How HTA Looks—at 10,000 m

- **Quasi-scientific Approach**
  “The process of systematically reviewing existing evidence and producing an evaluation of the effectiveness, cost-effectiveness, and impact, both on patient health and the health care system of health technology and its use.”

- **Evidence-Based Quantitative Assessment**
  - Effectiveness (compared to alternatives)
  - Cost- (comparison, to-benefits, per QALY)
  - Savings (replacement, long-term healthcare, societal benefit)

- **Appraisal Includes “Other” Criteria**
  - Appropriate use (adherence, management)
  - Patient psychological and social Impact (lifestyle, ability to work, tolerance, family, last chance, hope)
### When Does HTA Disadvantage Patients?

- When new (breakthrough) treatments are much more expensive than older treatments
- When new therapy has no comparator (first treatment)
- When therapies (for serious, life-threatening) conditions are approved with less definitive outcomes (surrogate, short-term measures)
- When patient population is small (rare disorders, children, subtypes) so outcomes are less robust
- When outcomes difficult to quantify or measure objectively or relationship between short-term surrogate measures and long-term outcomes unclear.

### Opportunities for Patient Input in HTA

<table>
<thead>
<tr>
<th>Consultee, Informant</th>
<th>Patient Representative</th>
</tr>
</thead>
</table>

<table>
<thead>
<tr>
<th>Individual Patients</th>
<th>Patient Groups</th>
</tr>
</thead>
</table>

**Patient Representativeness**
“Patients are the New Black”

- For Sustainable Drug Access, Patients Must be Engaged
  - To make appropriate choices and adhere to optimal use
  - To input on patient-relevant outcomes and real-world benefits and adverse effects
  - To agree on values and rules for equitable and sustainable resource allocation
- No Voice => No Agreement => No Adherence => No Benefits => No ROI

How to Attain “Best” Individual and Population Health Outcomes

- Attaining best possible individual health and optimal population health in ways that are sustainable
- Individual makes choices that he/she perceives as having good results, are do-able, and affordable
- System provides good options based on projected outcomes and cost so patients make good choices and achieve best possible outcomes
- Innovators earn sufficient incentive to provide technologies with better outcomes and/or cost-effectiveness
- Patient provides feedback, system reassesses evidence and all agree on better options
Patient Values ≠ System Values

<table>
<thead>
<tr>
<th>Patient Values</th>
<th>System Values</th>
</tr>
</thead>
<tbody>
<tr>
<td>“Chance for life”</td>
<td>“Faint hope”</td>
</tr>
<tr>
<td>Quality of Life</td>
<td>Convenience</td>
</tr>
<tr>
<td>Personalized treatment protocol</td>
<td>Standards of care for average patient</td>
</tr>
<tr>
<td>Timely access based on sufficient data</td>
<td>Delayed access based on long-term outcomes</td>
</tr>
<tr>
<td>Facilitative guidelines to allow physician to do optimal prescribing</td>
<td>Restrictive practice guidelines to avoid inappropriate prescribing</td>
</tr>
<tr>
<td>Large benefit to “1”</td>
<td>Small benefits to many</td>
</tr>
</tbody>
</table>

How Patient Views Are Included in Drug Reviews

- Clinical Trials
  - Quality of Life and Patient Reported Outcome Measures

- FDA/EMA Regulatory Review
  - Patients on Advisory Committees
  - Patient Testimonials

- HTA/Drug Review Agencies
  - Patients/Public on HTA Committees (Australia, NICE, Scotland, Canada)
  - Patient Submissions to HTA Committees
  - Citizen Advisory Councils
Patient Submission: Other Jurisdictions

- National Institute for Clinical Excellence (NICE) submission from individual, patient group and physician
- Scottish Medicines Consortium (SMC) submission from registered patient groups
- Australia Pharmaceutical Benefits Advisory Committee (PBAC) through consumer representative
- Others (German, Danish, Swedish, Dutch) have varied patient input processes; Taiwan HTA mandated patient involvement but not defined

Purported and Real Impact of Patient Engagement in HTA

<table>
<thead>
<tr>
<th>Looks Like</th>
<th>Really Happens</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient perceived outcomes built into clinical trials (QOL, PROs)</td>
<td>Scales unable to capture patient experience; no long-term impact</td>
</tr>
<tr>
<td>Patient submissions to HTA give qualitative experience; own words; collective group input</td>
<td>No process to integrate patient submissions into quantitative approach; ignore, discount</td>
</tr>
<tr>
<td>Patient or public members on HTA bodies assure perspective of patient or society at table</td>
<td>Patient/public member roles poorly defined; lack technical expertise; public ≠ patient</td>
</tr>
<tr>
<td>Multi-stakeholder forums, Citizens Councils, consultations are opportunities for input</td>
<td>Most public/patients unaware of processes; technical or academic</td>
</tr>
<tr>
<td>Plain language reports increase patient understanding and acceptance of HTA outcomes</td>
<td>Patients want full access; don’t trust unless present: want right to appeal</td>
</tr>
</tbody>
</table>
What We Say ...

- Drug slows disease progression, relieves symptoms, pain, ability to perform daily activities (including continuing or returning to work), quality of life, improved sleep patterns, and restored libido.
- Fear of fractures have important impact on patients’ quality of life
- Patients described eye symptoms as gritty, sore, burning, painful...with examples of quality of life affected by reduced ability to read, watch television, drive, and outside activities ... adverse effects of ophthalmic corticosteroids

What Is Heard

- The majority of symptoms identified by patient groups are included in SF-36 and HAQ-DI. No data related to restored libido or improved sleep patterns were captured.
- Patient outcome fracture in only one study; pain part of QoL scales
- No DB RCTs w/Moderate DED; post hoc not clinically relevant outcomes

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What We Say ...

- Lupus decreases QoL and ability to work; prednisone has SAE, IV therapy tolerable
- Impact on QoL and family, need for alternative to onerous therapy; reduce hospitalizations
- Chronic warfarin affect OoL: fear of bleeding; INR monitoring; potential drug, food, and alcohol interactions
- CAPS symptoms: rashes, joint pain and stiffness, conjunctivitis; LT kidney failure, deafness, blindness, arthritis, learning disabilities; 1st therapy with CTs; off-label daily injection painful, SAE

What Is Heard

- LT effects ns; variable trial outcomes; no QoL or prednisone reduction (pat imp)
- RCTs only with placebo; patient unmet need for alternatives; 25% failure; too costly
- Noninferior to warfarin; No QoL studies (not designed to measure); cheaper than dabigatran
- CTs: less disease flare but QOL not statistical; lack lifelong benefits data; other than rash no patient benefits submitted

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CDR: % List Recommendations: With/Without Patient Submission

- **All (n=70):**
  - List: 10%
  - List Conditional: 43%
  - Not List: 47%

- **With Patient Submission (n=49):**
  - List: 8%
  - List Conditional: 49%
  - Not List: 43%

- **No Patient Submission (n=21):**
  - List: 14%
  - List Conditional: 29%
  - Not List: 57%

**Reasons Cited in “LIST with Criteria” (2011-13)**

<table>
<thead>
<tr>
<th>Reason</th>
<th>N=30*</th>
<th>N=30*</th>
<th>N=30*</th>
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<tbody>
<tr>
<td>Clinical Trial Outcomes</td>
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<tr>
<td>(effectiveness, sufficient,</td>
<td>Same</td>
<td>Better</td>
<td>Other</td>
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<tr>
<td>similar, certain)</td>
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<td>6%</td>
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<td>7%</td>
<td>0%</td>
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<td>30%</td>
<td>0%</td>
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<td>Patient submissions</td>
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<tr>
<td>(reference, substantiated)</td>
<td>Included</td>
<td>Not addressed</td>
<td>None</td>
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<tr>
<td></td>
<td>60%</td>
<td>20%</td>
<td>20%</td>
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</tbody>
</table>

* % May not add to 100% since not all recommendations cited all reasons
Patient Submissions: Possible Role in “List/Do Not List”

- Patient submission NOT key factor
  - List: 100% cite CT outcomes and cost-effectiveness
  - Do Not List: Based on CT outcomes, cost-effectiveness, QoL (lack data)
  - List w/Criteria: Reference CT outcomes, cost-effectiveness, *patient submission*

- Patient submissions influence when confirmed by data
  - List w/Criteria: 60% confirm patient submission
  - Do Not List: 51% do not study or confirm patient submission

Are Patients/carers/public happier with NICE?

- Lack of research evidence on patient/carer views, experiences and preferences
- Quality of life measures often don’t reflect issues of most importance to patients
- Weighting on evidence from patients
- Process doesn’t take account wider societal costs
- Technical language and economics are difficult to engage with/challenge
- NICE ‘blight’/ variability in access to NICE recommended technologies

*from Victoria Thomas (NICE)*
Patient/Public Member Impact on HTA

Feedback from Patient/Public Members

- Patient and Public Members believe their participation is valuable but neither equal nor impactful.
- Patient submissions give added credibility to patient/public role; have something to contribute.
- Other HTA members not always receptive to patient submissions; qualitative information difficult to integrate; some would prefer quantitative but not sure what would be included.
- Patient submissions have little impact on discussion or decision; however, do increase understanding of rare or unusual conditions.
- Patient relations with pharmaceutical companies, even if declared, raises question of conflict of interest and diminution of input.
- Patient representatives in meetings would increase understanding of the patient submission.

Recommendations for Patient Engagement with HTA

- Provide patients with drug information: CT outcomes, targeted patients, risks and benefits, added value.
- Dialogue with patient groups to define submission: what information is useful, how to collect, how to present.
- Engage patients in CT design to ensure patient values included in measures.
- Train potential HTA patient-public members on technical processes of HTA and decision making.
- Provide means for patient-public members of HTA committees to dialogue with patient representatives.
- Train all HTA committee members on methods for integrating qualitative information.
- Promote transparent decision-making (records of deliberation as well as outcomes); open meetings.
Contact:

Durhane Wong-Rieger
Consumer Advocare Network
www.consumeradvocare.org
416-969-7435
durhane@sympatico.ca