



## The 65th ASH Annual Meeting Abstracts

## POSTER ABSTRACTS

## 902.HEALTH SERVICES AND QUALITY IMPROVEMENT - LYMPHOID MALIGNANCIES

**Patient Preferences for Fixed Versus Treat-to-Progression Therapies in Chronic Lymphocytic Leukemia**

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The treatment landscape for chronic lymphocytic leukemia (CLL) has evolved in the last 8 years to include novel targeted therapies as alternatives to chemoimmunotherapy. These treatments vary in their benefit-risk profiles, mode and frequency of administration, and duration of treatment. Previous preference studies in CLL have evaluated preferences for treatment attributes such as efficacy, tolerability, and mode and frequency of administration. However, few have assessed preferences for treatment duration independently of mode of administration. This study sought to further understand the factors that influence patient preferences for treatment duration and to quantify the tradeoffs that patients were willing to accept to have a fixed duration therapy versus a treat-to-progression therapy.

The study was conducted in 2 phases: (1) a qualitative phase of in-depth individual interviews with patients to identify the factors that influence patient preferences for treating until progression versus a fixed duration as well as the CLL treatment attributes that patients consider most important, and (2) a quantitative phase to estimate the tradeoffs that patients would accept among CLL treatment attributes. In the qualitative phase, the semistructured interview guide included open-ended questions and probes to understand perceptions of fixed-duration treatments compared with treat-to-progression regimens and to elicit a list of treatment attributes that influence CLL treatment preference. The results of the interviews informed the development of a web-based discrete-choice experiment (DCE) survey. Respondents to the online survey answered 12 DCE questions, each offering a choice between 2 hypothetical treatment profiles defined by 7 attributes with varying levels, including treatment duration, which included levels for treat to progression and fixed duration for either 6 or 12 months. Data were analyzed using a random-parameters logit model, and estimated preference weights were used to calculate the maximum acceptable risk (MAR) of treatment-related adverse events the average respondent would accept in exchange for a move from a treat-to-progression to a fixed-duration therapy. The MAR is estimated as the ratio of the relative importance of an improvement in an attribute to the relative importance of a unit change in the level of risk (i.e., tumor lysis syndrome (TLS), atrial fibrillation, or fatigue).

Interviews were conducted with 20 adults with a self-reported diagnosis of CLL. The mean age was 59 years, and 55% of participants (n = 11) identified as female. The mean time since diagnosis of CLL was 3 years, and 70% of participants (n = 14) had received treatment for CLL. Overall, 93 treatment attributes were spontaneously reported, including treatment duration. When probed, treatment duration was reported by 50% of participants as "very important" in their treatment decision. Almost all participants (n = 17) preferred a treatment with a fixed duration compared with treat-to-progression, assuming each had the same efficacy. Table 1 reports patients' perceived benefits and drawbacks of fixed-duration versus treat-to-progression therapies.

The DCE survey was completed by 229 adults with a self-reported diagnosis of CLL for at least 3 months. The mean age was 66 years, and nearly 60% of the sample identified as female (n = 136). About 60% of the sample (n = 138) was diagnosed 5 or more years ago, and 152 respondents (66%) had experience with treatment for CLL. The results of the preference analysis showed that respondents preferred a fixed duration of either 6 months or 12 months versus treat-to-progression and, based on the MAR estimates, were willing to accept the following levels of risks to have a fixed-duration treatment versus treat to progression: more than a 3% increased risk of TLS, 6%-7% increased risk of atrial fibrillation, and 21%-26% increased risk of fatigue (Table 2).

Past research has shown efficacy as the most important factor, yet qualitative interview participants also identified treatment duration as an important factor in their decision when choosing a CLL therapy. This finding was confirmed by the quantitative

preference study, which revealed a preference for fixed-duration therapies over treat-to-progression regardless of the time-frame (6 or 12 months). Results from this study can help inform shared decision-making when considering alternative therapies for CLL.

**Disclosures Ravelo:** *Genentech Inc.:* Current Employment, Current holder of *stock options* in a privately-held company. **Myers:** *RTI Health Solutions:* Current Employment, Other: I am a full time salaried employee of RTI Health Solutions which is a not for profit research institute which conducts research for the pharmaceutical industry. My salary is unconnected to the projects on which I work.. **Ervin:** *RTI Health Solutions:* Current Employment, Other: I am a full time salaried employee of RTI Health Solutions which is a not for profit research institute which conducts research for the pharmaceutical industry. My salary is unconnected to the projects on which I work.. **Bussberg:** *RTI Health Solutions:* Current Employment, Other: I am a full time salaried employee of RTI Health Solutions which is a not for profit research institute which conducts research for the pharmaceutical industry. My salary is unconnected to the projects on which I work.. **Koffman:** *Lilly:* Other: Funding to affiliated organisation; *Leukemia & Lymphoma Society (LLS):* Other: Funding to affiliated organisation; *Janssen:* Other: Funding to affiliated organisation; *Gilead:* Current equity holder in publicly-traded company, Other: Funding to affiliated organisation; *AbbVie:* Current equity holder in publicly-traded company, Other: Funding to affiliated organisation; *Astra Zeneca:* Current equity holder in publicly-traded company, Other: Funding to affiliated organisation ; *BeiGene:* Current equity holder in publicly-traded company, Other: Funding to affiliated organisation; *Loxo Oncology:* Other: Funding to affiliated organisation; *Bristol Myer Squibb:* Current equity holder in publicly-traded company, Honoraria; *Johnson & Johnson:* Current equity holder in publicly-traded company, Honoraria; *MEI Pharma:* Current equity holder in publicly-traded company; *Merck:* Current equity holder in publicly-traded company; *Miragen Therapeutics:* Current equity holder in publicly-traded company; *Novartis:* Membership on an entity's Board of Directors or advisory committees; *Portola Pharma:* Current equity holder in publicly-traded company; *Regeneron:* Current equity holder in publicly-traded company; *Sunesis Pharmaceuticals:* Current equity holder in publicly-traded company; *TG Therapeutics:* Current equity holder in publicly-traded company. **Manzoor:** *AbbVie Inc.:* Current Employment, Current holder of *stock options* in a privately-held company. **Biondo:** *Genentech, Inc.:* Current Employment; *F. Hoffmann-La Roche Ltd.:* Current holder of *stock options* in a privately-held company; *Genentech, Inc.:* Ended employment in the past 24 months. **Mansfield:** *RTI Health Solutions:* Current Employment, Other: I am a full time salaried employee of RTI Health Solutions which is a not for profit research institute which conducts research for the pharmaceutical industry. My salary is unconnected to the projects on which I work..

**Table 1. Participant-Reported Perceived Benefits and Drawbacks of Fixed-Duration Versus Treat-to-Progression Therapies**

	Benefits	Drawbacks
Fixed duration	<ul style="list-style-type: none"> <li>▪ Budgeting and anticipating expenses (i.e., being able to plan for medical expenses and not having to pay for treatment repeatedly over an indefinite period of time)</li> <li>▪ Convenience (i.e., not having to take a treatment [freedom from medication])</li> <li>▪ Being more in control</li> <li>▪ Not having to refill prescription</li> <li>▪ Not having to travel for treatment</li> <li>▪ No short-term side effects when off treatment</li> <li>▪ Reduced risk of long-term side effects</li> <li>▪ Getting back to "normal" life</li> </ul>	<ul style="list-style-type: none"> <li>▪ Concentrated costs (the cost can be very high over a short period of time)</li> <li>▪ Side effects might be worse if treatment duration is shorter</li> <li>▪ Their CLL might worsen or spread if they are not taking a medication</li> </ul>
Treat-to-progression	<ul style="list-style-type: none"> <li>▪ Doing something (i.e., the feeling of comfort gained by taking action and treating their cancer)</li> </ul>	<ul style="list-style-type: none"> <li>▪ Worry that the medicine may become less effective over time</li> <li>▪ Cost of treatment</li> <li>▪ Taking a medicine continuously is a constant reminder of the cancer</li> <li>▪ Inconvenience (i.e., always taking a medicine)</li> <li>▪ Getting refills</li> <li>▪ Following up with nurse or pharmacy</li> <li>▪ Continual risk of short- and long-term side effects</li> </ul>

CLL = chronic lymphocytic leukemia.]

**Table 2. Maximum Acceptable Risk of Treatment Side Effects in Exchange for Improvements in Chronic Lymphocytic Leukemia Treatment Characteristics**

Attribute	From level	To level	MAR of tumor lysis syndrome	MAR of atrial fibrillation	MAR of fatigue
			Mean (95% CI)		
Duration of treatment	Until the cancer progresses (gets worse)	Fixed: 12 months	> 3.0	6.2 (4.2-8.1)	21.2 (12.3-30.2)
	Until the cancer progresses (gets worse)	Fixed: 6 months	> 3.0	7.4 (5.3-9.4)	26.2 (17.1-35.2)
	Fixed: 12 months	Fixed: 6 months	N/A <sup>a</sup>	N/A <sup>a</sup>	N/A <sup>a</sup>

CI = confidence interval; MAR = maximum acceptable risk; N/A = not applicable; TLS = tumor lysis syndrome.

Note: MAR estimates outside the range of levels included in the study are noted as greater than the largest difference in levels of risk of TLS, atrial fibrillation, and fatigue: 3%, 10%, and 35%, respectively. Confidence intervals are not reported for these estimates. It is possible to estimate a specific value for the MAR outside the range of levels included in the study only by making the strong assumption that the disutility of each unit increase in risk remains constant beyond the greatest level of risk.

<sup>a</sup> The difference between these levels was not statistically significant. Therefore, the MAR for this change cannot be calculated as the change from 1 level to the other does not have a statistically meaningful impact on the average respondent's preferences.

**Figure 1**

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