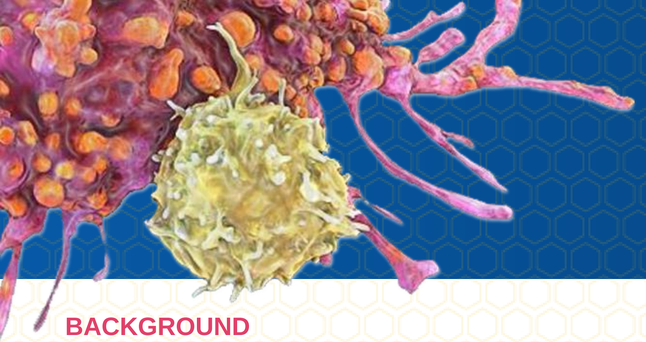

Using qualitative patient research to inform the design of a discrete choice experiment within relapsed/refractory multiple myeloma

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BACKGROUND

Relapsed/refractory multiple myeloma (RRMM) is a progressive, incurable cancer that is nonresponsive or has progressed following prior therapies. Novel therapies under investigation for treatment of RRMM, such as chimeric antigen receptor T-cell (CAR-T) therapy, differ from previously available therapies, including in their potential efficacy and treatment delivery. Limited evidence exists on patient preferences for these novel treatments.

This study aimed to identify treatment attributes most important to people with RRMM and use these findings to inform a subsequent discrete choice experiment (DCE).

METHODS

This qualitative study used individual, semi-structured interviews with people with RRMM (n=7) and healthcare professionals (HCP) (n=8) from France and the United Kingdom. Myeloma Patients Europe, a patient advocacy organisation, advised on the development of study design to ensure questions were appropriately framed in patient-centric language. Interviews were audio recorded and transcribed verbatim. Data were pooled across both countries and analysed separately for people with RRMM and HCPs. Two analysts used a thematic analysis approach to determine the frequency and prevalence of concepts within the interview transcripts. All respondents were also asked to rate the relative importance of 6 treatment attributes on a scale ranging from 'not important at all' (0) to 'extremely important' (10).

HCP interviews aimed to elicit HCP opinions and perceptions of patient preferences only. They were not used as a proxy for patient perspective.

RESULTS

Participant characteristics

See Table 1 for the characteristics of people with RRMM.

HCPs had been working within a MM speciality for an average of 17.2 years, ranging from 5 to 31 years. The HCPs reported that people with MM represented 15% to 40% of their patient caseload, with 2 HCPs reporting 90%.

Thematic analysis

Overarching themes are presented in Table 2, alongside the frequency with which those themes were coded for both people with RRMM and HCPs.

A main concern for people with RRMM was the pursuit of effective treatment (i.e., managing symptoms and achieving disease remission long-term):

> *"We want to be at peace for a few years"* (FR3, 4 lines of therapy).

Another concern was the ability to continue with their daily lives without significant treatment-related side effects. The possibility of experiencing either physical or cognitive side effects, and the severity of these, were prominent aspects in whether participants would accept an effective treatment or not:

> *"I do not want to have any side effects that could ruin my life afterwards"* (FR3, 4 lines of therapy).

These points were presented alongside: the efficiency and convenience of treatment; the need to attend a centre for treatment; and the travel distance required.

Reporting among people with RRMM was consistent. Perspectives of people with RRMM did not frequently differ depending on the number of lines of therapy experienced or by country. Some people with RRMM reported a heightened awareness of the potential side effects of their treatment as their treatment journey progressed. HCPs generally reinforced the views of people with RRMM.

Many people with RRMM were interested in the prospect of CAR-T, speaking positively about the potential for longer remission and convenience of a one time treatment:

> *"The idea sounds amazing. It is just a one off" (UK3, 5 lines of therapy).*

> *"If you have got to go through the pain barrier to get to the ultimate goal, then so be it' (UK3, 5 lines of therapy).*

However, some people with RRMM had concerns and worries around the side effects of the treatment:

> *'It is frightening because I think of the people who have a cytokine reaction.' (UK1, 4 lines of therapy).*

These concerns were mirrored by HCPs, who had additional concerns around increased costs and specialists centres for treatment. Perspectives of people with RRMM did not frequently differ depending on the number of lines of therapy experienced or by country.

Rating exercise

People with RRMM and HCPs demonstrated a close descriptive similarity in their ratings of the relative importance of treatment attributes (Table 3, where the number of ratings column represent the amount of participants who rated each attribute). Only the ratings of relevant importance assigned by people with RRMM were used to evaluate any choice attributes carried forward into the DCE, because these ratings were likely to be supported by HCPs. This ensured that any list of choice attributes for a subsequent DCE study would be determined from insights from patients.

The qualitative research was used to create a list of possible candidate treatment attributes for use in a DCE, consisting of: progression free survival; place of administration; method of administration; probability of experiencing physical or cognitive side effects; treatment duration, including the possibility of a one-time administration; dosing schedule and complexity; and distance of administration centre from a patient's usual residence. The results of this DCE will be reported separately.

CONCLUSIONS

For people with RRMM, the effectiveness of treatment to delay symptoms and extend life, the ability to prevent significant side-effects, and the convenience associated with administering treatment are suggested to be of key importance for treatment satisfaction. The prospect of CAR-T therapy was appealing for people with RRMM, but there were concerns about the severity of side effects. Understanding of the importance of these treatment attributes, particularly in the context of novel therapies, would be aided by future stated preference research. This qualitative research can subsequently be used to inform such further quantitative research.

TABLES

Table 1. People with RRMM characteristics

Variable	N=7	Proportion (%)
Country		
France	4	57.1
UK	3	42.9
Gender		
Female	1	14.3
Male	6	85.7
Age		
40 to 60	1	14.3
61 to 80	6	85.7
Years since diagnosis		
Less than 5 years	3	42.9
6 to 10 years	2	28.6
11 to 15 years	1	14.3
More than 15 years	1	14.3
Occupation		
Retired/ unemployed	4	57.1
Self-employed	1	14.3
Employed full-time	1	14.3
Employed part-time	1	14.3
Previous treatments*		
Immunomodulatory agent therapies	7	100
Proteasome inhibitor therapies	4	66.8
Anti CD38 antibody therapies	2	28.8
CAR-T therapy	1	14.3
Variable	**N=7**	**Median (range)**
Number of prior lines of treatment	7	4 (2-5)

*Participants reported multiple previous treatments

Table 2. Coding frequency themes across HCPs and people with RRMM

Theme	Patients coded (n=7)	Freq. of codes (patients)	HCPs coded (n=8)	Freq. of codes (HCP)
Symptoms	7	60	3	9
Quality-of-life impacts	7	83	5	11
CAR-T perspectives and evaluations	7	48	8	70
Effectiveness is the "main objective"	7	137	8	136
Limiting side effects, but with a willingness to "gamble" if manageable	7	89	8	109
Fewer centre visits let you "live a life"	7	69	8	40
Convenience and efficiency: less invasive, easier treatments preferred with fewer doses	7	56	8	73
Travel: "If you have to do it, then you do it…"	3	11	7	15

Abbreviations: CAR-T, chimeric antigen receptor T-cell; HCP, healthcare provider

Table 3. Mean ratings among people with RRMM and HCPs on the relative importance of treatment attributes for RRMM

Treatment attribute	People with RRMM rating (mean)	No. of ratings	HCP rating (mean)	No. of ratings
Progression-free survival	9.3	7	7.9	8
Efficacy of treatment	9.2	5	9.3	7
Method of administration	9.0	1	6.0	2
Place of administration	9.3	3	-	-
Side effects (unspecified)/tolerance	8.0	2	8.0	3
Treatment duration	7.6	7	6.9	8
Physical side effects	7.1	7	7.8	8
Cognitive side effects	6.7	7	6.9	8
Complexity	5.4	7	5.1	7
Distance of administration centre from usual residence	4.8	6	5.7	8

Abbreviations: HCP, healthcare provider; RRMM, relapsed/refractory multiple myeloma

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