UTILITY VALUES FOR CHRONIC MYELOGENOUS LEUKAEMIA CHRONIC PHASE HEALTH STATES FROM THE GENERAL PUBLIC IN THE UNITED KINGDOM

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INTRODUCTION

- Chronic myeloid leukemia (CML) is characterised by the malignant expansion of stem cells in the bone marrow. It is diagnosed using cytogenetic and molecular diagnostic techniques to detect a chromosomal abnormality known as the Philadelphia (Ph) chromosome and is the only known cause of CML.

- CML is a relatively rare disease having an annual incidence of approximately 1–2 per 100,000 people [1] and accounts for 15–20% of adult leukaemias [2-4]. The median age at presentation is 50–60 years and 12–30% of patients are >60 years old at diagnosis [5].

- The natural course of the disease involves three sequential phases (chronic, accelerated, and blast crisis), each becoming progressively more difficult to treat [6, 7].

- There are three levels of response following treatment of CML chronic phase: haematologic, cytogenetic and molecular response [7,8].

  ➤ Patients who achieve only a haematologic response are at risk of progressing to severe disease. Approximately 30% will survive for at least 5 years, unless they are switched to another more effective treatment [9].

  ➤ Patients who achieve a cytogenetic response are at less risk of progressing to severe disease and approximately 80% of patients will survive for at least 5 years [9].

  ➤ Patients who achieve a molecular response are unlikely to progress to more severe phases. Remission is expected to last for many years and approximately 90% of patients will survive for at least 5 years [10].

- To date, preferences for these CML chronic phase-related health states have not been measured.

- The objective of this study was to estimate time trade-off (TTO) preference values associated with CML chronic phase-related health states among members of the general public in the UK.

METHODS

Health States

- Descriptions of the four chronic phase-related health states under evaluation (i.e. chronic phase CML (i.e. pre-treatment and treatment failure), haematologic response, cytogenetic response and molecular response) were developed through literature review and clinical expert consultation.

- Each health state described the typical patient experience across several domains including symptoms, treatment, response, management and prognosis.

- Health states were refined after iterative review by clinical experts and piloting the descriptors among a sample of 18 respondents in the UK.

- The health states were designed to be easily understood by the general public.

Study Respondents

- The study was undertaken among a sample of randomly selected members of the general public across the UK.

- Respondents had to be at least 18 years of age.

- Potential respondents were excluded if they were not English-speaking, if they had apparent cognitive impairment, or if in the interviewer's opinion they were incapable of understanding the task.

- Recruitment occurred between February and May 2010. None of the respondents received remuneration for participation.

Data Collection And Analysis

- Data were collected through individual, face-to-face interviews. Participants were provided with descriptions of the four different health states associated with CML chronic phase.

- Participants were asked a range of socio-demographic questions about themselves. They were also asked what proportion of their remaining lifetime they would be willing to sacrifice in return for not living with the symptoms of CML associated with each of the four health states being evaluated.

- Utility scores (ranging from 1.0 for perfect health to 0 for death) were obtained for the different health states as described by Hammerschmidt et al [11].

- Differences between groups were tested for statistical significance using a Mann Whitney U-test. Multiple regression was also performed to assess the relationship between baseline parameters and outcomes.

RESULTS

- The study sample comprised 241 participants interviewed at one of eight locations in the UK. The participants' socio-demographic details are summarised in Table 1.

Characteristic	Mean number or percent (with 95% confidence interval)
Respondent's age	45.3 (43.1; 47.6) years
Percent female	51%
Marital Status	
Percent married/cohabiting	55%
Percent single	33%
Percent divorced/separated	6%
Percent widowed	5%
Employment Status	
Percent employed	54%
Percent retired	27%
Percent students	7%
Percent unemployed	7%
Percent at home	5%
Mean annual salary	£21,800 (£20,500; £23,100)
Percent with cancer at the time of the interview	7% had cancer for a mean 7.0 (6.2; 7.8) years
Percent of respondents who knew individuals with cancer	84%

Table 1: Respondents' sociodemographic details.

- All four chronic phase CML-related health states were associated with decreases in preference values from full health (Figure 1).

  ➤ The molecular response to treatment was the most preferred health state, with a mean utility of 0.94 in the overall sample.

  ➤ The second-most preferred health state was cytogenetic response followed by haematologic response (mean utilities in the overall sample were 0.89 and 0.80 respectively).

  ➤ The least preferred health state was chronic phase CML, with a mean utility of 0.72 in the overall sample.

  ➤ The utility values for each state were significantly different from one another: p <0.001.
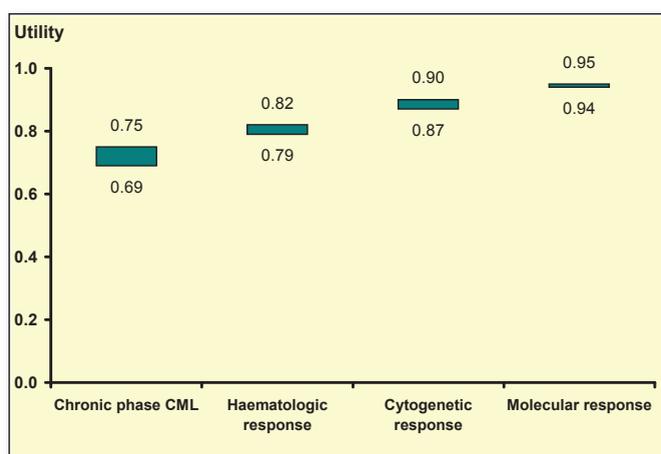

Figure 1: Mean utilities (mean and 95% confidence interval) for CML chronic phase-related health states.

- The mean utility values elicited from respondents with cancer were lower than the values elicited from those who did not have cancer (Figure 2). However, these differences were not statistically significant, possibly because of the small number of respondents with cancer (n=18).

  ➤ 0.65 versus 0.73 for chronic phase CML.

  ➤ 0.72 versus 0.81 for haematologic response.

  ➤ 0.83 versus 0.89 for cytogenetic response.

  ➤ 0.89 versus 0.95 for molecular response.
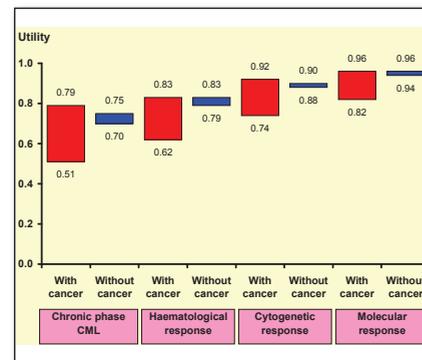
RESULTS (CONT'D)

Figure 2: Mean utilities (mean and 95% confidence interval) for CML chronic phase-related health states, stratified by respondents' cancer status.
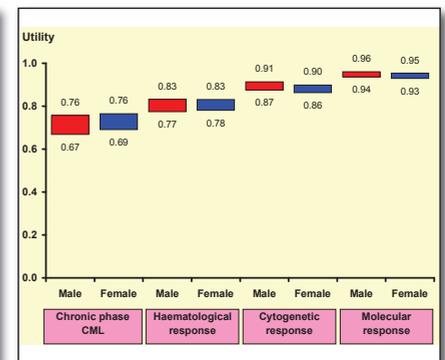

Figure 3: Mean utilities (mean and 95% confidence interval) for CML chronic phase-related health states, stratified by respondents' gender.

- The utility values elicited from respondents were not affected by their gender (Figure 3):

  ➤ 0.71 (males) versus 0.73 (females) for chronic phase CML.

  ➤ 0.80 (males) versus 0.81 (females) for haematologic response.

  ➤ 0.89 (males) versus 0.88 (females) for cytogenetic response.

  ➤ 0.95 (males) versus 0.94 (females) for molecular response.

- The utility values elicited from respondents decreased with their age (Table 2).

- The utility values elicited from respondents who were widowed or divorced/separated were lower than from respondents who were married/cohabiting or single (Table 2).

- The utility values elicited from retired respondents were lower than from the other respondents. Additionally, the utility values elicited from employed and unemployed respondents were lower than from respondents who were either at home or students (Table 2).

- The respondents' health state preference values were not affected by their annual salary (Table 2).

- None of these differences reached statistical significance, possibly because the sample size of each sub-group was too small.

	Mean utilities (mean and 95% confidence intervals) for			
	Chronic phase CML	Haematologic response	Cytogenetic response	Molecular response
Respondents' age				
<30 years (n=65)	0.76 (0.72; 0.81)	0.83 (0.80; 0.86)	0.91 (0.89; 0.92)	0.96 (0.95; 0.97)
30-39 years (n=39)	0.75 (0.69; 0.82)	0.85 (0.82; 0.88)	0.91 (0.89; 0.93)	0.96 (0.94; 0.97)
40-49 years (n=32)	0.74 (0.67; 0.81)	0.81 (0.76; 0.86)	0.90 (0.87; 0.92)	0.95 (0.94; 0.97)
50-59 years (n=44)	0.72 (0.65; 0.79)	0.82 (0.78; 0.86)	0.89 (0.86; 0.92)	0.94 (0.92; 0.96)
>60 years (n=61)	0.64 (0.58; 0.71)	0.73 (0.68; 0.79)	0.85 (0.81; 0.88)	0.92 (0.90; 0.95)
Respondents' marital status				
Widowed (n=12)	0.60 (0.43; 0.78)	0.72 (0.59; 0.85)	0.82 (0.74; 0.90)	0.94 (0.91; 0.98)
Divorced/separated (n=15)	0.62 (0.47; 0.76)	0.73 (0.63; 0.83)	0.84 (0.78; 0.90)	0.94 (0.93; 0.95)
Married/cohabiting (n=134)	0.72 (0.68; 0.76)	0.80 (0.78; 0.83)	0.89 (0.87; 0.90)	0.93 (0.93; 0.98)
Single (n=80)	0.76 (0.72; 0.80)	0.83 (0.81; 0.86)	0.91 (0.89; 0.93)	0.95 (0.94; 0.96)
Respondents' employment status				
Retired (n=65)	0.65 (0.58; 0.71)	0.75 (0.70; 0.79)	0.86 (0.83; 0.89)	0.93 (0.91; 0.95)
Employed (n=129)	0.73 (0.70; 0.77)	0.82 (0.79; 0.84)	0.89 (0.87; 0.91)	0.97 (0.95; 0.98)
Unemployed (n=18)	0.73 (0.65; 0.82)	0.81 (0.75; 0.87)	0.89 (0.85; 0.92)	0.95 (0.93; 0.97)
At Home (n=12)	0.79 (0.68; 0.90)	0.87 (0.81; 0.93)	0.92 (0.88; 0.97)	0.95 (0.93; 0.96)
Student (n=17)	0.83 (0.75; 0.90)	0.88 (0.85; 0.92)	0.94 (0.92; 0.96)	0.97 (0.95; 0.98)
Respondents' salary*				
Pension (n=56)	0.63 (0.56; 0.69)	0.73 (0.69; 0.78)	0.86 (0.83; 0.89)	0.92 (0.90; 0.95)
<£10,000 (n=20)	0.76 (0.68; 0.85)	0.83 (0.76; 0.89)	0.88 (0.82; 0.95)	0.93 (0.91; 0.95)
£10,000-£20,000 (n=41)	0.72 (0.65; 0.79)	0.80 (0.76; 0.85)	0.88 (0.84; 0.91)	0.94 (0.90; 0.98)
£21,000-£30,000 (n=40)	0.74 (0.69; 0.80)	0.81 (0.78; 0.85)	0.90 (0.88; 0.92)	0.95 (0.94; 0.98)
>£30,000 (n=33)	0.75 (0.66; 0.84)	0.84 (0.78; 0.89)	0.90 (0.87; 0.94)	0.96 (0.94; 0.98)

Table 2: Mean utilities (mean and 95% confidence interval) for CML chronic phase-related health states, stratified by sociodemographic parameters. (*4 respondents refused to provide their salary).

- Multiple regression demonstrated that respondents' preference values for any of the states were not affected by:

  ➤ Age.
  ➤ Gender.
  ➤ Location.
  ➤ Marital status.
  ➤ Employment status.
  ➤ Annual salary.
  ➤ Whether they had cancer.

DISCUSSION

- This is the first study to provide a direct measure of preferences for CML chronic phase-related health states (including treatment response categories) from the general public's perspective.

- Participant recruitment was undertaken in eight locations across the UK. However, these respondents may not be representative of the UK target population as a whole.

- The respondents' preference values for any of the states were not significantly affected by their age, gender, location, marital status, employment status, annual salary or whether they had cancer.

- TTO values are affected by respondents' time preferences and this may explain why utility values elicited from respondents tended to decrease with their increasing age. Nevertheless, these differences were not statistically significant.

- The utility values elicited from respondents with cancer were lower than the values elicited from those who did not have cancer. This difference did not reach statistical significance, possibly because of the small number of respondents with cancer.

- The TTO scores are similar to those obtained by Szabo et al [12] in the UK for the health states "chronic phase CML not responding to treatment (0.73)" and "chronic phase CML responding to treatment (0.91)". This TTO study added further specificity to the health state values for different levels of response to CML treatment.

- The preference values obtained from this study will enable a direct comparison between CML chronic phase-related health states, and calculation of quality-adjusted life years in economic evaluations of treatments for CML.

CONCLUSIONS

This study indicated greater preference values for deeper levels of treatment response and demonstrate the impact that deeper and more robust treatment responses have on the health-related quality of life of patients with chronic phase CML. These health state preference values can be used to estimate the outcomes of interventions in terms of quality-adjusted life years.

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