

Introduction

In recent years there has been a drive to better understand patients' treatment preferences to inform decision making. Quantitative evidence may be useful for informing regulatory and reimbursement decisions. To date there have been few quantitative preference studies in the context of acute leukemia (AL).

AL is a cancer of the white blood cells which progresses rapidly and aggressively. For some adult patients, following first-line treatment, remission is not achieved ('refractory disease'), and for others the leukemia returns after achieving remission ('relapse'). For these individuals, subsequent outcomes are typically poor. It is therefore important to understand patients' treatment priorities in this context.

The Acute Leukemia Advocates Network (ALAN), in collaboration with the Office of Health Economics (OHE), ran a quantitative patient preference study using a discrete choice experiment (DCE) to understand patients' treatment priorities in the relapse/refractory (R/R) setting.

Methods

Building upon formative qualitative research consisting of online bulletin boards (with a total of 21 participants with different types of AL) and 10 online one-on-one think-aloud pre-testing interviews conducted with people with AL, an online survey containing DCE was designed to explore patients' preferences for treatments in the R/R setting. In the DCE, respondents were asked to make choices between two hypothetical treatments.

The treatment characteristics (DCE attributes) were mode of administration (MoA); quality of life (QoL) during treatment; chance of response; duration of response; and QoL during response. Each respondent completed twelve scenarios containing two hypothetical treatments (Figure 1). After the DCE, participants were asked a range of demographic and clinical status questions.

Figure 1: Example of DCE scenario

	Treatment A	Treatment B	No active treatment
The experience of taking the treatment			
Duration of treatment	6 months Treatment	6 months Treatment	
Mode of administration	Injections (at regular outpatient hospital appointments) and tablets (taken at home)	Injections (at regular outpatient hospital appointments) and tablets (taken at home)	
Quality of life whilst you are receiving the treatment	50	25	
The chance of responding to the treatment			
Chance of responding to treatment	95%	65%	
The outcome of the treatment if you respond			
Duration of the response to treatment	18 months	9 months	
Quality of life whilst you are responding to the treatment	75	50	
Which would you choose?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Participants were recruited via Leukaemia Care, UK member organization of ALAN, and ALAN's website, newsletters and social media channels. They were eligible if they lived in the UK and had a diagnosis of AL. The data were analyzed using a latent class model.

Results

There was a total of 95 respondents of which 61 had acute myeloid leukemia (AML), 22 had acute lymphoblastic leukemia (ALL) and 12 had acute promyelocytic leukemia (APL). 59% (n=56) of respondents were female. Demographics and clinical status were varied.

Patient Preferences for Treatment in Relapsed/Refractory Acute Leukemia in the United Kingdom

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- Chance of response is the most important aspect of treatment within the R/R acute leukemia.
- There are clear variations among patients' preferences:
 - Some focus almost exclusively on the chance of response (more common for recently diagnosed patients).
 - Others care about a wide range of factors, including quality of life, duration of response, and mode of administration.

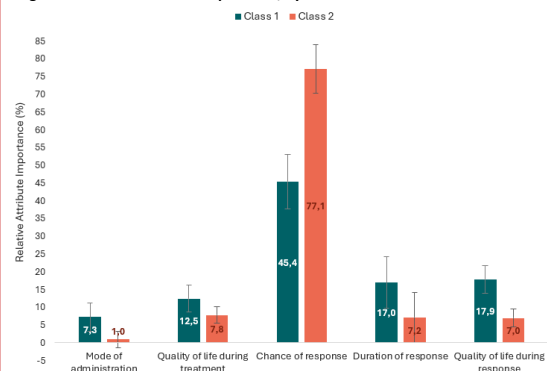
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Results

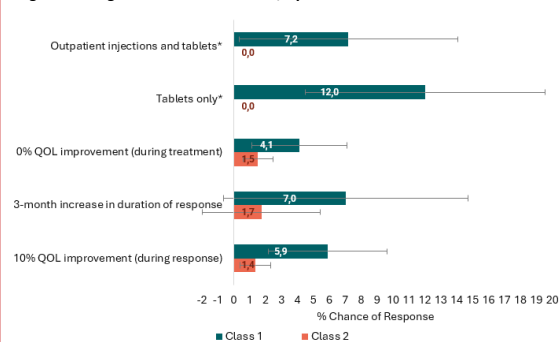
The latent class analysis identified two classes of respondents that had different preferences with around 63% of respondents fitting into class 1 and 37% into class 2 (Figure 2). For both, chance of response was the most important attribute. However, for class 1, every attribute was important whereas, for class 2, the only important attributes were QoL (during treatment and response) and chance of response. A greater proportion of respondents would fall into class 1 overall, and those with ALL or APL and those more recently diagnosed were more likely to be in class 2.

Figure 2: Relative attribute importance, by class



Respondents in class 1 were more willing to trade off some chance of response to obtain better treatment characteristics (Figure 3). For example, on average, people in this class would be willing to trade a 12% chance of response to receive a treatment as tablets taken at home only, rather than having injections during an inpatient hospital stay. Furthermore, they would be willing to trade a 5.9% chance of response for a 10% increase in QoL during response. In contrast, those in class 2 would not be willing to trade off any chance of response for a better MoA and would only be willing to trade 1.4% chance of response for a 10% increase in QoL during response.

Figure 3: Marginal rates of substitution, by class



Conclusion

In this study, we sought to understand patients' preferences for the treatment of R/R AL. Based on the DCE results, the most important aspect of treatment within this setting is the chance of response.

However, there is clear variation in patients' preferences - some care almost exclusively about the chance of response, whereas others care about a range of factors (including QoL, duration of response, and MoA).

Whilst our study has provided insights into the broad treatment preferences of people with AL, the focus has been on the R/R setting and all respondents were from the UK. Further research into patients' broad treatment preferences across different countries (USA, Germany, France, Italy and Spain) and treatment settings (e.g., first line) is ongoing and may also provide additional useful insights.