Patient Values and Preferences in Health Technology Assessment

Barry D. Stein
Colorectal Cancer Canada
Global Action for Cancer Patients
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 and Values.

There is pressure for better access to affordable & effective cancer treatments and a growing call for Value in cancer drugs.
Cancer Drugs Present 3 Sustainability Challenges*

Growth in the cancer drug pipeline:

Over 150 new cancer therapies and 300 new drug indication pairs under investigation in clinical trials.

Increase in the number new cancer cases in Canada:

By 2030, cancer cases are expected to grow by 40%.

Survival rates continue to improve.

There is an increasing rate of recurrent and new primary cancer diagnoses.

Rising Provincial Oncology Drug Budgets:

Oncology drug budgets tripled in less than a decade. Increasing between 12-15% annually.

Expanding lines of treatment, simultaneous use of multiple cancer drugs, targeted therapies and immunotherapies.

*January 19, 2017 - Canadian Association of Provincial Cancer Agencies
WHAT IS VALUE?

Value Based Medicine 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?
Why Engage Patients & Caregivers In HTA? (Value?)

Patient and Citizen Involvement Interest Group. HTAi.org

**RELEVANCE**
Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.

**FAIRNESS**
Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.

**EQUITY**
Patient engagement in HTA contributes to equity by seeking to understand their diverse needs with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.

**LEGITIMACY**
Patient involvement facilitates those affected by the HTA recommendations to participate in the process; contributing to the transparency, accountability and credibility of the decision-making process.

**CAPACITY BUILDING**
Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.
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 outcomes.

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.
Supporting the provinces and aligning with pCODR and pCPA

* INESSS is not directly linked to pCPA.
1. About your Patient Group.
3. Disease Experience.
4. Experience with Currently Available Treatments
5. Improved Outcomes.
6. Experience with the Drug Under Review.
7. Companion Diagnostic Test.
9. Anything Else?
10. Conflict of Interest declaration.

https://cadth.ca/pcodr-update-61
PATIENT GROUP INPUT is reviewed by pERC (pCODR Expert Review Committee) as part of the deliberative framework to assess a cancer drug for reimbursement.

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

- Clinical Benefit
- Economic Evaluation
- Adoption
- Feasibility

Patient-Based Values
PATIENT EVIDENCE

Experiential Evidence in respect of:

- Living with Illness
- Nature of Illness
- Needs & Expectations of Treatment
- Limitations that illness/Tx imposes on Pt, family or caregiver
### Framework Tool and Value Items

<table>
<thead>
<tr>
<th>Value Item</th>
<th>Framework Tool</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>PASKWIL*</td>
</tr>
<tr>
<td>Efficacy (OS, PFS, response rate)</td>
<td>✓</td>
</tr>
<tr>
<td>Toxicity</td>
<td>✓</td>
</tr>
<tr>
<td>QoL/palliation</td>
<td>✓</td>
</tr>
<tr>
<td>Treatment-free interval</td>
<td>✓</td>
</tr>
<tr>
<td>Level of evidence</td>
<td>✓</td>
</tr>
<tr>
<td>Patient preferences</td>
<td>X</td>
</tr>
<tr>
<td>Disease burden/ incidence</td>
<td>X</td>
</tr>
<tr>
<td>Unmet need</td>
<td>X</td>
</tr>
<tr>
<td>Novelty</td>
<td>X</td>
</tr>
<tr>
<td>Research cost</td>
<td>X</td>
</tr>
<tr>
<td>Drug costs</td>
<td>✓</td>
</tr>
<tr>
<td>Cost-effectiveness/ offsets</td>
<td>X</td>
</tr>
<tr>
<td>Budget impact</td>
<td>X</td>
</tr>
</tbody>
</table>
The CCC is embarking on a Patient Values Project (“PVP”) to better define, measure and weigh patient Values in cancer treatment.
General Objectives

DEVELOP A BETTER DEFINITION OF PATIENT VALUES, DETERMINE THE APPROPRIATE METRICS TO MEASURE THESE VALUES, ASSIGN AN APPROPRIATE WEIGHT TO THESE VALUES THAT CAN BE USED BY THE EXPERT COMMITTEE IN THE HTA DECISION MAKING PROCESS.

• Empower Patient Groups to provide research based input to assist expert committees in the evaluation of a drug or treatment.

• Encourage better quality Patient Group submissions in HTA.

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

• Provide objective and quantifiable input on Patient Values based on validated research techniques.
PATIENT VALUES PROJECT PHASES

Phase I
- SURVEY PATIENT VALUES AND PREFERENCES

Phase II
- DEVELOP KEY INDICATORS AS CAPTURED IN SURVEY DATA

Phase III
- DETERMINE WEIGHT TO BE ATTRIBUTED TO PATIENT VALUES IN CONTEXT OF CDN HTA IN ONCOLOGY DRUG REVIEWS
Survey Population to be Recruited

Survey population includes Canadian adults (18 years of age and older)

- Patients with non-metastatic CRC (n=300)
- Patients with metastatic CRC (n=300)
- Caregivers of patients with either non-metastatic or metastatic CRC (n=300)
- People from the General Population that are not patients or caregivers (n=300)
Thank You! Gracias! Obrigado! Grazie! Merci! ありがとうございました！Ačiū! Salamat! Terima Kasih!
Cross border efforts to improve patient care in Japan

Pancreatic Cancer Action Network Japan
President Yoshiyuki Majima
National Expenditures
10B USD

SOURCE: National Institute of Population and Social Security Research
Revolutionary new drugs with high price tag have arrived
WHO Resolution A67/33

- Many countries currently lack the capacity to assess the merit of HTA.

- HTA main purpose is to inform technology related policy making healthcare and thus improve the uptake of cost-effective new technologies and prevent the uptake of technologies that are of doubtful value in the healthcare system.

- Wasteful spending on medicines and other technologies has been identified as major cause of inefficiencies in the health service delivery.
## HTA in Asian Countries

<table>
<thead>
<tr>
<th>Country</th>
<th>Agency</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>CHINA</td>
<td>Various university centers</td>
<td>アカデミア</td>
</tr>
<tr>
<td>MALAYSIA</td>
<td>MaHTAS (HTA Section) and HTAU (HTA Unit)</td>
<td>厚生省/政府</td>
</tr>
<tr>
<td>PHILLIPPINES</td>
<td>National Center for Pharmaceutical Access and Management</td>
<td>健康局/政府</td>
</tr>
<tr>
<td>SINGAPORE</td>
<td>Research &amp; Technology Assessment Department</td>
<td>厚生省/政府</td>
</tr>
<tr>
<td>ROK</td>
<td>Health Insurance Review &amp; Assessment Agency / National Evidence-based Healthcare Collaboration Agency</td>
<td>厚生省/政府</td>
</tr>
<tr>
<td>TAIWAN</td>
<td>HTA Division – Center for Drug Evaluations</td>
<td>健康局/政府</td>
</tr>
<tr>
<td>THAILAND</td>
<td>HiTAP (Health Intervention &amp; Technology Assessment Program)</td>
<td>厚生省/政府</td>
</tr>
</tbody>
</table>

Source: HTA Workshop - Role of HTA in Asian Pacific Region, Madeleina Valeria
HTA is used not for drug approval, initial pricing, but for drug pricing adjustment using ICER

否の判断を行う場合の具体的な取り扱いなどを挙げた。

総会に先だって閉ざされた費用対効果評価専門部会では、土屋裕専門委員（エーザイ代表執行役）が、企業にとって新たな負担になると指摘した上で「薬価の調整では、引き上げも含めた評価をお願いしたい」と求めた。
New drug pricing process (2016)

Marketing Approval

Request for entry into the NHI Price List

1st DPO (Drug Price Organization)

Notice of calculated drug price

- Objection, No
- Objection, Yes

Appeal of dissatisfaction

2nd DPO

Notice of decision

- Hearing on marketing authorization holder’s opinion, if requested
- ≤60 days, as a rule
- ≤90 days at the latest

HTA

CE Evaluation Expert Group

Reporting of the NHI price to and consent from Chuikyo

No Objection, Yes

HTA listing (4 times/yr)
1. 保険者、被保険者、事業主を代表する委員

<table>
<thead>
<tr>
<th>姓名</th>
<th>所属</th>
</tr>
</thead>
<tbody>
<tr>
<td>矢内 邦夫</td>
<td>全国健康保険協会健保組合連合会</td>
</tr>
<tr>
<td>白川 修二</td>
<td>全国健康保険協会健保組合連合会</td>
</tr>
<tr>
<td>花井 彰子</td>
<td>日本労働組合総連合会</td>
</tr>
<tr>
<td>花井 千恵</td>
<td>日本労働組合総連合会</td>
</tr>
<tr>
<td>石山 恵司</td>
<td>全日本海員組合副組合長</td>
</tr>
<tr>
<td>田中 伸一</td>
<td>愛知県津島市長</td>
</tr>
<tr>
<td>伊藤 文郎</td>
<td>日本労働組合総連合会</td>
</tr>
</tbody>
</table>

2. 医師、歯科医師及び薬剤師を代表する委員

<table>
<thead>
<tr>
<th>姓名</th>
<th>所属</th>
</tr>
</thead>
<tbody>
<tr>
<td>鈴木 邦彦</td>
<td>日本医師会常任理事</td>
</tr>
<tr>
<td>安達 秀樹</td>
<td>日本医師会社会保険診療報酬検討委員会</td>
</tr>
<tr>
<td>中川 俊男</td>
<td>日本医師会副会長</td>
</tr>
</tbody>
</table>

3. 公益を代表する委員

<table>
<thead>
<tr>
<th>姓名</th>
<th>所属</th>
</tr>
</thead>
<tbody>
<tr>
<td>印南 一路</td>
<td>資料義塾大学総合政策学部教授</td>
</tr>
<tr>
<td>中丸 俊</td>
<td>吴唱田大学政治経済学部教授</td>
</tr>
<tr>
<td>関原 健夫</td>
<td>日本対がん協会常務理事</td>
</tr>
<tr>
<td>西村 万里子</td>
<td>明治学院大学法学部教授</td>
</tr>
<tr>
<td>木村 直</td>
<td>日本大学政治経済学部教授</td>
</tr>
<tr>
<td>関原 健夫</td>
<td>学習院大学法学部教授</td>
</tr>
</tbody>
</table>

4. 専門委員

<table>
<thead>
<tr>
<th>姓名</th>
<th>所属</th>
</tr>
</thead>
<tbody>
<tr>
<td>藤原 忠彦</td>
<td>長野県川上村長</td>
</tr>
<tr>
<td>井田 一哉</td>
<td>日本看護協会常任理事</td>
</tr>
<tr>
<td>島田 丸</td>
<td>日本臨床検査技師会会長</td>
</tr>
<tr>
<td>中村 佳明</td>
<td>千葉大学医学部附属病院歯科・顔・口腔外科教授</td>
</tr>
<tr>
<td>加藤 佳</td>
<td>塩野義製薬株式会社</td>
</tr>
<tr>
<td>増 一</td>
<td>やすや株式会社</td>
</tr>
<tr>
<td>吉村 恭彦</td>
<td>株式会社アストフォアストラゼネカ株式会社</td>
</tr>
<tr>
<td>田村 慶子</td>
<td>アポロジャパン</td>
</tr>
<tr>
<td>十河 功二</td>
<td>株式会社イノメディック</td>
</tr>
</tbody>
</table>

No Patient Advocates in the list
Mock HTA panel to discuss HTA Appraisal process (2016)

OVERVIEW:
- Appointing those with medical and economic backgronds to panel members and discuss opinions about Appraisal Process of HTA.

DISCUSSION:
- Concept of "Fair Inning Argument (FIA)" to deny treatment to elderly when competing with younger patients for resource?
- Saving lives suffering from deadly disease is ethical thing to do?
- Conditions to allow expensive and low cost-effective treatment

Clinicians (2)  Health Economist(2)  Citizen (Graduate Student) (1)
Bioetheist (1)  Nurse (1)

出典
中医協
平成27年6月24日
After listing of new drug, following process can be considered

- **After marketing**
- **Data submission by drug company**
- **Re-analysis**
- **Appraisal**
- **Chuikyo Approval**
- **Drug Price Change**

品目が選定された後、一定の準備期間を設ける
再分析の実施に一定期間を設ける
保険収載後、1回目～数回目の改定にあわせて実施することが考えられるのではないか。
Why patient (and caregiver) input to HTA is important
MHLW: Decides Initial Drug Price by Referencing Oversea Average Price

NICE  HAS  DIMDI
Reference pricing has an impact on drug access

Thanks to Panos Kanavos, LSE
### HTA Trial Evaluation of 7 Drugs

<table>
<thead>
<tr>
<th>Drug</th>
<th>Disease</th>
<th>Technology</th>
<th>ICER</th>
<th>Ethical/Social Factors</th>
<th>Reassessment Analysis</th>
</tr>
</thead>
<tbody>
<tr>
<td>SOVALDI</td>
<td>Hepatitis C</td>
<td>Anti-viral</td>
<td>(&lt; 5M/QALY)</td>
<td></td>
<td>No Adj</td>
</tr>
<tr>
<td>HARVONI</td>
<td>Hepatitis C</td>
<td>Anti-viral</td>
<td>(&lt; 5M/QALY)</td>
<td></td>
<td>Price Adj</td>
</tr>
<tr>
<td>VIEKIRAX</td>
<td>Hepatitis C</td>
<td>Anti-viral</td>
<td>(&lt; 5M/QALY)</td>
<td></td>
<td>Price Adj</td>
</tr>
<tr>
<td>Interferon</td>
<td>Hepatitis C</td>
<td>Interferon</td>
<td>(&lt; 5M/QALY)</td>
<td></td>
<td>Price Adj</td>
</tr>
<tr>
<td>ChemoTx</td>
<td>Melanoma, NSCLC</td>
<td>ChemoTx</td>
<td>(&lt; 5M/QALY)</td>
<td></td>
<td>Price Adj</td>
</tr>
<tr>
<td>(ASUNAPREVIR)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>(Daclatasvir)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>(OPDIVO)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>(KADCYLA)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

#### Price of Opdivo and Kadcyla

The price of Opdivo and Kadcyla have been singled out for price cut adjustment.
HTA process and Appraisal

Assessment (分析)
- 有効性、安全性等の評価
- 費用対効果評価
- 増分費用効果比 (ICER)
- 具体例 英、独、その他
- 承認・価格交渉、保険償還

Appraisal (総合評価)
- ICER 分析結果の解釈
- その他（臨床的、倫理的、社会的要素）影響の考慮

Decision (最終決定)
- 最終的な意志決定

Source: ISPOR 2013 Consumer Advocate Network
An important difference

- **Assessment**
  - What benefit does it bring?
  - To which patients?
  - For how long?
  - With what unwanted/side effects?
  - What safety profile?
  - [At what cost?]

- **Appraisal**
  - Assessment in national/regional/local context
  - How would it fit into this healthcare system’s priorities?
  - Is it worth it?
REQUEST FOR PATIENT PARTICIPATION IN HTA PROCESS

TO: Sakoi Masami, MD, MPH
MHLW Health Insurance Bureau Medical Economics Division

FROM: PanCAN Japan, CSR Project

1. Include patient advocates in HTA committee of Chuikyo
2. Include experienced patient advocates in Appraisal Mock Panel
3. Maintain transparency in setting ICER threshold
4. Provide and support patient education program for Appraisal participation
Liberating the NHS:

No decision about me, without me

Government response
Patient involvement in HTA is a must, especially in Japan where advocates are not engaged at all in HTA setting.

Clinical trial outcomes only provide a part of evidence. Understanding patient/caregivers experiences of the disease is critical, especially the use of PRO is very limited in Japan.

Guaranteeing rapid “Access” to new drugs is more important than creating better “Value” through HTA given NHI pricing scheme.

MHLW should provide more transparent approach to HTA and HTA training to patient advocates.
Our fight: Get the Seat at the HTA Table
Value of Cancer Care and Caring about Cancer

World Cancer Congress
October 2, 2018

Afsan Bhadelia, Research Associate
Harvard T.H. Chan School of Public Health
Research Objectives

• Contribute to improving understanding of the multidimensionality of the value of health

• Promote inclusion of patient perspective into normative views of value of care

• Develop a dynamic model of the value of cancer care and control, considering accrual of costs and benefits over time, across trajectories of care and from perspective of diverse stakeholders, including family/care givers, as well as society more broadly

• Provide recommendations on patient engagement for improving cancer care and caring about cancer, and health systems strengthening
Figure 1: Dynamic Model for Value of Cancer Care

SOCIETY

FAMILY/CAREGIVER
- Reward of meeting patient need
- Spend time with family member
- Satisfaction of caring

PATIENT
- QoL
- Dignity
- Hope
- Respect
- Empathy
- Security
- Worth
- Comfort
- Knowledge
- Meaning
- Freedom

CARE
- Ease process of saying good-bye
- Fear
- Social morbidity
- Anxiety
- Grief

BENEFITS OF CARE
- Improved population health
- Responsiveness
- Financial protection
- Meet cultural expectations
- Feel right thing to do
- Productivity gain
- Productivity & income loss
- Loss of savings
- Catastrophic health expenditure
- Impoverishment
- Lower level of physical health
- Lower level of mental health
- Limited leisure
- Health care expenditure

COSTS OF INADEQUATE CARE
- Premature mortality
Recommendations for Policy and Practice

• Promote **development of metrics** to capture patient value of care (cancer and beyond) to strengthen priority-setting frameworks

• Gather **evidence from diverse contexts**, particularly low and middle-income countries, to understand complexity of cultural norms, attitudes and perceptions around value of care

• Advance **advocacy agenda for civil society** to address gaps in knowledge

• **Engage patients/patient-advocates/patient advocacy groups**, care givers, and related associations

• Promote **network to undertake coordinated action**

• Mobilize a movement
Enabling Actions

• Conduct global mapping of stakeholders around value of cancer care and identify strengths, weaknesses, opportunities, and threats

• Garner funding for research, advocacy, and programmatic activities to advance efforts on value of care

• Develop a committed space for policy-oriented research focused on patient-centered care

• Train patient/care giver groups on effectively engaging in advocacy, as well as research and policy efforts more directly

• Organize opportunities for diverse stakeholders to conduct shared learning
Next Steps

• Validate framework through comprehensive consultation and review by patient groups
• Conduct qualitative research on range of expressed values around care from different cultures, health systems, and/or stakeholders to fill gap in knowledge
• Focus on neglected areas (e.g. palliative care)
Global Action for Cancer Patients

Towards global equity in cancer care

Carolyn Aldigé, Chair, GACP

2 October 2018
GACP CURRENT MEMBERS

**Steering Committee**
Carolyn Aldigé, USA (Chair)
Yoshiyuki Majima, Japan
Šarūnas Narbutas, Lithuania
Ignacio Zervino, Argentina
Stefania Vallone, Italy (Treasurer)
Thanks to Ansgar Hebborn
HTA and value frameworks

• Most of these frameworks were developed without active involvement of patients or patient organisations

• Frameworks often do not include the patient perspective as a component of value estimations
In 2014 GACP defined a plan to change this:

1. Literature search on patient perceptions of value in cancer care
2. Consultation process
3. Develop a definition of ‘value’ in cancer care from a patient perspective, and metrics required to measure it
   - Patient rights
   - Quality of life
   - Productivity
   - Cost to society
4. Publish paper on new definition / metrics
5. Online platform containing all resources
6. Implement global / local advocacy training

Advocate for the new definition to be incorporated into policy (e.g. HTA) in practice.
‘We want to ensure that patient values drive decision making in cancer care’
GACP Achievements

Contributed to session at 2016 World Cancer Congress
Paper published in Frontiers in Pharmacology, October 2017
*Overview on Patient Centricity in Cancer Care, Narbutas et al*

Patient Values in HTA: preliminary research concluded in Canada to support standardised method of presenting evidence-based patient values
Harvard Global Equity Group developed ‘*Dynamic model of cancer care*’ (*in press*)
Life worth living: Communicating the value of treatment in visual narrative form
Session at Health Technology Assessment International meeting
Multiple funders: Roche, BMS & Novartis
GACP Current & Future Plans

Strategic plan in place 2018-2019
Secretariat established in UK
Website in development
Three more narrative videos in progress
**Advocacy toolkit to support their use**

Session at Health Technology Assessment International meeting
Future projects on the theme of patient value in cancer care in development
GOAL: Global Oncology Advocacy Leaders

Meetings and outputs funded by BMS
GOAL Achievements
Summary

• Patients and patient groups believe that the patient perspective is the basis of good decision making about access to high quality cancer care
• A global approach is needed to ensure equity of access
• GACP is leading the efforts to make this happen
Access to treatment is access to life!
Global perspective on value

No frameworks about us without us!

✓ Depends on many individual factors (age, country, education, family)
✓ Changes over time (prognosis, co-morbidities)
✓ About more than just treatment (quality of life, social support, nursing care)
✓ We are not cost-agnostic (outcomes, out of pocket expenses)

Listening to patients results in better decision making & outcomes for all

“Patient Value: Perspectives from the Advocacy Community”
Published in Health Expectations, August 2017
Authors: Bonnie Addario, Ana Fadich, Jesme Fox, Linda Krebs, Deborah Maskens, Kathy Oliver, Erin Schwartz, Gilliosa Spurrier-Bernard, Timothy Turnham
Calls to action from global advocates

- **Framework developers**: Broader representation of patient voices
- **Advocacy community**: Strengthen OUR OWN body of evidence
- **Healthcare industry**: Advance methodologies for determining value
- **Decision makers**: Higher value on the patient perspective

*It’s a big world! How does all this apply to a global organization?*
Beyond value frameworks

Patients value...

....access to treatment!
Max’s approach, a bridge to treatment

Assist when health systems are unable to by:

- Treating those who can be treated today
- Preventing avoidable cancer deaths through
- Humanitarian access to treatment, care and support
The Max Access Solution (MAS) Model
A patient-centered system of humanitarian access to oncology treatment

**NETWORK OF DOCTORS**
An approved network of cancer specialists

**SHIPPING & DISTRIBUTION**
End-to-end validated supply chain

**DONATED MEDICINE**
Procurement of quality targeted treatment

**WRAPAROUND SUPPORT**
A support system around the patient
Actions to increase health equity

- Identify patient leaders & form patient organizations
- Create opportunities for patient leaders to build capacity
- Empower physicians to collect data & publish research
- Gather better data ourselves and publish our own findings
- Speak in global forums to represent ALL patients & make our voices heard!
Together, let's close the cancer divide
Capturing what patients report as important for their cancer care
Jean Mossman
Kuala Lumpur 2 October 2018
EQ-5D-5L

• EQ-5D-5L is a standardised instrument for use as a measure of health outcome
• 5 domains, 5 levels per domain
  • Mobility
  • Self-care
  • Usual activities
  • Pain/discomfort
  • Anxiety/depression
• EQ-5D-5L is used as the basis of cost/QALY calculations
• Sample is the general population
Sample 5 levels: Mobility characteristics

• I have no problems in walking about
• I have slight problems in walking about
• I have moderate problems in walking about
• I have severe problems in walking about
• I am unable to walk about
Advance HTA Work Package 4: Core research objectives

• To determine whether the preferences towards health related quality of life differ between the general population and defined patient groups, including those benefiting from personalised treatments

• Web-based survey of wide set of patients & caregivers across several countries

• Distribution list built through London School of Economics patient training courses and personal connections
  • 360 Patients, 73 caregivers
  • 257 organisations at national level
  • 24 organisations at European level
Research protocol

• EQ-5D-5L

• Supplementary question asked
  • Are there any aspects of your illness which have had a big impact on your health that were not covered in the interview?
    • If yes, please tell us what they were
Results – Response rate

• Patients
  Surveys started: 1031
  **Surveys completed: 767**
  Response rate: 74%

• 48 countries

• 11 countries with >10
  - Belgium (1%)
  - Croatia (2%)
  - Cyprus (4%)
  - Denmark (5%)
  - France (5%)
  - Germany (1%)
  - Greece (7%)
  - Netherlands (4%)
  - Romania (5%)
  - Slovenia (2%)
  - UK (52%)
Methods – Questionnaire design

1. Demographics; age, country of residence, marital status, education and employment status, relationship to the patient

2. Clinical characteristics; disease area, years since diagnosis/provision of care, satisfaction with treatment received

3. EQ-5D-5L Health state utility (EQ-5D-5L), self-perceived health (EQ-5D-5L VAS)

4. Views about important QoL aspects not captured by EQ-5D-5L

5. Additional QoL dimensions; patients’ disability (Barthel Index) and caregivers' emotional burden (Zarit Burden Interview)

Results – Responses

- **Blood cancer**: 125
- **MDS**: 53
- **Blood cancer**: 33
- **Asthma**: 180
- **RA**: 40
- **BC**: 24
- **EDS**: 15
- **Other**: 33
- **UK**: 32
- **Greece**: 31
- **France**: 33
- **Denmark**: 37
- **Romania**: 34
- **Netherlands**: 39
- **Cyprus**: 55
- **Other**: 53

- **Blood cancer**: 180
- **MDS**: 53
- **Blood cancer**: 33
- **Asthma**: 180
- **RA**: 40
- **BC**: 24
- **EDS**: 15
- **Other**: 33
- **UK**: 32
- **Greece**: 31
- **France**: 33
- **Denmark**: 37
- **Romania**: 34
- **Netherlands**: 39
- **Cyprus**: 55
- **Other**: 53
EQ-5D-5L domains

n= 326 (all cancer patients)
Perfect health scores mostly reported by BC (5.5%) and MDS (12.7%) patients
EQ-5D-5L domains

Proportion of individuals reporting extreme and/or severe problems in each EQ-5D-5L domain:
“Are there any aspects of your illness, which have had a big impact on your health, that were not captured by the EQ-5D-5L?”
Conclusion

- EQ-5D-5L does not capture everything that patients say is important
  - Only for blood cancers did more than 50% of patients say it captured everything that matters
- If we base cost/QALY calculations on generic measures, may not reflect patient priorities
- Need to extend the research to get robust evidence of the limitations of EQ-5D-5L
### EQ-5D-5L limitations: domains omitted

<table>
<thead>
<tr>
<th></th>
<th>Fatigue /Insomnia</th>
<th>Medication SEs</th>
<th>Work limitation &amp; financial issues</th>
<th>Maintenance of relationships/social life</th>
<th>Cognitive problems</th>
<th>Co-morbidities</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>BC</strong></td>
<td>20%</td>
<td>22.6%</td>
<td>4.8%</td>
<td>4.8%</td>
<td>9.5%</td>
<td>-</td>
</tr>
<tr>
<td><strong>MDS</strong></td>
<td>39%</td>
<td>13%</td>
<td>-</td>
<td>3.2%</td>
<td>3.25</td>
<td>-</td>
</tr>
<tr>
<td><strong>Blood cancer</strong></td>
<td>20%</td>
<td>10%</td>
<td>-</td>
<td>10%</td>
<td>10%</td>
<td>20%</td>
</tr>
<tr>
<td><strong>KC</strong></td>
<td>50%</td>
<td>12.5%</td>
<td>-</td>
<td>12.5%</td>
<td>-</td>
<td>-</td>
</tr>
</tbody>
</table>
Aspects not captured by EQ-5D-5L: Sample Responses

- “Comorbidities”
- “Cost of medication and other treatment. Difficulty with arranging doctor's appointments and appropriate onward referrals”
- “Financial stress”
- “Concentration”
- “Social isolation” “Stigma-Exclusion from Social Life”
- “Looking after my children”
- “Energy levels, making a contribution to family life, exercise”
- “Effects of long term medication, not of initial illness”
- “Uncertainty about the future. No distinction between discomfort due to disease and that due to treatment”
- “Partnerships and the possibility of raising children”
- “Each day is different so taking it for just one day is not indicative of the overall effects”
- “Lower self-esteem and self-image related with disabilities, social life, personal relationships”
- “Fatigue, and it only measures one day whereas many conditions result in varying abilities across different days”