Prospective Patient Preference Study for Bruton Tyrosine Kinase Inhibitor (BTKi) Treatment Attributes and Factors Affecting Patient Shared Decision-Making in Chronic Lymphocytic Leukemia (CLL) and Small Lymphocytic Lymphoma (SLL) in the United States (USA)

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Introduction: The prognosis for CLL/SLL has improved with the advent of novel therapeutic classes, including BTKis. While comparative data for several BTKis have been published, there are limited data on patient preferences in BTKi treatment selection. Understanding and integrating patient perspective in the BTKi treatment selection process is crucial to shared decision-making and attaining optimal treatment outcomes. To understand patients' priorities for different treatment attributes that impact their treatment decisions, a comprehensive quantitative analysis of patient preferences on BTKi treatment attributes was conducted.

Methods: A patient survey with a discrete-choice experiment (DCE) design was conducted from March to June 2024 among USA adults (≥18 years) with confirmed diagnosis of CLL/SLL, recruited through online patient panels, physician referrals, and support groups. BTKi treatment attributes were selected based on results of a targeted literature review and clinical inputs. Patients responded to DCE questions on attributes related to efficacy, safety (eg, impacts of diarrhea, headache, atrial fibrillation, and hypertension on quality of life [QoL]), formulation type, and dosing frequency. The impact of adverse events (AEs) on QoL was defined as the extent to which AEs caused interruptions in patients' ability to engage in their usual day-to-day activities. A conditional logistic regression model was used to calculate the relative importance of each attribute, as well as patient willingness to trade off specified BTKi treatment attributes.

Results: A total of 200 patients with CLL/SLL completed the survey (median age: 61 years; 78% White; 55% female; 60% commercially insured; 82% suburban/urban residence). Less than half (43%) were diagnosed ≥5 years ago, and 61% received ≥3 lines of therapy. Almost all (89%) patients reported having experienced ≥1 AE from treatment previously, with the most common AEs being fatigue (76%), diarrhea (51%), headache (51%), and nausea and/or vomiting (51%). When considering the importance of efficacy measures, most patients prioritized CLL/SLL treatments that extended life expectancy (93%), followed by those that increased the likelihood of remission or cure (84%) and those that paused the progression of disease (67%). Patients preferred treatments with higher efficacy, less impact of AEs on QoL, and lower dosing frequency (*P*<.001). The top 3 treatment attributes with the highest relative importance to patients were impact of atrial fibrillation on QoL (24%), progression-free survival (PFS; 19%), and impact of headache on QoL (18%), followed by impact of diarrhea (14%) and hypertension (14%) on QoL, dosing frequency (9%), and formulation type (3%). On average, patients were willing to accept a reduction of 2.6, 1.9, 1.4, and 1.4 years of PFS to receive a treatment with less (none or mild vs significant) impact of atrial fibrillation, headache, diarrhea, and hypertension on QoL, respectively. Patients were willing to accept a reduction of 1.0 year of PFS to receive once-daily vs twice-daily treatment.

Conclusions: Findings from this patient preference survey suggested that impact of atrial fibrillation on QoL, PFS, and impact of headache on QoL were the most important attributes of BTKi treatment for patients with CLL/SLL in the USA. Shared decision-making in CLL/SLL treatment selection should include an informed discussion about AEs, as besides efficacy comparisons, patients may prefer treatments with less impact of AEs on their QoL. Future prospective studies evaluating the effects of shared treatment decision-making on treatment adherence and outcomes are needed to better understand their impact on CLL/SLL patient care and inform clinical practice.