

PCR76

Generating health-state utility values in patients treated with second-line systemic therapy for relapsed or refractory large B-cell lymphoma

Ioana-Alexandra Soare, MSc,<sup>1</sup> Matthew Little, PhD,<sup>1</sup> Sven L. Klijn, MSc,<sup>2</sup> Ahmed Elsada, MSc,<sup>2</sup> Adam Gibson, PhD,<sup>1</sup> Fei Fei Liu, MBA,<sup>2</sup> Frauke Becker, PhD<sup>1</sup>

<sup>1</sup>Putnam PHMR, Newcastle Upon Tyne, UK; <sup>2</sup>Bristol Myers Squibb, Princeton, NJ, USA

Introduction

- Patients with relapsed or refractory (R/R) large B-cell lymphoma (LBCL) who had failed their first line of treatment have a poor prognosis and limited treatment options<sup>1</sup>
- In addition, trial-generated data for health-related quality of life (HRQOL) in R/R LBCL are scarce, especially for later stages of the disease
- The aim of this study was to generate utility values associated with the HRQOL of patients treated with second-line (2L) systemic therapy for R/R LBCL to inform cost-effectiveness models for health-technology assessments (HTA)

Methods

Figure 1. Overview of study design



TTO, time trade-off.

- Our study was composed of 4 different stages (Figure 1)
- Based on evidence from the literature and qualitative phone interviews with patients (n = 6), bespoke health state vignettes were developed representing different R/R LBCL disease stages. The vignettes were then amended and validated via qualitative semi-structured remote interviews using the feedback of 2 clinical experts
- All vignettes were valued by members of the United Kingdom (UK) general population in an online interviewer-assisted survey using the TTO methodology.<sup>2</sup> The TTO methodology involves a series of structured stated preference tasks that ask respondents if they would prefer living a longer life in a specific health state vignette or a shorter life in full health. Based on the responses, the amount of time in full health is varied until respondents become indifferent between their 2 choices, at which point a utility value for the health state valued is determined
- Our study aimed to follow the established international EQ-5D-5L valuation protocol as closely as possible<sup>3</sup>
- Qualitative data were analyzed using MAXQDA 2020 (20.2.1) and quantitative data were analyzed using Stata 15

Results

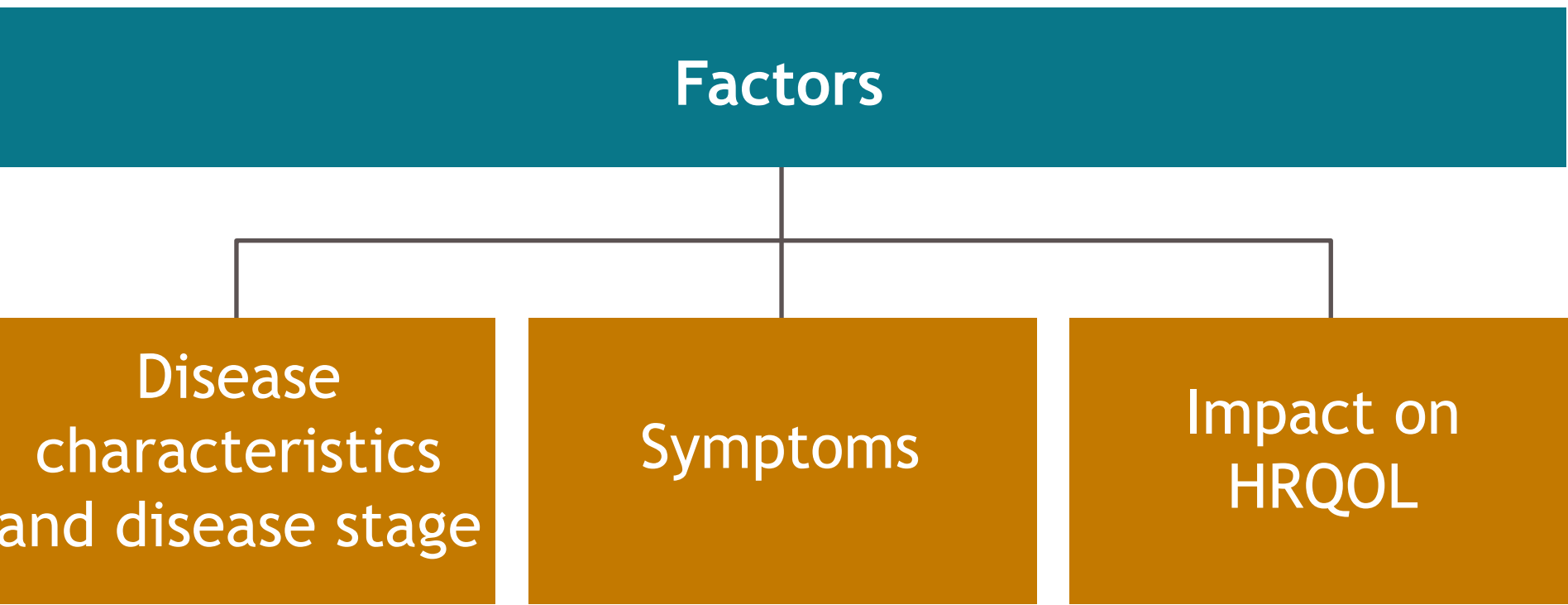
Table 1. Health state vignettes

Chronic health scenario	Description
EFS	Representative of the average patient treated with 2L systemic therapy for R/R LBCL (ie, up to 2 years after treatment)
Prolonged EFS	Representative of the average patient treated with 2L systemic therapy for R/R LBCL whose treatment worked and who remained in long-term remission without progressing (ie, 2+ years after treatment)
Progression	Representative of the average patient treated with 2L systemic therapy for R/R LBCL for whom treatment did not work or whose disease had progressed

EFS, event-free survival.

- The final 3 vignettes described different chronic health scenarios of patients treated with 2L systemic therapy for R/R LBCL (Table 1)

Figure 2. Overview of health state vignette design



- All 3 vignettes (Figure 2) included a brief description of the following:
  - Lymphoma and the disease stage (eg, progression)
  - Symptoms experienced (eg, fatigue), as well as their frequency and severity specific for each disease stage
  - Impact associated with patients’ daily life (eg, social life)

Table 2. Respondent characteristics

Characteristics	Respondents (N = 201)	UK population <sup>a</sup>
Median age, y	44	40
Gender, n (%)		
Female	108 (54)	(51)
Ethnicity, n (%)		
White	197 (98)	(82)
Highest formal education, n (%)		
College or university	112 (56)	(51)
Higher or secondary education	62 (31)	(17)
Secondary school	27 (13)	(23)
Economic activity, n (%)		
Working full time	107 (53)	(40)
Working part time	40 (20)	(17)
Retired	31 (15)	(22)
Student	13 (6)	(6)
Other	17 (8)	N/A
General health, n (%)		
Very good	86 (43)	(48)
Good	87 (43)	(34)
Fair	26 (13)	(13)
Bad	2 (1)	(4)

<sup>a</sup>UK Census 2021 that is representative of the respondents’ population and is not exhaustive for each category. N/A, not available.

- Online survey participants’ demographics
  - A total of 201 respondents who were representative in terms of age and gender for the UK population completed the online interviewer-assisted TTO survey (Table 2)
  - No respondents were excluded from the analysis due to “speeding,” as all respondents spent more than one-third of the median time (3.7 minutes) completing the survey

Table 3. Summary of health state utility values

Health state	Mean (SD)	Median (IQR)
EFS	0.53 (0.39)	0.6 (0.4–0.8)
Prolonged EFS	0.65 (0.32)	0.7 (0.5–0.9)
Progression	0.29 (0.46)	0.4 (0.0–0.6)

IQR, interquartile range; SD, standard deviation.

- The health state vignette with the highest mean utility value (0.65) was prolonged EFS, followed by EFS that was associated with a mean utility value of 0.53 (Table 3)
- As expected, the health state vignette with the lowest mean utility value (0.29) was associated with the disease progression state

Table 4. Difference in mean utility values between health states

Health state	Difference (95% CI; P value <sup>a</sup> )	% change
EFS	Not applicable	
Prolonged EFS	0.12 (0.08, 0.16; < 0.001)	23%
Progression	–0.24 (–0.29, –0.18; < 0.001)	–45%

<sup>a</sup>Compared with the EFS health state. CI, confidence interval.

- Compared with the EFS health state, prolonged EFS had a statistically significant higher mean utility (P < 0.001) (Table 4)
- Progression was associated with a statistically significant lower mean utility compared with the EFS health state (P < 0.001)
- Reported utilities might be lower than those obtained during clinical trials. This observation is commonly found in the literature due to the use of different methodologies (ie, TTO vs generic preference-based instruments)<sup>5</sup> and study populations (ie, general population vs patients)<sup>6</sup>

Conclusions

- The utility values obtained in this study followed an expected logical pattern that suggests the HRQOL of patients treated with 2L systemic therapy for R/R LBCL is poor, especially in the later stages of the disease (ie, progression)
- The utility data obtained directly from patients by administering a generic preference-based HRQOL instrument (ie, EQ-5D) is preferred for HTA in the UK. However, when such instruments cannot adequately capture patients’ HRQOL, the use of a vignette-based approach is acceptable<sup>4</sup>

References

1. Crump M, et al. *Blood* 2017;130:1800–1808.  
2. Oppe M, et al. *Pharmacoeconomics* 2016;34:993–1004.  
3. Stolk E, et al. *Value Health* 2019;22:23–30.  
4. National Institute for Health and Care Excellence. NICE health technology evaluations: the manual. <https://www.nice.org.uk/process/pmg36>. Published January 31, 2022. Accessed October 16, 2023.  
5. Hill S, et al. *Value Health* 2022;25:S368.  
6. Helgeson G, et al. *Qual Life Res* 2020;29:1465–1482.

Acknowledgments

- This study was funded by Bristol Myers Squibb
- All authors contributed to and approved the presentation; writing and editorial assistance were provided by Jeremy Henriques, PhD, CMPP, of The Lockwood Group (Stamford, CT, USA), funded by Bristol Myers Squibb