Patients Engagement in HTA

Giora Sharf
Acute Leukemia Advocates Network
27.9.2020
The Book Of Karen Facey – Patients Involvement in HTA—Opens with “If you’re not involving patients, you’re not doing HTA”

• It’s that simple. Patient involvement improves the quality, relevance, and value of HTA. It is difficult to conceive of health technology assessment being conducted in a meaningful way in the twenty-first century without the involvement of patients.

• As the President and CEO of a Health Technology Assessment agency, and as Chair of the International Network of Agencies for Health Technology Assessment (INAHTA), I am a strong advocate for patient involvement in HTA."
Importance and value of patient’s perspectives in HTA

• Decisions should not be made only on the ground of clinical efficacy of a treatment. What about?
• Less side effects and better QOL?
• The ability to continue working and have social activities?
• The influence on family and the ability to plan pregnancy and expansion of the family?

Patients input is critical to better understand the value and influence of a treatment on the patients life.
Value of patients’ perspectives

Living with an illness

➢ ‘No one knows better what it is like to live with an illness day in, day out, than those who are doing this – the patients and their family and friends who care for them.’

Understanding HTA. Health Equality Europe
http://www.htai.org/index.php?id=744

➢ Qualitative context to quantitative data

➢ Challenges to professional assumptions
Where are we in Israel currently?

The Ministry of health initiated last year a possibility for patients to submit personal programmed letters to the HTA committee. This is only a small first step and a long way to go for real patients engagement.

I hear quite often: You, the patients can not participate in HTA since you are not objective and will represent only your own disease interests.

Also: Patients Organizations are not independent as they are supported by Pharma.

We need Drs collaboration to produce RWE which we usually do not recieve.

It is clear we need a major conception change.
Patient & consumer involvement over years

<table>
<thead>
<tr>
<th>Year</th>
<th>Count</th>
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</thead>
<tbody>
<tr>
<td>2008</td>
<td>167</td>
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<td>2009</td>
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<td>2014</td>
<td>633</td>
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<tr>
<td>2015</td>
<td>743</td>
</tr>
<tr>
<td>2016</td>
<td>770</td>
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</tbody>
</table>
Evolution of Patient Engagement at FDA

- FDA Patient Network webpage launched
  - Patient-Focused Drug Development initiative established under PDUFA V
- FDA announces launch of Patient Engagement Advisory Committee
- FDA establishes Health Professional Liaison Program
- FDA patient representatives receive voting rights
- HIV/AIDS patient group founded
- 1991
- 1996
- Patient Representative Program expands, patients now serve as consultants to reviewers during review cycle
- 2001
- 2006
- MedWatch encourages voluntary reporting
- 2008
- FDA working group established to discuss FDASIA section 1137 (evolved to become Patient Council)
- 2012
- FDA holds inaugural Patient Engagement Advisory Committee
- FDA Patient Affairs Staff Established
- FDA launches Patient Engagement Collaborative with CTTI
- 2013
- 2015
- 2016
- 2017
- 2018
- FDA-EMA Patient Engagement Cluster founded
- First FDA Patient Council meeting held
- FDA Patient Affairs Staff establishes MOU with NORD
- Patient Focused Drug Development Initiative
- PDUFA V
- FDASIA - Food and Drug Administration Safety and Innovation Act
- EMA - European Medicines Agency
- CTTI - Clinical Trials Transformation Initiative
- PFDD - Patient-Focused Drug Development
- NORD - National Organization for Rare Diseases

Source: FDA website, client materials
Thank you for your attention
Patient involvement in access decisions?

Zack Pemberton-Whiteley
Patient Advocacy Director - Leukaemia Care
Acute Leukemia Advocates Network – Global Summit - 26.9.20

zackpw@leukaemiacare.org.uk
Why do we need patient involvement?

Image: https://twitter.com/headinthebooth/status/1092096892094296064 (Last Accessed, 30/03/2020)
From the perspective of an HTA agency:

“NICE's approach to patient and public involvement is based on two key principles:

• that lay people, and organisations representing their interests, have opportunities to contribute to developing NICE guidance, advice and quality standards, and support their implementation, and

• that, because of this contribution, our guidance and other products have a greater focus and relevance for the people most directly affected by our recommendations.”

Decisions for the ‘patient’ or ‘public’?

“Facilitate development and access to medicines: EMA is committed to enabling timely patient access to new medicines, and plays a vital role in supporting medicine development for the benefit of patients”

“NICE's role is to improve outcomes for people using the NHS and other public health and social care services.”
https://www.nice.org.uk/about/what-we-do
### Involvement opportunities in the different UK processes

<table>
<thead>
<tr>
<th></th>
<th>NICE</th>
<th>SMC</th>
<th>AWMSG</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Scoping</strong></td>
<td>✓</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td><strong>Technical Engagement</strong></td>
<td>✓</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td><strong>Evidence Submission</strong></td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td><strong>Patient Focused Meeting</strong></td>
<td>?</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td><strong>Committee Meetings</strong></td>
<td>✓</td>
<td>✓</td>
<td>X</td>
</tr>
<tr>
<td><strong>Opportunity to Appeal</strong></td>
<td>✓ (ACD and FAD)</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td><strong>Publication</strong></td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
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**NICE** – National Institute for Health and Care Excellence  
**SMC** – Scottish Medicines Consortium  
**AWMSG** – All Wales Medicines Strategy Group

**PACE** (For rare and end of life medicines)  
**CAPIG** (For rare diseases only)
Does patient involvement have an impact?

- Patient Organisations and Patient Experts “do not feel that their efforts to contribute to the process are seen as being credible by NICE committees”
  
  (NICE Patient Group Workshop, Jan 19)

- Patient testimony is usually qualitative (e.g. patient testimony), so the impact on decision making is not usually obvious
  
  (NICE and Myeloma UK, Measuring Patient Preferences, June 19)

- Where is the opportunity to impact in a QALY based system?
  
  - Survival
  - Quality of Life

Changing the mindset: ‘Simple COVID health economics’?

Dr Amanda Adler, Chair of NICE technology appraisal committee B:

“Gel on the left ‘kills 99.99% of germs’, so is more effective than the right one which kills only 99.9%.
One might expect to pay more; but, the left gel is 79% the price of the other even correcting for volume.
Cheaper, more effective = dominant”

Image: https://twitter.com/DrAmandaAdler/status/1306293645297946625 (Last Accessed, 22/09/2020)
Responsibility on patient organisations

- Developing **capability** and **capacity** to engage effectively in access decisions
- Evidence-based advocacy of patient views and experience
  - Trainings on evidence-based advocacy
  - Generate evidence – in a format that stakeholders can use for decision making

**Evidence-Based Advocacy**
Advocating in a targeted, evidence-based, well-educated and professional manner, and measure impact and outcomes of what we do.

Image from WECAN course on evidence-based advocacy - https://wecanadvocate.eu/eba/
Responsibility on industry

- This goes **beyond HTA**
- Lots of opportunities for patient involvement in medicines **R&D**
- Lack of patient involvement in R&D **influences HTA decisions**
- We already have a roadmap...

Geissler et al (2017) - Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap - https://journals.sagepub.com/eprint/6J5ErcVgCvIBDoC7FD/full
Adele K. Fielding

Professor of Haematology
Group Leader UCL Cancer Institute

Former Chair, UK NCRI Adult ALL subgroup
Chair European Working Group on ALL
Patients’ involvement in access decisions

Pauline McGuire, Principal Pharmaceutical Analyst,
Scottish Medicines Consortium
Health technology appraisal – the Scottish approach

www.scottishmedicines.org.uk
Public Involvement Network (PIN) Advisory Group

NHS Scotland

ALLIANCE Health and Social Care

Scottish Cancer Coalition

RARE DISEASE UK

Healthcare Improvement Scotland | SMC Advice on new medicines
Patient Group Involvement

For all medicines
Patient group partners provide written evidence—supported by public involvement specialists.

PACE meeting
End of Life/orphan medicine
Patient representatives attend a meeting with clinicians to discuss condition medicine.

SMC papers
Include patient group submissions and PACE statements as evidence for decision makers.

SMC meeting
Patient groups submissions and PACE overview are presented. Patient group representatives attend to answer questions.
Patient and Clinician Engagement (PACE) Meetings
"The value of these additional months and years gained is enormous to a patient, often allowing them to return to work, contribute to society, care for their families and watch their children grow up."

"Many carers will have to give up work. Any possible reduction in the rate of progression is likely to have a positive impact on carers/family."

"When making choices about treatment women are primarily driven by a need to survive or improve PFS. Most feel that the side-effects of treatment are a small price to pay in order to achieve this."
What matters to patients/families/carers?
(Thematic analysis of PACE year 1)
SMC Decision Making
Thank you for listening

www.scottishmedicines.org.uk
Patients’ involvement in access decisions

Presented by Francesco Pignatti on 26 September 2020 (The views presented are personal)
### DECISIONS: FROM MARKETING AUTHORISATION TO ACCESS

<table>
<thead>
<tr>
<th>Regulatory Agency</th>
<th>Healthcare System/Payors</th>
<th>Patients and Physicians</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Marketing Authorisation</strong></td>
<td><strong>Reimbursement</strong></td>
<td><strong>Clinical decision</strong></td>
</tr>
<tr>
<td>Technical requirements: Efficacy/Safety; Balance of Benefits and Harms</td>
<td>Societal goals: Cost-effectiveness, health care policies (national level)</td>
<td>Personal goals: Balance of benefits and risks for individual</td>
</tr>
</tbody>
</table>

- **Approved**
- **Reimbursed**
- **Prescribed**
- **Used**
- Clinical trials
- Off-label use
- Compassionate use
Whose values matter in benefit-harms trade-offs?

- On what basis should regulators base their decisions to approve or reject medicines?
  - More or less harm-tolerant patients?
  - Should the basis be individual or collective perspectives?
  - Or perhaps the regulators’ own perspectives?
- Is there a justification for paternalistic medicines regulation to protect patients but limit their autonomy?
Conclusion

- **Patients know best** about benefits and harms trade-offs
  - The views that matter: Patients acting voluntarily in an adequate decision context judging if the balance of benefits and harms (as described by regulators) is positive
  - Inform other actors about trade-offs between benefits and harms of drugs
  - Ideally supported by robust evidence (like stated preference studies)

- **Regulators**
  - Ascertain that the effects are real and well described for other actors to decide
  - Assess if there are patients, in the right decision context, who would consider that benefits outweigh the harms – communicate to inform subsequent decisions
THANK YOU!

ACUTE LEUKEMIA GLOBAL VIRTUAL SUMMIT