

# The role of Patient Generated Evidence in HTA

**Zack Pemberton-Whiteley**

**CML Horizons - Warsaw – 6<sup>th</sup> May 2018**

Campaigns and Advocacy Director, Leukaemia Care (UK)

CML Advocates Network – Steering Committee – Western Europe

Chair, Acute Leukemia Advocates Network (Global)

[zackpw@leukaemiacare.org.uk](mailto:zackpw@leukaemiacare.org.uk) or 01905 755977

## Why are we doing it?

- Representing patient perspective
  - Experience of broader population
  - Individual/Personal experiences
- Looking to shift perceptions and change behaviour/decisions
- Make a difference for patients
  - e.g. access to: treatment, diagnostics, testing, information, support

## What is the value of patient involvement?

New evidence and information

New Evidence:

- What matters to patients?

Challenges to evidence or conventional wisdom e.g. outcomes

Challenging measurements:

- Providing detail on patient views – what outcomes are important?

Qualitative context to quantitative data

Qualitative vs Quantitative:

- Evidence is king!
- Individual evidence helps to explain the impact of decisions

## What is the value of patient involvement?

Challenges to professional assumptions

Challenging views:

- E.g. different perception of risk?

Being Involved?

- I would rather not be involved in a positive decision, than be involved in a negative one

Triangle of evidence:

- Important to provide different perspectives

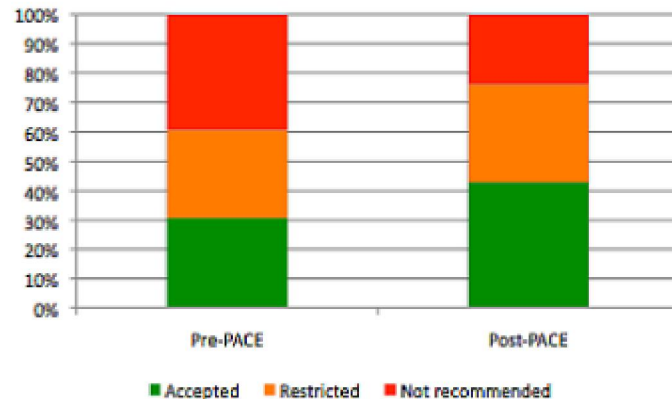
Value to the patients in being involved

Completing the triangle (of evidence)

## Does patient input have an impact?

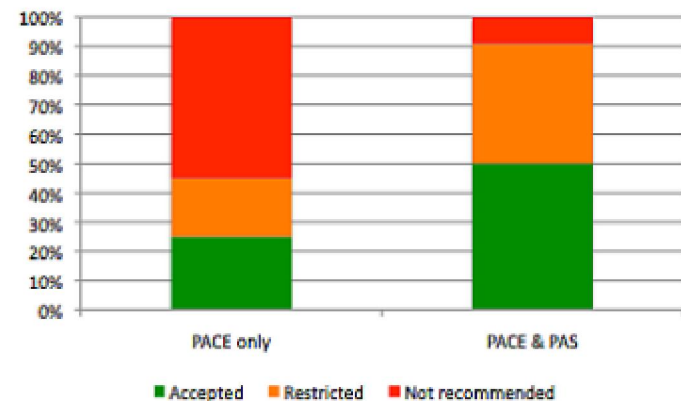
- Does it actually influence the number of drugs being approved?
- Does it influence WHICH drugs are approved?

Figure 10: Change in SMC recommendations, pre and post PACE



Source: Analysis of SMC data

Figure 14: SMC recommendations including PACE deliberations, with and without PAS



Source: Analysis of SMC data

Example:

Health Technology Appraisals in the UK

## 3 Different Processes in UK

- NICE – National Institute for Health and Care Excellence
- SMC – Scottish Medicines Consortium (PACE)
- AWMSG – All Wales Medicines Strategy Group

## Comparison of Different Processes

	NICE	SMC	AWMSG
Scoping	✓	X	X
Technical Engagement	✓ (NEW)	X	X
Evidence Submission	✓	✓	✓
Patient Focused Meeting	X	✓ PACE (For rare and end of life medicines)	✓ CAPIG (For rare diseases only)
Committee Meetings	✓	✓	X (Public Gallery)
Opportunity to Appeal	✓ (ACD and FAD)	X	X
Publication	✓	✓	✓

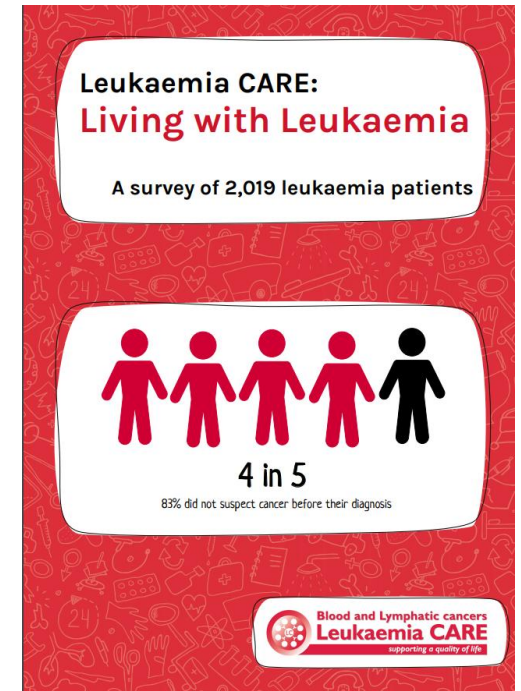


## Generating and Submitting Patient Views

- Evidence based advocacy (not opinions)
- Demonstrate what matters to patients  
(gather through surveys and/or focus groups)  
“the plural of anecdotal is evidence”
- Importance of early and continued involvement (e.g. at scoping stage)

## Where does the evidence come from?

- Living with Leukaemia Survey
  - 2019 leukaemia patients
  - CML Breakdown (292)
  - Launched in Houses of Parliament



<http://www.leukaemiacare.org.uk/living-with-leukaemia>

## Other Evidence?

It may already be available:

- Social Media/Forums
- Published literature

That you can go out and gather:

- One-to-one discussions (e.g. telephone helplines)
- Focus Groups
- Surveys
- Use your networks – other charities? Medical Advisors?

## What information are they looking for?

- Patient and carer views:
  - “Impact of decisions”
  - Living with the condition – what is it like for patients and carers?
  - New medicine: advantages/disadvantages? (A balanced overview)
  - What treatments are currently available? – Is there an unmet need?
  - Quality of life
  - Specifics (e.g. what is different about a specific sub-group of patients)

## What information are they NOT looking for?

- Clinical or scientific evidence (e.g. trial data) as this will be covered by the company and clinical experts
- Summarised information from other sources
- Exceptional/unusual cases (broader experience preferred)

## Evidence Submission - Tips

1. Clear – facts and information
2. Be concise – accurate summaries
3. Balanced – include advantages AND disadvantages
4. Patient “quotes” are powerful/emotive
5. BUT ... evidence of collective patient views is stronger!  
(Surveys are key!)
6. Be realistic – you may not be able to cover everything

## Example: Inotuzumab Ozogamicin (ALL)

- Scoping, Evidence Submission, ACD – using evidence
- FAD – Appeal
  - LC Appeal – number of cycles of treatment used
  - 4 Representatives – ZPW, 2 Patients, 1 Family Member
  - “The appeal panel also upheld the appeal on the grounds that the appraisal committee’s recommendation is unreasonable in the light of the evidence submitted to NICE with regards to ground 2.1 (appellants Leukaemia CARE and the joint appellant).”
- Outcome - TBD

## Where are we at now?

- Current opportunities for involvement
- Areas where involvement needs to be improved
- Does involvement = impact?



# Where are we going?

- Rhetoric:
  - NICE: Patient Involvement Consultation (December 2016)
- Reality?
  - NICE: Increasing technology appraisal capacity consultation (October - November 2017)

## How do we turn rhetoric into reality?

- Evidence based advocacy?
- Working with all stakeholders
- Changing focus – involvement v impact?