A Multinational Study to Explore Patient Preferences for Chronic Myeloid Leukaemia (CML) Treatment: *Results from the United States and Canada*

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INTRODUCTION While tyrosine kinase inhibitors (TKIs) allow most patients with chronic-phase chronic myeloid leukemia (CML) to achieve near-normal life expectancy, they are associated with a range of potential side effects. Maintaining tolerability is important to sustain treatment adherence and thereby achieve optimal outcomes. Therefore, **understanding patient preferences is critical to ensure that treatment decisions reflect what matters most to patients**.

METHODS We administered an online discrete choice experiment (DCE) to understand patient preferences for TKI treatment. Patient respondents were recruited via the CML Advocates Network from North America (USA, Canada), Europe (UK, France, Germany, Spain, Italy), and East Asia (China, Japan). We focus here on USA and Canada. The DCE asked respondents to make 12 choices between hypothetical treatments (Figure 1). We estimated overall **part-worth utilities (PWUs) for attribute levels** and **attribute relative importance** by the respondents' line of therapy (LoT).

TABLE 1: Respondent characteristics

	USA (N=77)	Canada (N=27)	Combined (N=104)
Data collection			
Opened	2025-04-29	2025-05-01	
Data cut	2025-08-11	2025-08-11	
Female	50 (64.9%)	21 (77.8%)	71 (68.3%)
Age group			
18-34	6 (7.8%)	1 (3.7%)	7 (6.7%)
35-44	13 (16.9%)	2 (7.4%)	15 (14.4%)
45-54	13 (16.9%)	3 (11.1%)	16 (15.4%)
55-64	15 (19.5%)	13 (48.1%)	28 (26.9%)
65-74	23 (29.9%)	7 (25.9%)	30 (28.8%)
75+	7 (9.1%)	1 (3.7%)	8 (7.7%)
Time since CML diagnosis			
<90 days	1 (1.3%)	0 (0.0%)	1 (1.0%)
90-365 days	13 (16.9%)	5 (18.5%)	18 (17.3%)
1-5 years	30 (39.0%)	7 (25.9%)	37 (35.6%)
6-10 years	16 (20.8%)	6 (22.2%)	22 (21.2%)
>10 years	17 (22.1%)	9 (33.3%)	26 (25.0%)
Line of treatment			
Not yet started	1 (1.3%)	0 (0.0%)	1 (1.0%)
First	34 (44.2%)	10 (37.0%)	44 (42.3%)
Second	21 (27.3%)	8 (29.6%)	29 (27.9%)
Third	10 (13.0%)	2 (7.4%)	12 (11.5%)
Fourth+	8 (10.4%)	3 (11.1%)	11 (10.6%)
Treatment-free (TFR)	3 (3.9%)	4 (14.8%)	7 (6.7%)
Analytic sample	**75**	**27**	**102**

RESULTS We excluded 2 "flatliners" who always chose A or always chose B in all tasks, giving an analytic sample of 102 respondents (Table 1). In Figure 2A, the vertical bars show the relative importance of each attribute in respondent choices, by line-of-therapy (LoT), excluding respondents in TFR (N=7). Figure 2B shows overall part-worth utilities (PWUs), or relative value of each attribute level relative to its baseline level (PWU=0). Positive PWUs indicate more preferred levels; negative PWUs represent less preferred levels. Error bars represent 95% confidence intervals (CIs) around relative importance and PWUs.

Day-to-day experience of fatigue was the most important attribute in respondent choices, followed by chance of DMR, risks of adverse events, and how treatment is taken. There were no significant differences by LoT. Overlapping CIs suggest most attributes had similar weight within both LoT groups.

PWUs indicate that respondents preferred lower levels of fatigue, a higher chance of DMR, lower risk of adverse events (AEs), and more convenience in how treatment is taken, especially avoiding fasting. Based on these PWUs, respondents would theoretically be willing to forego a 23.6% increase in the chance of DMR to avoid moving from moderate to strong day-to-day fatigue.

DISCUSSION The importance of fatigue in patient choices – ahead of chance of DMR – highlights **fatigue is an important unmet need in the treatment of CML**. These results also confirm **ongoing unmet needs around a better chance of treatment success and lower risks for all adverse events**.

With respect to previous DCEs in CML, Mason *et al.*[1] tested a slightly different set of attributes and found that patients prioritised the risk of a blockage of a major blood vessel over all other attributes, including risk of fatigue. They also found a strong aversion to fasting requirements, and that this attribute was more important than AEs like nausea and diarrhoea. Hirji *et al.*[2] explored preferences and willingness-to-pay (WTP) for different options of dosing frequency and fasting. WTP was greatest for dosing options that did not require fasting. We found a similar aversion to fasting, but this attribute was less important than the other aspects of treatment we tested, and that the importance of long-term CV problems was similar to the other AEs we tested.

These results demonstrate **the importance of understanding and accounting for the day-to-day experience of patients** alongside clinical endpoints like chance of DMR and adverse events when assessing the value of treatments for CML.

[1] Mason *et al.* Quantifying Patient Preferences for Tyrosine Kinase Inhibitors in Chronic Myeloid Leukemia: A Discrete-Choice Experiment. European Hematology Association 2021.

[2] Hirji *et al.* Patient Preferences for Chronic Myeloid Leukemia Medication Regimen Attributes and their Potential Impact on Adherence: Results from a Multi-national Conjoint Study. JHEOR. 2014 Sep 3;2(1):75-86.

DCE ATTRIBUTES & LEVELS The DCE was developed in consultation with patient stakeholders from all study countries. Attributes were identified through qualitative interviews with patients and levels were assigned to represent plausible ranges based on clinical evidence (Table 2).

TABLE 2: DCE attributes and levels

Attribute	Levels			
How treatment is taken	Once per day, No fasting	Twice per day, No fasting	Once per day, 3-hour fast	Twice per day, 3-hour fast
Chance of Deep Molecular Response (DMR) within 2yrs	15 out of 100 patients	35 out of 100 patients	50 out of 100 patients	
Experience of fatigue	No fatigue most days	Moderate fatigue most days	Strong fatigue most days	
Risk of gastrointestinal (GI) problems	15 out of 100 patients	45 out of 100 patients	75 out of 100 patients	
Risk of breathing problems	5 out of 100 patients	25 out of 100 patients	40 out of 100 patients	
Risk of long-term cardiovascular (CV) problems	5 out of 100 patients	15 out of 100 patients	25 out of 100 patients	

FIGURE 1: Example choice task

Treatment A	Treatment B
How the treatment is taken	**How the treatment is taken**
Pill taken **once daily** (with or without food)	Pill taken **twice daily** on an **empty stomach** (no food 2 hours before and 1 hour after)
Chance of Deep Molecular Response (cancer cells reducing to a very low level) within 2 years	**Chance of Deep Molecular Response (cancer cells reducing to a very low level) within 2 years**
35 out of every 100 patients (35%)	15 out of every 100 patients (15%)
Experience of fatigue	**Experience of fatigue**
Strong fatigue most days	**No fatigue** most days
Risk of gastrointestinal problems	**Risk of gastrointestinal problems**
75 out of every 100 patients (75%)	25 out of every 100 patients (25%)
Risk of breathing problems	**Risk of breathing problems**
40 out of every 100 patients (40%)	10 out of every 100 patients (10%)
Risk of long-term cardiovascular problems	**Risk of long-term cardiovascular problems**
10 out of every 100 patients (10%)	5 out of every 100 patients (5%)
☐ **Select Treatment A**	☐ **Select Treatment B**

FIGURE 2A: Relative attribute importance (N=95, excluding TFR)

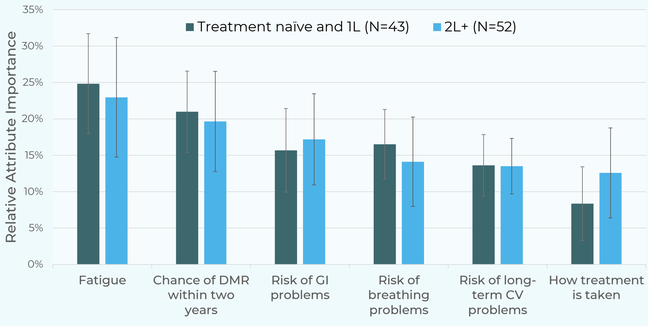
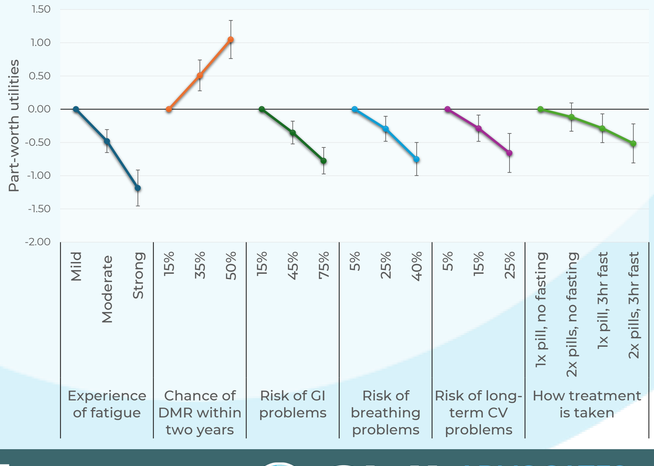


FIGURE 2B: Part-worth utilities by attribute (N=102)



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