

Introduction

Evidence on patient preferences is increasingly being used to inform regulatory and reimbursement decisions. In acute leukemia, the number of treatment options is expanding but there is limited quantitative evidence on patients' treatment preferences, especially in the event of relapse.

The Acute Leukemia Advocates Network (ALAN) in collaboration with The Office of Health Economics (OHE) are running a quantitative patient preference study using a discrete choice experiment (DCE) to:

- Elicit adult acute leukemia patients' preferences for treatment outcomes and characteristics in the event of a future relapse
- Explore the tradeoffs that patients are willing to make between different hypothetical treatments
- Describe preference heterogeneity in the patient population and;
- Provide useful insights that could demonstrate patient value for a range of upcoming health technology assessments of relapsed/refractory treatments and guide innovation and development throughout the medical product lifecycle.

Here we report on two stages of qualitative research: 1/ Online bulletin boards (OBBs) to identify potential attributes for the DCE and 2/ Cognitive 'think aloud' pilot interviews to test participant understanding of the draft DCE survey.

Methods

Two structured OBBs were conducted – one each for acute myeloid leukemia (AML) and acute lymphoblastic leukemia (ALL). Each day, participants were asked up to four open questions. Participants could see and respond to each other's answers. Three researchers acted as moderators and engaged in discussions where appropriate.

Figure 1: Structure of each OBB

Day 1: Your diagnosis and expectations of treatment	Day 2: Your first experience of treatment	Day 3: Treatment following a relapse	Day 4: Your treatment priorities
<ul style="list-style-type: none"> How were you diagnosed with acute leukaemia? How did the diagnosis affect you? What were your expectations about treatment (before receiving it)? How did you feel about the way treatment was discussed with you? 	<ul style="list-style-type: none"> What type of treatment did you receive/are you receiving? How is/was your experience with treatment? 	<ul style="list-style-type: none"> A. Not relapsed <ul style="list-style-type: none"> Would you have different expectations around treatment? Why? What would be your main concerns about treatment? Etc. B. Relapsed <ul style="list-style-type: none"> How did you find out that you had relapsed? How did you feel about the treatment options following your relapse(s)? 	<ul style="list-style-type: none"> What are the main aspects of treatment that would be most important to you if you were to need treatment for your acute leukaemia again in the future? What trade-offs would you be willing to make when it comes to future treatment? Do you think it is likely that you would reject treatment in the future? If so, why?

Following the design of the DCE survey, a series of online one-on-one think-aloud interviews with AML and ALL participants was subsequently conducted to pilot the DCE survey. Interviewees were asked to verbally reflect on their responses, with occasional interviewer prompts. The pilots were conducted in two separate weeks, with a week in between to allow for changes to be made.

All participants were recruited via ALAN and its members.

Results

A total of 21 patients took part in the OBBs.

Table 1: Characteristics of participants

Characteristic	TOTAL (AML+ALL)	AML	ALL
Count	21	12 (57.1%)	9 (42.9%)
Average age age range: 26-71	51.1	53.1	48.4
Gender	6 males 15 females	5 males 7 females	1 male 8 females
Risk level/subtype		Standard risk 2 (16.7%)	Ph- 5 (55.6%)
		High risk 3 (25%)	Ph+ 2 (22.2%)
		APL 1 (8.3%)	Left blank 2 (22.2%)
		Unknown 6 (50%)	
One relapse	2 (9.5%)	2 (16.7%)	0
One transplant	9 (42.9%)	4 (33.3%)	5 (55.6%)

Understanding Relapsed/Refractory Acute Leukemia Patients' Treatment Preferences: Insights from Qualitative Research

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- Effectiveness of the treatment in achieving long-term stable remission is the most important consideration
- Severe long-term side effects is an important concern, but some patients would put up with severe short-term side effects for a good chance of long-term survival
- Length of hospital stays, availability of psychological/mental/emotional support and mode of administration are also a concern

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Results

Amongst the 21 patients, years of living with the disease were varied: 29% (n=6) were diagnosed in the last 3 years, 19% (n=4) were diagnosed 3-5 years ago, 29% (n=6) were diagnosed 5-10 years ago and 24% (n=5) more than 10 years ago.

Both the AML and ALL groups agreed that the effectiveness of the treatment in achieving long-term stable remission would be most important. Severe long-term side effects would also be an important concern, but many participants said they would put up with potentially severe short-term side effects for a good chance of long-term survival. Additional concerns were the length of hospital stays, availability of psychological/mental/emotional support, catheter-related pain and infections, and mode of administration. Several participants argued that an individualized approach to treatment is preferred.

Thematic analysis of the OBB data led to the identification of five attributes for the DCE: chance of response (20-95%), duration of response (6-18 months), quality of life (QoL) during treatment (0-50%), QoL during response (25-75%), and mode of administration (tablets taken at home, injections requiring a hospital stay, injections at outpatient appointments). The DCE was designed using an efficient experimental design and coded as part of an online survey, and subsequently piloted.

Figure 2. Example DCE Scenario

	Treatment A	Treatment B	No active treatment
The experience of taking the treatment	6 months Treatment	6 months Treatment	
Duration of treatment	0 3 6 9 12 15 18 21 24 6 months	0 3 6 9 12 15 18 21 24 6 months	
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	0 50 100 Worst Best 50	0 25 100 Worst Best 25	
The chance of responding to the treatment	95%	65%	
Chance of responding to treatment	95%	65%	
The outcome of the treatment if you respond	6 months Treatment 18 months Response	6 months Treatment 9 months Response	
Duration of the response to treatment	0 3 6 9 12 15 18 21 24 18 months	0 3 6 9 12 15 18 21 24 9 months	
Quality of life whilst you are responding to the treatment	0 75 100 Worst Best 75	0 50 100 Worst Best 50	
Which would you choose?	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

In week 1 of the pilots (n=5 interviews), 'duration of response', was often misinterpreted as 'duration of treatment'. Interviewees also interpreted treatment and response as potentially overlapping, and therefore struggled to separate the two QoL attributes. Therefore, we made several changes including explicitly structuring the choice tasks to distinguish treatment and response phases. The changes improved understanding in week 2 (n=5 interviews) and raised some additional minor issues for consideration.

Conclusion

People with acute leukemia have a range of concerns about treatments in the context of a relapse. However, the primary issues identified in the qualitative research relate to the chance and duration of treatment success, quality of life during and after treatment, and the mode of administration. The pilot interviews were valuable in improving the wording and overall quality of the final survey.

The survey has since been fully launched in the UK, USA and EU4 (Italy, Spain, France and Germany). Recruitment is completed in the UK and ongoing in other countries.