit</th>
</tr>
</thead>
<tbody>
<tr>
<td>4A.</td>
<td>Add the Clinical Benefit Score, Toxicity Score, and Bonus Points (Step 1), and Bonus Points (Step 1). This yields a Net Health Benefit Score. Write this number in the box labeled “Net Health Benefit.” The maximum points available for Net Health Benefit are 130 (100 + 30 bonus points).</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>4B.</td>
<td>Net Health Benefit</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Step 5: Determine the regimen’s COST:**

<table>
<thead>
<tr>
<th>Step</th>
<th>Description</th>
<th>Formula/Calculation</th>
<th>Example</th>
<th>Cost/Year (per 100 patients)</th>
</tr>
</thead>
<tbody>
<tr>
<td>5A.</td>
<td>Insert the drug acquisition cost (DAC) and patients covered based on the treatment regimen costs per month.</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Fig 1.** ASCO Value Framework: advanced disease. Future versions of the framework will allow for patients weighting their preferences such that the fractional contribution of each element (clinical benefit, toxicity, etc.) to the overall score can be modified, thereby individualizing the net health benefit. ASCO, American Society of Clinical Oncology; CR, complete response; DAC, drug acquisition cost; OS, overall survival; PFS, progression-free survival; PR, partial response; RR, response rate.
ESMO-Magnitude of Clinical Benefit Scale (MCBS)

The ESMO-MCBS v1.0 provides an objective and reproducible approach that allows comparisons of the magnitude of benefit between studies that incorporate different primary outcomes (OS, PFS, QoL) and different designs.

• Developed only for solid cancers.
• Accomplished through a process of variable weighting of primary outcomes and adjustments for significant secondary outcomes and toxicity.
• Based on the data derived from phase III clinical trials or meta-analyses, the tool derives a relative ranking of the magnitude of benefit that can be anticipated from a new treatment.
• A dynamic tool and its criteria will be revised on a regular basis.
The ESMO-MCBS evaluation assigns the highest grade to trials having adequate power for a relevant magnitude of benefit, and makes appropriate grade adjustments to reflect the observed magnitude of benefit.

To achieve this goal, a dual rule was implemented:

- **First**, taking into account the variability of the estimated Hazard Ratio (HR) from a study, the lower limit of the 95% Confidence Interval (CI) for the HR is compared with specified threshold values;

- **Secondly**, the observed absolute difference in treatment outcomes is compared with the minimum absolute gain considered as beneficial.
ESMO-Magnitude of Clinical Benefit Scale (MCBS)

- Given the profound differences between the curative and palliative settings, the tool is presented in two parts.
- **Form 1 is used to evaluate adjuvant and other treatments with curative intent.**
- This form is used for adjuvant and neoadjuvant therapies and for localised or metastatic diseases being treated with curative intent.
- This scale used in Form 1 is graded A, B or C. Grades A and B represent a high level of clinical benefit.
ESMO-Magnitude of Clinical Benefit Scale (MCBS)

- Form 2 forms are used for studies of new agents or approaches in the management of cancers **without curative intent**.
- Uses a scale that is graded 5, 4, 3, 2, 1, where grades 5 and 4 represent a high level of proven clinical benefit.
- Form 2a is for studies with OS as the primary outcome. Form 2a is prognostically sub-stratified for studies where the control arm produced OS greater or less than or equal to 1 year.
- Form 2b is for studies with PFS or TTP as primary outcomes. Form 2b is sub-stratified for studies where the control arm produced PFS greater or less than or equal to 6 months.
- Form 2c is for studies with QoL, toxicity or response rate (RR) as primary outcomes and for non-inferiority studies.
Visualisation of ESMO-MCB scores for curative and non-curative setting.

ESMO MCBS evaluation

Curative

Non-curative

Curative-Evaluation form 1: for new approaches to adjuvant therapy or new potentially curative therapies

Non-curative-Evaluation forms 2a, b or c: for therapies that are not likely to be curative

# ESMO-Magnitude of Clinical Benefit Scale (MCBS)

## Table 6.
Field testing ESMO-MCBS v1.0: colorectal cancer

<table>
<thead>
<tr>
<th>Medication</th>
<th>Trial name</th>
<th>Setting</th>
<th>Colorectal cancer</th>
<th>OS control</th>
<th>OS gain</th>
<th>OS HR</th>
<th>Qol. Toxicity</th>
<th>ESMO-MCBS</th>
<th>Ref.</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>FOLFOX ± panitumumab</strong></td>
<td>PRIME</td>
<td>First-line metastatic (pre/loc KRAS, KRAS BRAF WT)</td>
<td>PFS</td>
<td>7.0 months</td>
<td>1.7 months</td>
<td>0.72 (0.59–0.90)</td>
<td>18.2 months</td>
<td>9.0 months</td>
<td>0.70 (0.62–0.99)</td>
</tr>
<tr>
<td>Panitumumab ± mFOLFOX6 versus bevacizumab + mFOLFOX6</td>
<td>PEAK</td>
<td>First-line metastatic (KRAS-WT)</td>
<td>PFS</td>
<td>NS</td>
<td>24.3</td>
<td>9.5</td>
<td>0.62 (0.44–0.89)</td>
<td>4</td>
<td>[75]</td>
</tr>
<tr>
<td><strong>FOLFIRI + cetuximab</strong></td>
<td>CRISTAL</td>
<td>First-line metastatic, stratified for KRAS-WT (pre/loc KRAS, KRAS BRAF WT)</td>
<td>PFS</td>
<td>8.4 months</td>
<td>3.0 months</td>
<td>0.56 (0.41–0.76)</td>
<td>10.2 months</td>
<td>5.5 months</td>
<td>0.60 (0.54–0.80)</td>
</tr>
<tr>
<td>Cetuximab versus best supportive care</td>
<td>Refractory metastatic KRAS-WT</td>
<td>OS</td>
<td>1.9</td>
<td>1.8</td>
<td>0.4</td>
<td>0.30–0.64</td>
<td>5.5</td>
<td>4.7</td>
<td>0.55 (0.41–0.74)</td>
</tr>
<tr>
<td><strong>FOLFOX ± panitumumab</strong></td>
<td>PRIME</td>
<td>First-line metastatic KRAS-WT</td>
<td>PFS</td>
<td>8 months</td>
<td>2.0 months</td>
<td>0.80 (0.69–0.97)</td>
<td>15.4 months</td>
<td>9.6 months</td>
<td>0.63 (0.47–0.89)</td>
</tr>
<tr>
<td><strong>FOLFIRI + cetuximab</strong></td>
<td>CRISTAL</td>
<td>First-line metastatic, stratified for KRAS-WT</td>
<td>PFS</td>
<td>8.4 months</td>
<td>1.5 months</td>
<td>0.70 (0.56–0.87)</td>
<td>20 months</td>
<td>5.5 months</td>
<td>0.80 (0.67–0.95)</td>
</tr>
<tr>
<td>IL-2 + bevacizumab</td>
<td>First-line metastatic</td>
<td>OS</td>
<td>15.6 months</td>
<td>4.7 months</td>
<td>0.60 (0.54–0.81)</td>
<td>3</td>
<td>[82]</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>FOLFIRI ± panitumumab</strong></td>
<td>E3200</td>
<td>Second-line metastatic KRAS-WT</td>
<td>PFS</td>
<td>3.9</td>
<td>2 months</td>
<td>0.73 (0.59–0.90)</td>
<td>9.3 months</td>
<td>2.1 months</td>
<td>0.75 (0.60–0.99)</td>
</tr>
<tr>
<td><strong>FOLFOX ± bevacizumab versus bevacizumab alone</strong></td>
<td>Third-line metastatic after FOLFIRI</td>
<td>OS</td>
<td>10.8 months</td>
<td>2.1 months</td>
<td>0.75 (0.60–0.99)</td>
<td>2</td>
<td>[83]</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Panitumumab, versus best supportive care</td>
<td>Third-line metastatic stratified for KRAS</td>
<td>PFS</td>
<td>7.3 weeks</td>
<td>5 weeks</td>
<td>0.45 (0.34–0.59)</td>
<td>2</td>
<td>[85]</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>FOLFIRI bevacizumab versus FOLFOXIRI bevacizumab</strong></td>
<td>First-line metastatic</td>
<td>PFS</td>
<td>9.7 months</td>
<td>1.5 months</td>
<td>0.75 (0.62–0.90)</td>
<td>N/A</td>
<td>2</td>
<td>[86]</td>
<td></td>
</tr>
<tr>
<td>TAS-102 versus placebo</td>
<td>CONCOURSE</td>
<td>Third-line or beyond metastatic</td>
<td>OS</td>
<td>5.3 months</td>
<td>1.9 months</td>
<td>0.68 (0.50–0.90)</td>
<td>18.6 months</td>
<td>6 months</td>
<td>0.69 (0.59–0.81)</td>
</tr>
<tr>
<td>Regorafenib versus placebo</td>
<td>CORRECT</td>
<td>Third-line metastatic</td>
<td>OS</td>
<td>5</td>
<td>1.4 months</td>
<td>0.77 months</td>
<td>0.64–0.94</td>
<td>2</td>
<td>[88]</td>
</tr>
<tr>
<td>Second-line chemotherapy ± bevacizumab</td>
<td>HL10147</td>
<td>Second-line progression on bevacizumab</td>
<td>OS</td>
<td>9.6 months</td>
<td>1.5 months</td>
<td>0.81 (0.69–0.94)</td>
<td>1</td>
<td>[89]</td>
<td></td>
</tr>
<tr>
<td><strong>FOLFIRI ± afibricapte</strong></td>
<td>VELOUR Second-line after oxaliplatin-based treatment</td>
<td>OS</td>
<td>4.7 months</td>
<td>2.2 months</td>
<td>0.76 (0.66–0.87)</td>
<td>12.1 months</td>
<td>1.5 months</td>
<td>0.82 (0.71–0.94)</td>
<td>1</td>
</tr>
<tr>
<td><strong>FOLFIRI ± Ramucirumab</strong></td>
<td>RAISE Second-line metastatic after bevacizumab, oxaliplatin, fluopyrimidine</td>
<td>OS</td>
<td>11.7 months</td>
<td>1.6 months</td>
<td>0.84 (0.73–0.97)</td>
<td>1</td>
<td>[91]</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*Unbalanced crossover.*
Patient Group Input In HTA - Canadian Context

- The Canadian context is different from the US and European context due to our health care system and societal values, but may come under pressure from the ASCO algorithm and ESMO guidelines.

- Patient Values are inherent to the Patient Group Submission in the Pan-Canadian Oncology Drug Review (“pCODR”/ CADTH) process in which accredited patient groups are asked to provide their input on new drugs being evaluated primarily from an experiential point of view.
The HTA process in cancer drugs has evolved in Canada as a consequence of pCODR/CADTH.

pCODR has enabled patient groups to provide input as part of the criteria to be evaluated by expert committees when deciding on whether or not to recommend the reimbursement of new cancer therapies to Provincial/Territory Health Ministries.

Q. Will this continue?

Q. Will this extend to the Common Drug Review (CDR) for all drugs?

Q. Will INESSS in Quebec adopt this approach?
Patient Evidence

- **Cancer patient views are distinct** from that of the other stakeholders and from that of the average citizen who has not experienced a life threatening illness.

- **Patients and their caregivers can provide experiential evidence** to expert committees about living with the illness, its nature, the limitations that the illness imposes on them and their families and caregivers.

- **Compiling patient and caregiver information on patient needs, preferences and what is of value to them and their caregivers** in the context of assessing a particular drug is not an easy task, but may have an impact on drug approval and reimbursement.
pCODR Review Process

http://www.pcodr.ca/idc/groups/pcodr/documents/pcodrdocument/pcodr_content_review_pdf.pdf
Data Collection and Analysis
Sample of CCAC patient survey

Part 2: Expectations for the new drug being reviewed
Only answer the following questions if you are not currently on the drug under review and have had no previous experience using the drug.

Question 8: How much do you know about [drug name]?
   1  2  3  4  5  6  7
   (Nothing)  (Extremely knowledgeable)
   ○ ○ ○ ○ ○ ○ ○ ○

Question 9: On a scale of 1 (extremely unimportant) to 7 (extremely important), which of the following symptoms are the most important for [drug name] to manage.

<table>
<thead>
<tr>
<th></th>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>(Extremely unimportant)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

[Symptom] ○ ○ ○ ○ ○ ○ ○ ○
[Symptom] ○ ○ ○ ○ ○ ○ ○ ○
[Symptom] ○ ○ ○ ○ ○ ○ ○ ○
[Symptom] ○ ○ ○ ○ ○ ○ ○ ○
[Symptom] ○ ○ ○ ○ ○ ○ ○ ○
Other: ______

Question 10a: Which of the following side effects would you be willing to tolerate if [drug name] were to improve your overall daily functioning.

[Side effect] [Side effect] [Side effect] [Side effect] [Side effect] [Side effect] Other
   ○ ○ ○ ○ ○ ○

Question 11: Please describe your overall experience with [drug name].

Question 12: Please select the symptoms of [specific cancer] that [drug name] manages or managed better than previous therapies you have used.

[Symptom] [Symptom] [Symptom] [Symptom] [Symptom] [Symptom] [Symptom] Other
   ○ ○ ○ ○ ○ ○

Question 13: Which of the following side effects have you experienced as a result of using [drug name].

[Side effect] [Side effect] [Side effect] [Side effect] [Side effect] [Side effect] [Side effect] Other
   ○ ○ ○ ○ ○ ○

Steps
1)  Create survey questions
2)  Send to cohort of recruited patients
3)  Analyze data internally
Data Presentation to pCODR

pCODR Presentation Template

; Patient Advocacy Group Input on a Drug Review

2.1 Experience Patients Have with This Type of Cancer

The diagnosis of cancer impacts all aspects of patients’ lives. Furthermore, different cancers, and stages of cancer, affect patients in different ways. Recognizing this, the focus of the information requested in this section relates to the impact of the cancer for which the drug under review is indicated. What are the symptoms and problems associated with this cancer that impact a patient’s day-to-day life and quality of life? Examples of the type of information that could be included are:

- Which aspects (e.g., cough, pain, edema, appearance) of this cancer are more important to control than others?
- How do ongoing symptoms affect day-to-day life?
- Describe any limitations as a result of the cancer.

You may delete the instructions and examples under each heading for more space.

2.2 Patients’ Experience with Current Therapy

How well are patients managing their disease with currently available treatments? Examples of the types of information that might be included are:

- What therapies are patients using to treat this type of cancer?
- How effective is the current therapy in controlling the common aspects of this cancer, e.g., pain, fatigue?
- What are common adverse effects and are some more difficult to tolerate than others?
- Would patients be willing to tolerate potential adverse effects resulting from treatment, if the benefits were only short-term?
- Are there hardships in accessing current therapy? Can patients readily access available treatments in their own communities?
- In addition to the drug cost, are there other financial implications to patients or caregivers (e.g., traveling costs, drug disposal issues, drug administration supplies)?
- Are there needs, experienced by some or many patients that are not being met by current therapy? What are these needs?

You may delete the instructions and examples under each heading for more space.

Sections include:

- Information about the advocacy group
- Experience patients have with this type of cancer
- Patients’ experience with current therapy
- Impact on caregivers
- The expectations for the new drug
- Experiences patients had to date with the new drug
- Conflict of interest declarations
The CCAC is embarking on a Patient Values Project (“PVP”) to better define, measure and weigh patient Values in cancer treatment.
The Patient Values Project

CONTEXT

• The high costs of cancer drugs has resulted in increased pressure on the healthcare system to make better choices and adopt value models when considering drug reimbursement or coverage as well as treatment choices.

• The financial strainwhether borne by governments, insurers or patients directly, may make healthcare less affordable and may ultimately deprive patients access to the healthcare they require.

• Facing this dilemma, both the American Society of Clinical Oncology (ASCO) and the European Society of Clinical Oncology (ESMO) have developed independent Value Frameworks to assess the value of cancer drugs, but they do not consider patient preferences.

• While there are many stakeholders in the healthcare system, the perspective of the patient is central to the definition of value, yet it is likely the least understood and most difficult to measure.

• Attributing what weight Patient Values should carry in reimbursement and coverage decision making is challenging.
The Patient Values Project
Patient Preferences

- Individual patient perceptions of value may be subjective and vary with different treatment options and depending on whether they are curative or non-curative options or cross over.

- Furthermore patient preferences may change over time and vary due to age, comorbidities, life circumstances, personal finances, individual goals, religious beliefs and other values.

- Patient’s needs, goals and preferences must be taken into account in a dynamic framework, yet little work has been done to define, measure and attach weight to patients values.

- To further complicate this assessment patients do not gather or interpret their information in the same way as in the past, with information arriving at an accelerating rate on a daily basis.

- Both the ASCO Value Framework and the ESMO (MCBS) are dynamic and may be modified to take into account better patient weighting in the future.

- Previous validated assessment tools on patient preferences and values may not have evolved sufficiently to take account of these changes.

The Patient Value Project aims to address these issues.
Patient Values Project Objectives

- Develop a better **definition** of patient Values and determine the appropriate **metrics to measure** these Values,

- **Empower Patient Groups** to be better able to provide research based input to assist expert committees in the evaluation of a drug or treatment.

- **Include a better understanding of the patient perspective** allowing for better quality in patient group submissions in HTA.

- **Allow for a more reasoned and balanced rationale** in the assessment of new cancer drugs by the expert committees.

- **Provide objective and quantifiable input** on patient Values based on validated research techniques.

- **Assign an appropriate assignable Weight to the measured Values** that can form part of an expert HTA Committee decision in drug evaluation.
PVP Co-Chairs

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Psychosocial Oncologist

Dr. Deborah Marshall
HTA Expert

Dr. Judith Glennie
HTA Expert

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Barry D. Stein president CCAC
Jiahui Wong Scientist de Souza Institute
Feng XIE McMaster
Observer: Mona Sabharwal
Two approaches:

A. Develop metrics that captures patient values ("PV") so that an instrument could be used to measure the benefits of a treatment, which would then be reported to decision making bodies. We would advocate that decision making body utilize the PV and that it carry a specific weight/percentage as part of their drug reimbursement recommendation.

B. Identify the principle components of PVs and develop a hierarchy of evaluable clinical social and other outcomes ("HECSO") that would be ranked by relevance of the PV’s. The HECSO would be the guiding document for those conducting clinical trials to determine the outcomes that they should measure and report on in their clinical trials. We would advocate that decision making bodies would utilize the HECSO as the relevant determinants of whether the drug is worth recommending.

General Questions:

i. What is the most up to date description/definition of Patient Values ("PV") in terms of what patients value in their oncology treatments?

ii. What are the appropriate metrics to attach to PVs so that they can be best measured and reported on for the purposes of HTA (pCODR/INESSS) bodies? (A)

iii. What is/are the best assessment tool(s) utilizing these metrics to measure PVs, so that a single score (value) can be determined to represent these metrics? (A)

iv. Does a new assessment tool based on the discrete choice experiment ("DCE") better reflect PVs in the current context? (A)

v. What percentage of the HTA expert committee decision on reimbursement recommendations for oncology drugs should be based on this value (in addition to anecdotal input)? (B)

vi. What are the outcomes that can be measured that are valued by patients being treated for cancer, and what is their hierarchy (B)
Research Questions:

Phase 1 (survey):
1a. How do colorectal cancer patients (early stage and metastatic) value different aspects of treatment when weighing the associated benefits and risks?
1b. How do values differ based on patients’ demographics, quality of life, stage of cancer and experiences?
1c. What are the importance weights for the attributes of treatment decisions?

*Treatment refers to drugs and medications specifically and does not include surgery or other cancer treatment options.

APPROACH: Survey

Phase 2 (development of key metrics/indicators to measure values captured in survey data):
2a. What combination of attributes in treatment decisions provide colorectal cancer patients with the greatest personal utility?
2b. How can we apply the attributes to inform a framework for drug reimbursement decisions?

APPROACH: Consultation with experts to develop Patient Values Framework

Phase 3 (generate/assign a weight to patient values to become part of the patient submission to pERC (PCODR expert review committee)):
3a. How could these patient values be explicitly incorporated into the current HTA Agency evaluative process for new (colorectal cancer) drug treatments?
3b. What weight (or proportion) based on the Patient Values metric should be allocated for the Patient Values Submission of the reimbursement decision for oncology drugs?
3c. How does including Patient Values as developed in our study impact drug reimbursement decisions?

APPROACH: Consultation with experts to develop framework, focus groups and interviews
Project Steps

Determine what CRC patients Value through use of a metrics-based SURVEY and anecdotal interviews (Publish)

Develop metrics and measure the Values captured in the SURVEY

Assign Weight to the Values so a more objective input may be submitted to expert committees

Adapt the SURVEY to other cancer sites

Develop Consensus Statement For Publication
University of Calgary (UC)

• Literature searches to be performed independently by and UC and Harvard Group

• Develop, disseminate & analyze the Survey on patient Values starting with a pilot program in one Canadian City. UC to develop DCE and SCC the balance based on validated assessment tools.

• The survey will be furnished through a phone app (Patient Storylines™) on proprietary software of Self Care Catalysts & personal interviews for those unable to access the software on smart phones or PC.

• Patient Storylines™ transforms real time, real world data into meaningful analytics that fills the current gap in knowledge to identify gaps in care.

• In Phase II the appropriate metrics will be determined to measure the patient values in order to better understand and utilize same in patient submissions to HTA committees.
Patient Values Survey

The Survey components:

• EQ-5D

• EORTC Questionnaires
  a. Non metastatic CRC (EORTC QLQ – CR29)
  b. Metastatic CRC (EORTC QLQ – LMC21)

• Discrete Choice Experiment (DCE)

• Carry out survey via Web App, Smart Phone App, (Patient Storylines), and Face to Face.
Phases I-II

Phase I
Literature Search
Develop Survey
i. Background information, experience questions, demographic questions, quality of life questions and the Discrete Choice Experiment (DCE).

ii. The Survey will measure quality of life and use the DCE to measure trade-offs and how patients value certain attributes in treatment decisions specific to metastatic colorectal cancer as this seems to be where the key issues in treatment choices are. The stage of the disease will be noted in the Survey to distinguish variances between early and late stage patients.

iii. The deliverables from the Survey will be a final Survey and a published manuscript.

iv. The results of the Survey will provide evidence for Phase II of the project.

Phase II
Development of key metrics/indicators to allow for measuring of values captured in Survey data
i. This Phase of the project will involve developing a framework for using key patient value metrics/indicators for drug treatment funding based on the Survey data.

ii. To be carried out while waiting for publication of Phase I

Phase III
Generate/assign a weight/score to the Patient Values to become part of the patient submission to HTA authorities such as the pCODR/CADTH expert review committee (pERC)

i. This phase of the project will involve developing recommendations for what weight/score patient values should have in patient submissions.

ii. To be carried out while waiting for publication of Phase I
Phase III

- Phase III of the project includes the determination of what weight should be given to patient “Values” by HTA expert review committees.
- The creation of a consensus statement to recommend the appropriate weight that should be given to patient Values by specific HTA expert committees through in-person meetings.
- The process is dynamic and will adapt as the project unfolds as new drugs are introduced.
Patient Values Interviews

• Anecdotal Interviews with Photos and Web/FB Page

• Record anecdotal stories from interviews based on photos and short simple questions put to patients on what they value or are looking for in their cancer treatments.

• The style it will take may be that of “Humans of New York” or “Faces of Healthcare” with videos and stills and may be reflected on a Web or Facebook page.

• Groups participating worldwide:
  i. Adapt the survey in the colorectal cancer in other countries.
  ii. Adapt the survey to other cancer sites.
  iii. Add anecdotal photos and interviews worldwide.
  iv. Combine all the information on one site and compare differences between countries.
An International panel of patient organizations representing different cancer disease site groups in the USA, Japan, Lithuania, Argentina, Brazil, Canada, Italy, the Philippines and Spain and the UK, Australia & New Zealand have similar interests and have expressed interest in working together on the Patient Values Project.

Toronto 2014  
Milan 2015  
Copenhagen/ Singapore
Conclusions

• Therapeutic innovation continues to grow as targeted therapies enter clinical practice.

• Patients have a vested interest & their input must play an essential role in determining the “Value” of cancer treatments in the entire drug approval process from the initial planning of clinical trials and on.

• Patient Values need to be better defined and measured and weighed to have better impact.

• VBM must aim to help identify those drugs that offer the most overall Value to patients in accordance with what patients actually value.

• Unequal access to drugs that demonstrate Value is unacceptable.
What you have to do to get your point across
Thank You!

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