

CLL-1077

Prospective Patient Preference Study for Chronic Lymphocytic Leukemia (CLL) Treatment Attributes Impacting Patient Shared Decision-Making

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Conclusions

- This patient preference survey showed that **progression-free survival (PFS), impact of headache on quality of life (QoL), and impact of atrial fibrillation on QoL** were the top 3 most important attributes of treatment for CLL patients
- Treatment duration (fixed duration vs continuous duration) did not have a statistically significant impact on patient preferences
- To support patient-centred care, shared decision-making in CLL treatment selection should incorporate a comprehensive discussion on adverse events (AEs) alongside efficacy, as patients may prioritize treatments with less impact of AEs on their QoL
- Future prospective studies assessing the effects of shared treatment decision-making on treatment outcomes are warranted, to better understand their impacts on CLL care and clinical practice

Background

- CLL is a largely incurable and heterogeneous disease with a constantly evolving therapeutic landscape in which multiple options exist for treatment^{1,3}
- Outcomes to treatment for CLL differ in terms of efficacy, safety, treatment duration, and monitoring needs, all of which can impact patients’ QoL and overall treatment experience
- While previous studies have assessed preferences for treatment attributes, including treatment duration⁴⁻⁸, none have incorporated attributes including the monitoring components associated with varying treatment durations
- Discrete choice experiment (DCE) is a research method that uses surveys to quantify individual preferences and trade-offs between different features in decision-making

Objective

- To comprehensively understand patient preferences for various CLL treatment attributes, which may impact treatment decision-making

Methods

Data Source and Study Population

- A web-based patient survey with a DCE design was conducted from December 6th, 2024 to February 12th, 2025 among adults (≥18 years) from the United States with a confirmed diagnosis of CLL
- Patients were recruited through online patient panels, physician referrals, and support groups

Study Design

- The DCE survey was developed to assess patients’ preferences for different treatment options for CLL, in accordance with the recommendations of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Good Research Practices for Conjoint Analysis Task Force^{9,10}
- Treatment attributes were selected based on results of a targeted literature review and clinical inputs, including efficacy (PFS), safety (impacts of diarrhea, headache, atrial fibrillation, hypertension, and tumor lysis syndrome [TLS)/kidney dysfunction on QoL), and treatment duration (continuous vs fixed duration with monitoring/hospitalization requirements) (**Table 1**)
- Patients were presented with a series of 11 choice cards in the DCE survey and asked to indicate their preference between two hypothetical treatment profiles (Treatment A and Treatment B), with varying combinations of levels associated with each attribute in each choice card (**Figure 1**)
- The survey additionally included questions related to patients’ sociodemographic and clinical characteristics
- Importance of efficacy measures related to pausing disease progression, increasing the chance of remission or cure, and increasing life expectancy were evaluated on a scale of 0 to 10, with 0 indicating “not at all important” and 10 indicating “extremely important”

Statistical Analysis

- Continuous variables were reported using means, medians, and standard deviations; categorical variables were reported using frequency counts and percentages
- To assess patients’ preferences, DCE data were analyzed using a conditional logistic regression, and derived coefficients were used to calculate the relative importance of each attribute

Table 1. Attributes and Levels

Type of attributes	Attributes	Levels
Efficacy	Prevention of disease progression	3 years 5 years 7 years
	Impact of diarrhea on quality of life	None or mild Moderate Significant
Safety	Impact of headache on quality of life	None or mild Moderate Significant
	Impact of atrial fibrillation on quality of life	None or mild Moderate Significant
	Impact of hypertension on quality of life	None or mild Moderate Significant
	Impact of kidney dysfunction/tumor lysis syndrome on quality of life	None or mild Moderate Significant
Treatment duration	Continuous treatment until cancer progresses with no need for hospitalization or monitoring visits	
	Fixed duration (at least 12 months and the ability to discontinue if the cancer cells in your blood decrease significantly or disappear) with frequent blood tests to monitor for dangerous side effects and potential hospitalization if results are abnormal	
	Fixed duration (at least 12 months and the ability to discontinue if the cancer cells in your blood decrease significantly or disappear) with hospitalization (1-2 days) for monitoring for side effects at the start of treatment	

Figure 1. Example of a Choice Task

Treatment Features	Treatment A	Treatment B
The treatment can <u>prevent disease progression</u> for ...	3 years	5 years
Impact of <u>diarrhea</u> on quality of life	Moderate	None or mild
Impact of <u>headache</u> on quality of life	Moderate	Significant
Impact of <u>atrial fibrillation</u> on quality of life	None or mild	Significant
Impact of <u>hypertension</u> on quality of life	Significant	Moderate
Impact of <u>kidney dysfunction/tumor lysis syndrome</u> on quality of life	Moderate	Significant
<u>Treatment duration</u> (continuous vs fixed duration)	Continuous treatment until cancer progresses with no need for hospitalization or monitoring visits	Fixed duration (at least 12 months and the ability to discontinue if the cancer cells in your blood decrease significantly or disappear) to monitor for dangerous side effects and potential hospitalization if results are abnormal
Which treatment do you prefer?	☐	☐

Note: When a patient hovers over or clicks on an attribute (underlined in the figure), the description of the attribute will be shown in a pop-up window.

Disclosures

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Results

Patient Characteristics

- A total of 199 patients with CLL completed the survey and passed quality checks (median age: 60 years; 91% White; 46% female; 66% with a bachelor’s degree or above; 46% employed; 54% commercially insured; 86% suburban/urban residence) (**Table 2**)

Table 2. Summary of Patient Demographic Characteristics

	Patients (N=199)		Patients (N=199)
Age, mean ± SD [median]	57.9 ± 14.7 [60.0]	Residence area, n (%)	
Gender, n (%)		Suburban or urban	172 (86.4)
Male	107 (53.8)	Rural	27 (13.6)
Female	92 (46.2)	Education level,^a n (%)	
Race, n (%)		Below bachelor’s degree	66 (33.2)
White or Caucasian	180 (90.5)	Bachelor’s degree or higher	132 (66.4)
Black or African American	15 (7.5)	Employment, n (%)	
American Indian or Alaska Native	4 (2.0)	Full-time, part-time, self-employed	91 (45.7)
Asian or Pacific Islander	0 (0.0)	Retired	76 (38.2)
Ethnicity,^a n (%)		Unemployed	15 (7.5)
Not Hispanic or Latino	186 (93.5)	Other	17 (8.5)
Hispanic or Latino	11 (5.5)	Insurance coverage, n (%)	
Region of residence, n (%)		Commercial/private insurance	107 (53.8)
South	74 (37.2)	Public insurance	113 (56.8)
West	45 (22.6)	Other health insurance	2 (1.0)
Midwest	42 (21.1)	Uninsured	1 (0.5)
Northeast	38 (19.1)	Prescription medications covered by insurance	186 (93.9)

^aResponse categories do not add up to 100% because the proportion of respondents who selected “Prefer not to answer” is not presented in the table. SD, standard deviation.

- Almost half (49%) of patients were diagnosed ≥5 years ago (**Table 3**)
- While 30% of patients had received ≥3 lines of therapy, 23% of all patients were treatment-naïve, 25% received 1 line of prior therapy, and 23% received 2 lines of prior therapy (**Table 3**)
- Most patients (88%) reported having experienced ≥1 AE from treatment previously, with the most common AEs being headache (53%), fatigue (53%), diarrhea (44%), and nausea and/or vomiting (34%) (**Table 3**)

Table 3. Summary of Patient Clinical Characteristics

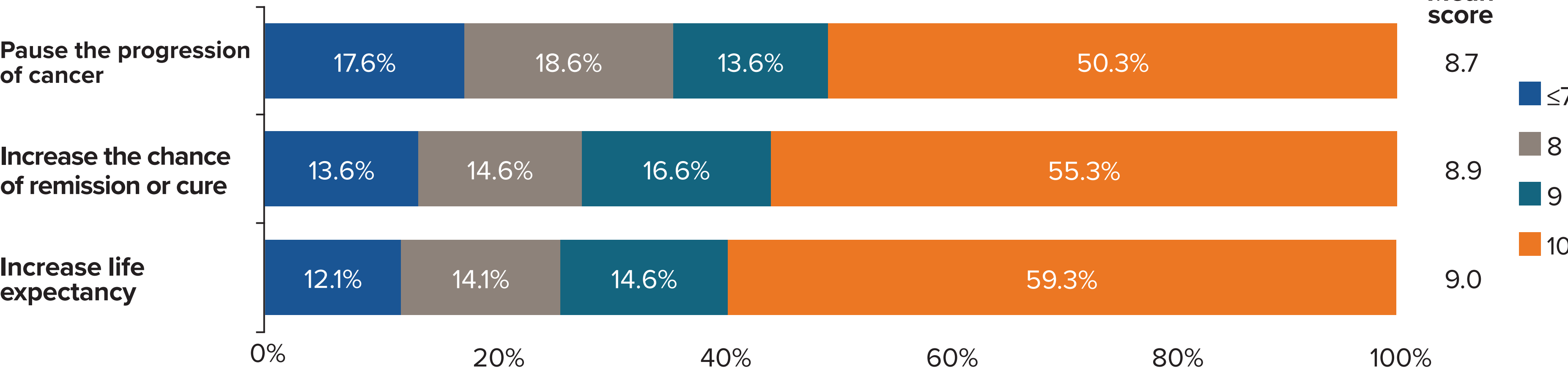
	Patients (N=199)		Patients (N=199)
Time since diagnosis, n (%)		Most common side effects experienced from treatment,^{a,b} n (%)	N=154
Less than a year ago	9 (4.5)	≥1 side effect ^c	136 (88.3)
1 to < 2 years ago	31 (15.6)	Headache	82 (53.2)
2 to < 5 years ago	61 (30.7)	Fatigue or extreme tiredness	82 (53.2)
5 or more years ago	98 (49.2)	Diarrhea	67 (43.5)
Line of treatment, n (%)		Nausea and/or vomiting	52 (33.8)
Treatment-naïve	45 (22.6)	Anemia	47 (30.5)
First line	49 (24.6)		
Second line	45 (22.6)		
Third line and above	60 (30.2)		

^aCategories were not mutually exclusive; ^bAsked among participants who have ever received any treatment for their blood cancer; ^cOnly the five most common side effects are presented.

Importance of Efficacy Measures

- Of patients who rated the importance of efficacy measures to be 8, 9, or 10, most prioritized CLL treatments that extended life expectancy (88%), followed by those that increased the likelihood of remission or cure (86%) and those that paused the progression of disease (82%), with average rating scores of 9.0, 8.9, and 8.7 out of 10, respectively (**Figure 2**)

Figure 2. Importance of Efficacy Measures

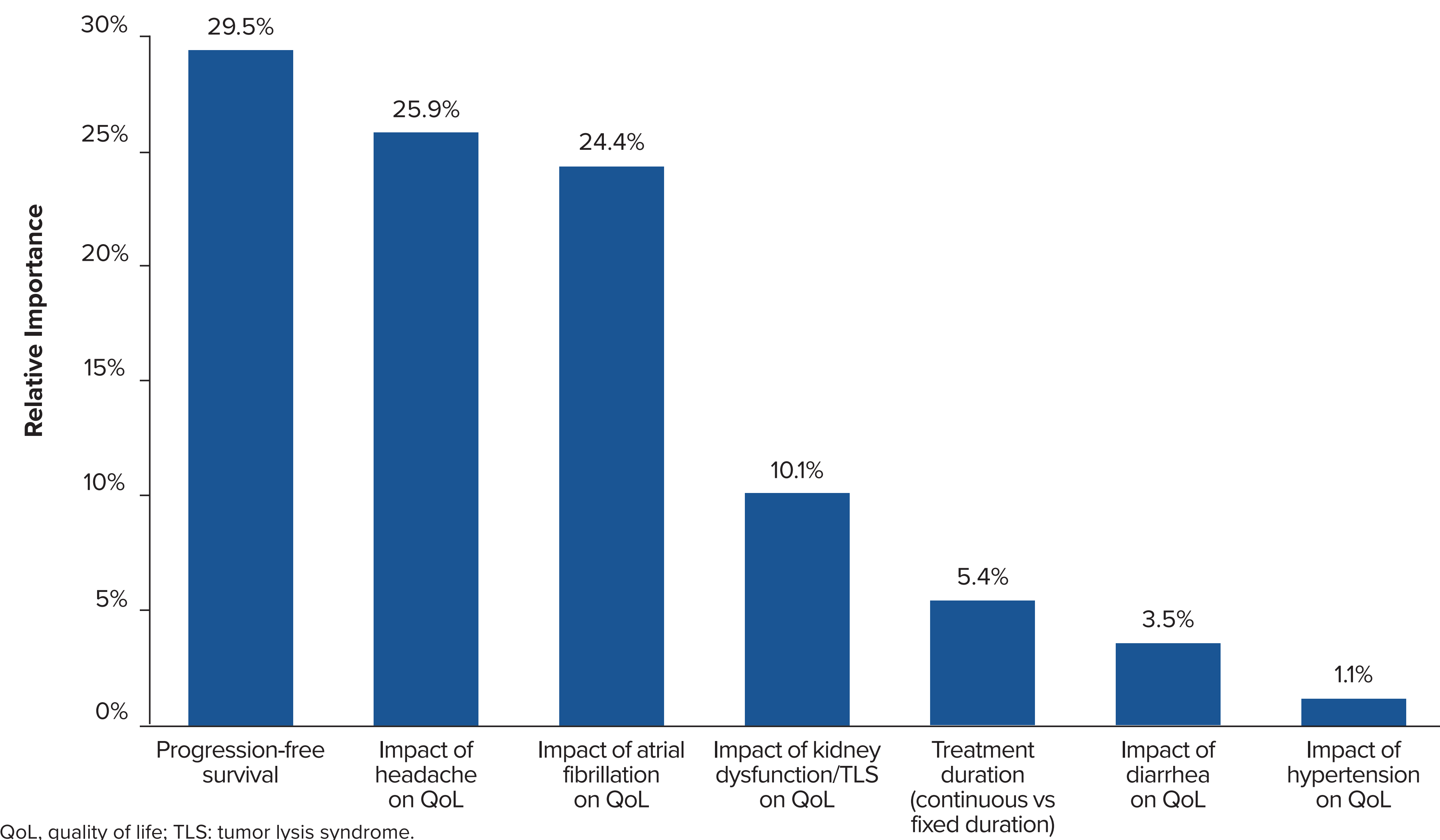


Note: Patients were asked to rate the importance of each efficacy measure in their decision to select a treatment using a scale from 0 to 10, with 0 indicating “not at all important” and 10 indicating “extremely important”. The bar plot displays the percentage of patients who rated each efficacy measure at ≤7, 8, 9, and 10. Additionally, the mean score for each efficacy measure was calculated as the average rating across all patients.

Patient Preference from DCE Results

- The top 3 treatment attributes with the highest relative importance to patients were **PFS (29.5%), impact of headache (25.9%) and impact of atrial fibrillation on QoL (24.4%)**, followed by impact of kidney dysfunction/TLS (10.1%) on QoL, treatment duration (5.4%), and impact of diarrhea (3.5%) and hypertension (1.1%) on QoL (**Figure 3**)
- The DCE results showed that patients preferred treatments that resulted in longer PFS and reduced impact of headache, atrial fibrillation and kidney dysfunction/TLS on QoL ($P<.001$). Impact of diarrhea and hypertension on QoL and treatment duration (continuous vs fixed duration) did not have a statistically significant influence on treatment preferences

Figure 3. Relative Importance of Attributes to Patients



QoL, quality of life; TLS: tumor lysis syndrome.

Discussion

- Understanding patients’ perspectives on treatment attributes is critical to educating both patients and healthcare providers to help with shared decision-making
- Additionally, treatment duration (fixed duration vs continuous duration) did not significantly impact patient preferences in this study. While a previous study reported that patients preferred fixed duration over continuous treatment,¹¹ it did not evaluate mode of administration and practical burdens such as hospitalization and blood test requirements. The findings reported here highlight that preferences may change when both treatment duration and monitoring/hospitalization requirements are considered
- The perspectives captured in this DCE survey may also not be reflective of the overall population of patients with CLL, limiting generalizability. While the number of attributes included in this DCE survey were in line with DCE literature guidelines, treatment attributes were limited to minimize participant response burden. Other treatment attributes not assessed in this study may have an impact on patient preferences

References

- Patel K, et al Blood. 2019;134(19):1573-1577.
- Oscier D, et al Br J Haematol. 2012;5(159):541-564.
- Goede V, et al Drugs Aging. 2011;28:163-176.
- Landfeldt E, et al Leuk Res. 2016;40:17-23.
- Laurenti L, et al HemaSphere. 2022;6(9):e771.
- Le H, et al Patient Prefer Adherence. 2021;15:99-110.
- Mansfield C, et al Blood Adv. 2017;1(24):2176-2185.
- Le H, et al Blood. 2019;134:4730.
- Bridges JFP, et al Value Health. 2023;26(2):153-162.
- Reed Johnson F, et al Value Health. 2013;16(1):3-13.
- Ravelo A, et al Future Oncol. 2024;20(28):2059-2070