INVESTIGATING THE IMPORTANCE OF EQ-5D DOMAINS AND TWO BOLT-ON ITEMS: HOW DO THE PREFERENCES OF PEOPLE WITH ACUTE LEUKAEMIA DIFFER FROM THE GENERAL POPULATION?

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INTRODUCTION

- Acute leukaemia (AL) and its treatment substantially affect **health-related quality of life** (HRQoL).
- In health technology assessment (HTA), it is often recommended to capture HRQoL using generic measures such as **EQ-5D**[1].
- When taking a **patient-centric** view, there are two potential limitations of the typical approach to capturing benefits in HTA:
  1. Some aspects of health that matter to patients **might not be explicitly captured** by EQ-5D.
  2. EQ-5D value sets are usually based on **general population preferences** rather than the preferences of patients.

OBJECTIVES

- The first objective of this study was to **explore the preferences of people with AL** for the 'core' five domains of EQ-5D, alongside two additional 'bolt-on' items of relevance in AL:
  - Mobility
  - Self-care
  - Usual activities
  - Pain/discomfort
  - Anxiety/depression
  - Tiredness [Bolt-on]
  - Cognition [Bolt-on]
- An additional objective was to **compare** patients' preferences with those of the general population.

METHODS

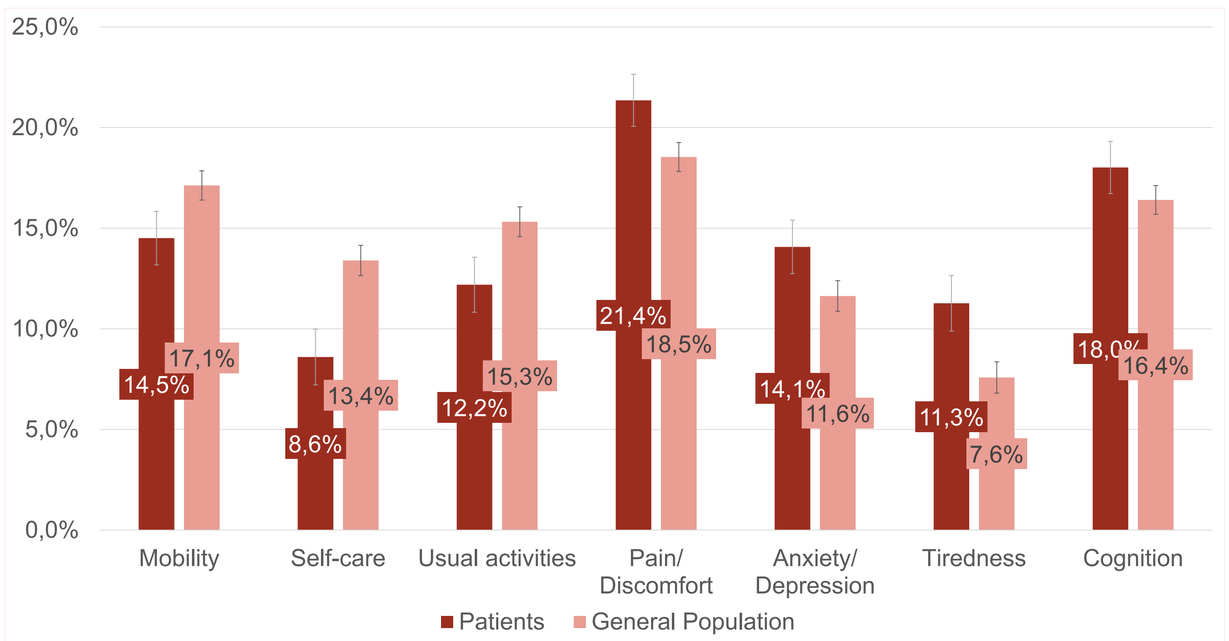
- An online survey was designed that contained a profile-case (case II) **best-worst scaling exercise** (BWS).
- In the BWS exercise, respondents were shown **a series of health states** describing problems on the five EQ-5D dimensions and the two bolt-on items.
- For each profile, respondents had to **select the 'best' (or 'least bad') item and the 'worst' item** from the health state.
- To simplify the design, **three severity levels** were included for each dimension (no problems; moderate problems; and extreme problems/unable to).
- The survey was launched in **five countries** (UK, USA, France, Germany, and Italy) with adults with AL as well as adult members of the general population.
- The BWS data were pooled across countries and analysed using a **modelling approach** (marginal-sequential conditional logit models).
- **Relative importance (RI) scores** were calculated to enable comparisons of the importance of the seven dimensions across the two samples.

RESULTS

TABLE 1. RESPONDENT CHARACTERISTICS

N (%)	PATIENTS (N=212)	GEN. POP. (N=511)
Age (Mean (SD))	53.7 (13.7)	48.2 (16.2)
Gender		
Male	77 (36%)	241 (47%)
Female	135 (64%)	270 (53%)
Education		
Completed high school	192 (91%)	433 (85%)
Has degree or equivalent	106 (50%)	336 (66%)
Responsible for children		
Yes	67 (32%)	190 (37%)
No	144 (68%)	315 (62%)
Not reported	1 (<1%)	6 (1%)
Leukaemia type		
Acute lymphocytic leukaemia (ALL)	62 (29%)	
Acute myeloid leukaemia (AML)	133 (63%)	
Acute promyelocytic leukaemia (APL)	17 (8%)	
Years since diagnosis (Mean (SD))	3.4 (3.5)	
Treatment status		
Not on treatment currently	89 (42%)	
On treatment	76 (36%)	
Awaiting or had recent transplant	32 (15%)	
Not sure/other	15 (7%)	
Relapse history		
Not achieved remission	28 (13%)	
Never relapsed	98 (46%)	
One or more relapses	86 (41%)	

FIGURE 1. RELATIVE IMPORTANCE SCORES, BY SAMPLE



- A total of 212 patients and 511 members of the general population (GP) completed the survey across the five study countries (Table 1). The patient sample was older on average, and a greater proportion were female, compared to the GP sample.
- All dimensions were of importance (Fig 1), and pain/discomfort was the most important dimension in both samples – however, it was less important for the GP sample.
- For patients, pain/discomfort was closely followed by cognition in 2nd, and mobility was ranked 3rd. These rank orderings were the other way around for the GP sample.
- The rank orderings of the final four dimensions varied more considerably, with patients ranking anxiety/depression as 4th (6th for GP), tiredness as 6th (7th for GP) and self-care as the least important (5th for GP).

CONCLUSIONS

- Patients and the general population both ranked pain/discomfort and mobility among their top concerns. However, these results suggest that cognition – a dimension not explicitly captured within the EQ-5D – was also among the top concerns of both groups.
- If EQ-5D does not adequately capture improvements in cognition over time (its responsiveness in dementia is inconclusive[2] for example), then then the use of a cognition bolt-on item in clinical trials may be warranted – and, based on these results, improvements in cognition may be valued highly.
- These results also suggest that the preferences of patients and the general population do not perfectly align. For example, patients were more concerned about tiredness compared to the general population, which may reflect their lived experience of AL.
- There has been a longstanding debate in health economics[3] around whether value sets should be based on patient or general population preferences – these results add further evidence to show that, as preferences differ between these groups, this normative choice matters.

REFERENCES

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FULL REPORT

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