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Exploring perceptions of using preference elicitation methods to inform clinical trial design in rheumatology: A qualitative study and OMERACT collaboration

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ABSTRACT

Background: Clinical trial design requires value judgements and understanding patient preferences may help inform these judgements, for example when prioritizing treatment candidates, designing complex interventions, selecting appropriate outcomes, determining clinically important thresholds, or weighting composite outcomes. Preference elicitation methods are quantitative approaches that can estimate patients' preferences to quantify the absolute or relative importance of outcomes or other attributes relevant to the decision context. We aimed to explore stakeholder perceptions of using preference elicitation methods to inform judgements when designing clinical trials in rheumatology.

Methods: We conducted 1-on-1 semi-structured interviews with patients with rheumatic diseases and rheumatology clinicians/researchers, recruited using purposive and snowball sampling. Participants were provided pre-interview materials, including a video and a document, to introduce the topic of preference elicitation methods and case examples of potential applications to clinical trials. Interviews were conducted via Zoom and were audio-recorded and transcribed. We used thematic analysis to analyze our data.

Results: We interviewed 17 patients and 9 clinicians/researchers, until data and inductive thematic saturation were achieved within each group. Themes were grouped into overall perceptions, barriers, and facilitators. Patients and clinicians/researchers generally agreed that preference elicitation studies can improve clinical trial design, but that many considerations are required around preference heterogeneity and feasibility. A key barrier identified was the additional resources and expertise required to measure and incorporate preferences effectively

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in trial design. Key facilitators included developing guidance on how to use preference elicitation to inform trial design, as well as the role of external decision-makers in developing such guidance, and the need to leverage the movement towards patient engagement in research to encourage including patient preferences when designing trials.

Conclusion: Our findings allowed us to consider the potential applications of patient preferences in trial design according to stakeholders within rheumatology who are involved in the trial process. Future research should be conducted to develop comprehensive guidance on how to meaningfully include patient preferences when designing clinical trials in rheumatology. Doing so may have important downstream effects for shared decision-making, especially given the chronic nature of rheumatic diseases.

Introduction

Decision-making in rheumatic diseases requires balancing trade-offs across a patient's lifespan. Patients with inflammatory arthritis need to weigh the risks, benefits, and costs of different treatment options. People with osteoarthritis need to weigh the risks, benefits and costs of non-pharmacologic treatment, medications, and surgery [1–3]. With rheumatoid arthritis, patients have multiple drug options available, and need to balance benefits with potential side effects, rare adverse events and costs [4]. In organ and life-threatening rheumatic diseases, such as lupus, clinicians and patients need to make difficult decisions between treatments that can have substantial adverse events and morbidity [5,6]. Understanding patient preferences for these trade-offs may be helpful in designing the clinical trials used to inform these trade-offs, for example when prioritizing treatment candidates, designing complex interventions, selecting appropriate outcomes, and determining clinically important thresholds, or when weighting composite outcomes. Preference elicitation methods, including utility-based approaches (e.g., standard gamble, time-trade-off) and stated preference methods (e.g., discrete-choice experiments) are quantitative approaches that can be used to estimate patients' preferences to quantify the absolute or relative importance of outcomes or other attributes relevant to the decision context [4,7].

In recognition of the potential role of patient preferences and preference elicitation methods in clinical trial design, we recently established a working group with Outcome Measurement in Rheumatology (OMERACT) to further efforts towards the incorporation of patient preference information within clinical trials in rheumatology. Our group has conducted a stakeholder engagement session to understand the perceived importance and role of preference elicitation methods in rheumatology trials [8], and a scoping review to map examples of how preference elicitation methods have been applied in clinical trials of any health condition [9].

The aim of this study was to understand stakeholder perceptions on the application of preference elicitation methods to clinical trial design in rheumatology. Specifically, we sought to explore the perceived value and feasibility of using preference elicitation methods to inform trial design, according to both patients and clinicians/researchers.

Methods

Study design

Our qualitative study was conducted within a pragmatic paradigm and follows the Consolidated criteria for reporting qualitative research. (Supplementary Materials). This study was approved through the University of Calgary Conjoint Research Ethics Board (REB20-0146).

Eligibility criteria and recruitment

The eligibility criteria were adults (>18 years) who spoke English and were either working within rheumatology or living with a rheumatic disease. For patient participants, we aimed to recruit those with a range of experience in clinical research and patient preference research specifically (i.e., novice to very experienced), also aiming for representation from different countries and gender. Additionally, we aimed to capture views of patients relevant to different rheumatic conditions, where the considerations about when to use preference elicitation methods may differ (e.g., treatment side effects, dosage, etc.), but did not exclude any patients based on their condition. For clinicians/researchers we aimed to recruit those of different countries and genders, and a range of experience with preference elicitation methods.

Clinicians and researchers were identified using a purposive approach through known contacts of our special interest group (SIG), with further participants identified through snowball sampling approach. Patients were also identified purposively, and by advertising to patient groups. We created a posting with Arthritis Research Canada to identify and recruit patient participants within Canada. For non-Canadian participants, we liaised with international patient partners through OMERACT. Patient participants were asked to complete a brief screening survey of demographic information that was used to inform the coding process. Specifically, we characterized participants according to their experience in rheumatology research, and their diagnosed rheumatic condition(s). For all participants, we aimed to recruit individuals both affiliated and unaffiliated with OMERACT to include different perspectives.

Semi-structured interviews

We conducted one-on-one semi-structured interviews lasting approximately 60 minutes via Zoom. Participants were informed about study goals, and of the interviewer's (MT) role within the project. Separate interview guides were developed for patients and clinicians/researchers (Supplementary materials). Draft interview guides were developed in an iterative fashion with patient partners, researchers, and rheumatologists.

We conducted four pilot interviews, two each with patients and clinicians/researchers. Pilot interview findings were not incorporated in the final analysis but used to refine the interview guides. To start the interviews, we asked questions about patient preferences in general, then questions focused specifically on the role of preference elicitation methods. Based on feedback from the pilot interviews, we decided to include four case examples to aid in understanding how preference elicitation methods can be used to inform clinical trial design (prioritizing outcomes, developing a composite outcome, defining minimally important differences, and trial prioritization), which were identified and developed from previous work[8]. These case examples were provided to participants ahead of their interviews and were reviewed with each participant during the interview. We also developed a short video to prime participants on the topic of preference elicitation methods to prepare them for an in-depth discussion on the topic (Supplementary materials). Detail on the development of the pre-interview video can be found elsewhere [8].

Analysis

We used Braun and Clarke thematic analysis, as our goal was to describe the range of perspectives on participant perceptions of using

preference elicitation methods to design clinical trials [10]. We interviewed participants until data saturation (the degree to which new data repeat what was expressed in previous data) and inductive thematic saturation (the degree to which new codes or themes are identified) were achieved [11]. All interviews were conducted by MT, a female graduate researcher, audio-recorded, transcribed verbatim, and coded for overall analysis. Raw transcripts were created using Zoom's auto-transcript feature. Raw transcripts were cleaned while listening to the interview audio, allowing MT to become immersed in the data. The data were first analyzed iteratively by MT, organized with NVivo software. Inductive coding of the data began with identification and collating of raw codes (preliminary themes using participants' exact words) derived directly from the data, to develop a single coding scheme [12]. Through collating, constant comparison, and memo-writing, raw codes were transformed into organizing themes (middle-order themes which transform basic themes into similar concepts) and eventually themes to capture global patterns in the data. A combination of latent and semantic coding was used to capture both the surface level meanings as well as the underlying assumptions of what was being expressed by participants. To ensure reliability of the coding, following the generation of initial themes, other team members with content knowledge (GH, ALS, SB, DM) and expertise in qualitative methods (GH, ALS, SB) independently reviewed two patient and two clinician/researcher transcripts. The four team members and primary coder (MT) then discussed their findings on a web call. Once satisfactory reliability was established, MT coded all transcripts, using trustworthiness strategies to establish credibility, including reflexive journaling, and discussion of themes with research team members [13]. Following analysis, we developed a narrative summary of our findings.

Results

Participant characteristics

We interviewed 26 stakeholders in total (17 patients, 9 clinicians/researchers). Patient participants had a wide range of rheumatological conditions and disease duration, as well as varying levels of experience with clinical research (Table 1). Clinicians/researchers were from a range of countries including Australia (n=1), Denmark (n=2), Canada (n=2), the UK (n=1), and USA (n=3), and were evenly split between men (n=5) and women (n=4). For clinicians/researchers, 6 participants were both clinicians and researchers, and 3 were researchers in rheumatology.

Table 1
Patient participant demographics.

Characteristic	Frequency (%) (N=17)
Country	
Australia	2 (12)
Canada	11 (64)
Netherlands	1 (6)
UK	1 (6)
USA	2 (12)
Gender identity (participants could state more than one)	
Man	2 (12)
Woman	15 (88)
Non-binary	1 (6)
Prefer not to disclose	0
Prefer to self-describe	0
Age range (years)	
18-35	2 (12)
36-49	3 (18)
50-64	8 (47)
65-79	4 (23)
80+	0
Rheumatological condition (participants could state more than one)	
Ankylosing spondylitis	1 (6)
Dermatomyositis	1 (6)
Juvenile idiopathic arthritis	1 (6)
Lupus	1 (6)
Osteoarthritis	8 (47)
Polymyalgia rheumatica	2 (12)
Psoriatic arthritis	1 (6)
Raynaud's disease	1 (6)
Rheumatoid arthritis	6 (35)
Sjogren's syndrome	2 (12)
Still's disease	1 (6)
Experience with clinical research (including clinical trials)	
Yes	14 (82)
No	3 (18)

Qualitative findings

From in-depth thematic analysis, three themes capturing global patterns with subthemes were constructed, described below. Reflective quotations can be seen below and in Table 2.

Perceptions of including patient preferences in clinical trial design

Conceptualization and perceived value of patient preferences. Most participants appeared to understand the concept of patient preferences as it was intended and framed in the pre-reading material and video. Both stakeholder groups recognized the concept of trading off between risks and benefits of treatments and how this can impact treatment selection.

"*It's about the benefits and risks, the process characteristics of the treatment or intervention and you can't always optimize everything, so you have to make a choice and trade-off between the benefits, risk and process attributes to get to make a decision on what gives you the greatest value personally.*" (Patient participant 1, UK)

While recognizing the role of patient preferences in clinical trial design, participants also alluded to the value towards informing shared decision-making between physicians and individual patients. Patient preferences were perceived to be important because patients with rheumatic diseases must balance benefits and risks of different treatments throughout their life. Many patients strongly emphasized that patients are the end-users of treatments, and therefore clinical trials should include patient preferences to measure what matters to patients and mitigate the existing power imbalance between clinicians/researchers and patients. This aligned with participant perceptions that clinical trials often do not measure what is most important to patients and fail to consider the practical concerns patients have for their day-to-day lives. A few clinicians/researchers perceived patient preferences as primarily valuable when dealing with areas of conditional (weak) evidence, and not always necessary to consider, aligning with patient perceptions of existing power imbalances in research and care.

"If there is a right thing to do, I don't feel like you need preferences." (Clinician/researcher participant 1, USA)

Part of this narrative stemmed from a perception that patient preferences are often viewed as "touchy-feely", which may lessen their perceived value to trialists.

Despite the general understanding of patient preferences as it was intended in the pre-reading material and videos, there was a tendency by some to conflate 'patient preferences' with other concepts such as patient engagement and measuring patient-reported outcomes. This contributed to some participants perceiving value of patient preferences or patient engagement in general, but not as clearly understanding how preference elicitation methods could be used to quantify patient preferences.

Where to involve patient preferences in the clinical trial process. When asked where in the clinical trial process patient preferences should be included, patient and clinician/researcher participants tended to state

Table 2
Thematic summary of findings with representative quotations.

Global Pattern	Subtheme	Quotes
Perceptions of including patient preferences in clinical trial design	***Conceptualization and perceived value of patient preferences***	*"It's about the benefits and risks, the process characteristics of the treatment or intervention and you can't always optimize everything, so you have to make a choice and trade-off between the benefits, risk and process attributes to get to make a decision on what gives you the greatest value personally." (Patient participant 1, UK)* *"The trials don't really reflect how big of a difference that these medications can make on a person in real life. […] it's what a person is able to do. Can you put on your socks? Can you tie your shoes? Can you make yourself a meal, open a can? Or things like that. Those are small things, but they're big in real world living." (Patient participant 2, Canada)* *"Well, I think, at the end of the day, clinicians are looking for medications that address the needs of the people sitting in front of them […] I think that if you have studies which show that those outcomes are being improved, then it is relevant to clinicians because I think it's important in their decision-making and it helps them to talk to patients about the medication." (Clinician/researcher participant 2, UK)* *"Patient preference, it almost sounds a little political. It's usually the clinician's preference and in theory we have shared decision-making, but in practice, I think a lot of doctors think it takes too much time to do proper shared decision-making" (Patient participant 3, Canada)* *"If there is a right thing to do, I don't feel like you need preferences." (Clinician/researcher participant 3, USA)* *"I mean preferences feel touchy feely to a lot of people, that is vague and murky, and I think doctors find them nuisance-y and I'm not sure that's honestly going to carry a whole lot of weight." (Clinician/researcher participant 3, USA)* *"I think there's a big challenge with people not really understanding what patient preferences are, how it's implemented, what you can and can't use it for. So, there's an educational awareness aspect where you need to train the people who are designing clinical trials about patient preferences." (Patient participant 1, UK)*
	Where to involve patients in the clinical trial process	*"If patients aren't in every step of that process it's going to miss so much. There's going to be a wealth of information that is left untapped. When we've got patients at the beginning where they're helping with design of clinical trials and at the end where they're helping with that research output I think it makes everything in between that much stronger." (Patient participant 4, USA)* *"Being able to set the benchmark and judging a treatment based on what patients think are the most important or whether there was a meaningful difference could possibly change the outcomes of whether a drug is successful or not in meeting the endpoints or whether it gets approved, etc., so it has a direct implication later on the track, whether that drug is available to patients or not, as an option to choose from." (Patient participant 1, UK)* *"There's such an important difference between a statistically significant difference and a clinically meaningful difference. And I think you really need to get it, what is clinically meaningful, and speaking to patients is one of the ways of doing it. So how much reduction in fatigue is actually important, how much reduction in time off work is important, how much pain is important and increasingly studies do report on clinically significant differences it, as well as statistically significant differences, but I think getting patient perspectives, this is really critical." (Clinician/researcher participant 2, UK)* *"I think it has to be a co-design, so you need you need the perspective of the consumer, you need the perspective of the clinician, and you need the perspective of the trialist, because you need to work out what's actually achievable, what's practical, but that all has to be done in the context of what's important to the people for whom the outcomes will actually be important. And that has to be early in the trial design." (Clinician/researcher participant 5, Australia)*
	Value of patient knowledge and lived experience	*"I live with rheumatoid arthritis, seven days a week, 24 hours a day. I've had this for 35 years. I know this disease, and I know my body and I know more about*

(*continued on next page*)

Table 2 (*continued*)

Global Pattern	Subtheme	Quotes
		rheumatoid arthritis than my family doctor does." (Patient participant 2, Canada)
		"A lot of these diseases are not curable […]. My disease is chronic, so it's a different mindset." (Patient participant 5, Canada)
Barriers of including patient preferences in clinical trial design	***Additional work required to incorporate patient preferences***	*"Because honestly, let's face it, sometimes it's easier for researchers to take the easy way and pick things that they want to study that are easier to get. The information that's harder to get, that patients might want them to get, that might take a little more effort." (Patient participant 2, Canada)*
		"I mean it's just hard work doing it and it takes time and people don't always have the time, the first thing, and the second thing is that they don't always have- I don't think they always have the skill and the training and the background in how to do it and they sometimes default to not doing it." (Clinician/researcher participant 2, UK)
		"Well, I think there are some things that we as patients can't weigh in on because it's just too scientific and difficult." (Patient participant 7, USA)
		"My fear is that people will overweight or underweight things that really in fact should be quite different, but they don't and that that might again require more numeracy and health literacy than maybe you're going to find." (Clinician/researcher participant 3, USA)
		"We just can't snap our fingers and say okay we're going to change the way we do research with patient partnerships and patient preferences […] I think we're going to have to be cautious and realize there's a caveat that we're going to turn off some clinical scientists and researchers when they realize the magnitude of what this means to do it properly and not do it in a tokenism way." (Patient participant 8, Canada)
		"But I think you also need to be thinking about the unintended consequences which is again involving people but then not listening to them or asking and then not including the feedback. And I think that's a very delicate challenging tightrope to walk." (Clinician/researcher participant 6, USA)
	Complexity and diversity of preferences	*"So, I think with patient preferences, the important thing is that there's an S on preference. […] The 'S' is important, when I hear things like 'the patient voice'; we don't all speak with one voice." (Patient participant 5, Canada)*
		"I guess there's a few options, so one would be to make sure you have enough patients with enough diversity, and we also have a problem in research in general that all of our samples are white most of the time. So, looking at diversity broadly diversity in sociodemographic characteristics, but also in disease characteristics." (Patient participant 9, Canada)
		"It's not just asking the people who have been selected to be in trial groups, because they're not the only ones who are involved. They're not going to be the only ones that are going to be taking whatever the treatment is that they're investigating" (Patient participant 10, Australia)
		"Maybe your preference for things would change depending on the point in time you're answering the questions." (Patient participant 11, Canada)
		"I feel like preference is often thought about in a more of discrete moment in time kind of way, but the reality is that preference kind of needs to be elicited and in some sense reassessed or resampled at discrete times that often it isn't." (Clinician/researcher participant 3, USA)
Facilitators for including patient preferences in clinical trial design	***Role of external decision-makers***	*"If there's guidance from the regulators and health authorities on using patient preferences to design clinical trials, the pharmaceutical companies will jump on that and be like okay, we have to do this now because the regulators and the health authorities are asking for this. So, it's partially getting the external decision makers on board with it. (Patient participant 1, UK)*
		"I think granting bodies, so I mean it's been such a powerful force, and I think if you submit a grant and there's no there's no consumer involvement, it just doesn't get on to the next step, so that's going to change people's behaviour." (Clinician/researcher participant 5, Australia)
	Need for guidance	*"How do we take that abundance and simplify it into something that is logical, that is a guiding tool, that is a shining path of how we should go? And how do we create all*

(*continued on next page*)

Table 2 (*continued*)

Global Pattern	Subtheme	Quotes
		these tools and modes of educational material to give to people?" (Patient participant 8, Canada) *"We need to develop new methods for figuring out how to appropriately incorporate them so and whether or not by using different outcome measures, by using subsets by directing trials to patient preferences, and so on. So, I think there's a lot to learn. But I think it's really important, and that that is the way to move forward." (Clinician/ researcher participant 1, USA)*
	Changing culture around patient engagement and advocacy	*"I think we're past the point where not having patients is acceptable." (Patient participant 9, Canada)* *"I'm old enough now to have seen the cultural change in the last couple of decades around consumer involvement and that's become really apparent, where it was kind of unique early on. And so, then I think it just becomes the norm, and […] I think that that's that helps to solve a lot of the downstream problems." (Clinician/researcher participant 5, Australia)* *"I'm thrilled that there is movement in the whole patient engagement world that from the research perspective we can now even look at that fine pointy end of patient preferences." (Patient participant 8, Canada)* *"No one followed up with me, no one told me what was accepted, so there has to be a closing the loop of giving information to the people, kudos, thanks and appreciation etc. […] if there's truly going to be patient preference and acceptance of patients' input". (Patient participant 8, Canada)* *"I really think they need the patient point-of-view and not just the educated patient but also the naive patient too […] because you get a fresher viewpoint from them." (Patient participant 3, Canada)* *"The challenge of involving patients and research is that you're going to end up getting these very activated almost professional patients where there's this line that's blurred between the research." (Clinician/researcher participant 6, USA)* *"So, I think it is a very good thing to do, I think it must be done, and has to be done, however, if it's not done correctly, with the proper caveats of inclusion respect […] if it is not going to be done properly, then it should not be done because it's not going to be respectful or it's going to be tokenism." (Patient participant 8, Canada)*

patients should be included early in the development and design stages to have the greatest impact.

"I think it has to be a co-design, so you need you need the perspective of the [patient], you need the perspective of the clinician, and you need the perspective of the trialist, because you need to work out what's actually achievable, what's practical, but that all has to be done in the context of what's important to the people for whom the outcomes will actually be important. And that has to be early in the trial design." (Clinician/ researcher participant 2, Australia)

When commenting on the case examples provided, participants recognized the potential value in using preference elicitation for specific design aspects of trials, including prioritizing outcomes, weighting components of composite outcomes, determining meaningful endpoints for outcomes, and powering clinical trials. Most participants agreed that when conducting these preference studies, involving patients from the start is also critical. Early engagement of patients aligned with the concept of co-designing trials with patients.

Value of patient knowledge and lived experience. Many patient participants emphasized the importance of their lived experience of having a rheumatic condition and knowledge of their own well-being, which brings unique insights into what matters most to them.

"I live with rheumatoid arthritis, seven days a week, 24 hours a day. I've had this for 35 years. I know this disease, and I know my body and I know more about rheumatoid arthritis than my family doctor does." (Patient participant 2, Canada)

Patients also stressed that the chronic nature of rheumatic diseases contributes to different considerations and experiences which may not be shared by patients with non-chronic illnesses. Some patient participants mentioned that patients would likely have different preferences, based on where they were in their disease journey.

Barriers of including patient preferences in clinical trial design

Additional work required to incorporate patient preferences. Patient and clinician/researcher participants highlighted that including patient preferences and using preference elicitation methods to design trials requires more resources, time, and effort. Further, some noted that not all researchers will have the necessary skill set to meaningfully incorporate patient preferences in trials, as preference elicitation exercises need to be carefully developed to ensure appropriate understanding from patients and avoid potential biases.

"I mean it's just hard work doing it and it takes time and people don't always have the time, the first thing, and the second thing is that they don't always have- I don't think they always have the skill and the training and the background in how to do it and they sometimes default to not doing it." (Clinician/researcher participant 3, UK)

A fear was raised that these biases (deviations in the findings of a preference elicitation study from the 'true' preferences) may then lead to inappropriate decisions or have a potential for negative unintended consequences if preferences are measured but then ignored.

Complexity and diversity of preferences. Participants noted the complex and evolving nature of patient preferences, with many patients emphasizing the diversity in patients and the subsequent diversity in

preferences, which should be considered when eliciting patient preferences.

"So, I think with patient preferences, the important thing is that there's an S on preference. […] The 'S' is important, when I hear things like 'the patient voice'; we don't all speak with one voice." (Patient participant 3, Canada)

Understanding the diversity of the patient population led to participants feeling concerned about how to identify an appropriately representative sample of patients of the broader patient population, not just those involved or participating in clinical trials.

Facilitators for including patient preferences in clinical trial design

Role of external decision-makers. Participants spoke to the role of other necessary stakeholders for the inclusion of patient preferences in trials, including funding agencies/granting bodies, regulatory agencies, and journals.

"I think that anything that the FDA does not recognize, and for which there really isn't any meaningful opportunity for getting a label claim, I think is going to diminish the importance of what patients think." (Clinician/researcher participant 1, USA)

Participants felt that having these external decision-makers engaged would be important for the behavior changes required for patient preferences to be considered more systematically in clinical trial design.

Need for guidance. Clinicians/researchers identified that even when researchers are interested in incorporating patient preferences in clinical trials they may not know where to begin. Participants also identified a lack of awareness on the potential impact of incorporating patient preferences in trial design and guidance on how to operationalize this.

"I think a take-home message could be that none of us really know how to involve patients […]. So, this might be the real shortcut if this group could develop the instruments. (Clinician/researcher participant 4, Denmark)

To mitigate this, participants suggested developing comprehensive tools and guidance on best practices to elicit preferences, tailored to researchers conducting trials.

Changing culture around patient engagement and advocacy. Participants spoke to a cultural shift in research, including the movement towards patient advocacy and engaging patients in clinical research, and how this overall cultural shift could help support the inclusion of patient preferences in trials.

"I'm thrilled that there is movement in the whole patient engagement world that from the research perspective we can now even look at that fine pointy end of patient preferences." (Patient participant 4, Canada)

Robust patient engagement throughout the trial design was viewed as a necessary pre-requisite for any incorporation of patient preferences. Many patients emphasized the need for meaningful engagement, with proper processes in place to ensure a respectful partnership where patients feel comfortable, citing previous experiences where they were not kept informed about their input in the process. Participants also noted the need to involve both patients with substantial research experience as well as patients who are new to research.

Thematic synthesis

Comparison between patients and clinicians/researchers allowed us to identify similarities and differences between stakeholder groups. Both groups agreed that patient preferences are important to consider and saw perceived value of preference elicitation methods in the design of clinical trials. Patient participants viewed the value as self-evident because they are the end-users of treatments and have experiential knowledge of living with a rheumatic disease. Patients also viewed including preferences in trials as important for reducing existing power imbalances and improving their day-to-day lives. Clinician/researcher participants, in their roles as prescribers, tended to view the importance of including patient preferences through the lens of shared decision-making with their patients. There was perceived value from both patients and clinicians/researchers for co-designing trials with patients and designing trials to measure what is most important to patients, although the direct value of preference elicitation methods to inform clinical trial design was less apparent, particularly to clinicians/researchers. This stemmed from the uncertain impact of the knowledge provided, methodological challenges in ensuring unbiased and representative results, and a lack of resources, expertise, or incentives to formally conduct preference elicitation studies. Both stakeholder groups agreed that the changing culture of research is a facilitator that could enable the inclusion of patient preferences in trials (via preference elicitation), and that system-level guidance and direction could help accomplish this.

Discussion

In this study we found that patients and clinicians/researchers generally agreed that preference elicitation studies can improve clinical trial design, and that this may ultimately lead to more patient-relevant information. Patients saw the value as self-evident, as the end-users of treatments being studied, whereas clinicians/researchers tended to focus on the application to shared decision-making and expressed concerns around feasibility, and methodological challenges. Further exploration may be needed to mitigate this hesitancy and identify ways to limit methodological challenges of preference elicitation.

Participants generally understood the concept of patient preferences and preference elicitation methods after being provided pre-interview education materials. However, the value of preference elicitation methods was still conflated at times with the value of broader patient engagement efforts by both patients and clinicians/researchers. This lack of clarity is reflected in the literature and cited as a potential barrier to conducting preference elicitation research [14–16]. The perception of patient preferences as subjective or abstract may contribute to this barrier. To further the use of preference elicitation methods in clinical trial design, there will be a need to clearly explain and distinguish these methods from broader concepts of patient engagement, Ultimately, though all these concepts are connected and interdependent, and there is a need to embed efforts with preference elicitation methods in an environment of robust patient engagement.

In rheumatic diseases, incorporating patient preferences in outcome measure development and clinical trials is shown to have promise. Leveraging patient preferences has allowed for researchers in rheumatology to identify a range of applications, including the development of ways to rank adverse events in rheumatoid arthritis [17], to considering which treatments are considered acceptable to patients when conducting clinical trials [18,19]. However, these examples tend to be done outside of clinical trials. We have identified a need to engage with patients as well as external stakeholders (funders, regulators, etc.) to conduct preference elicitation early on. Doing so can allow for an impact at the trial design stage, which will influence shared decision-making downstream for patients with rheumatic diseases [20].

Our findings align not only with those from rheumatology-focused research, but also with other groups that have identified barriers and facilitators for integrating patient preferences [14,21–23]. The Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle (PREFER) project has conducted studies in a range of conditions, investigating the potential role of preference elicitation based methods in health technology assessment (HTA) decisions [21,22,14,15]. A recent PREFER study conducted stakeholder interviews with regulators, industry, HTA bodies to identify decision points in the medical product lifecycle where patient preference information could be included [15]. Our study builds on this, focusing and expanding on the role of preference elicitation methods specifically within the design of clinical trials in the field of rheumatology. Ultimately, many agree that the use of preference elicitation methods can add value to clinical trial design, but

there is a need for engaging external decision-makers, such as those working in regulatory agencies or industry, and for adequate guidance to integrate patient preferences into trial design.

Strengths and limitations

A strength of this research is the contribution of patient partners, rheumatologists, and researchers to the overall study design, which was enabled by embedding the work within OMERACT. Following guidance on thematic analysis, the researcher most immersed in the interview content led the analysis, and code development was discussed and verified with others [10]. There are limitations. North American participants were overrepresented in comparison to European and Australian participants. Further, we were unable to recruit participants from other continents, which should be considered when applying our findings in other contexts. Interview participants may have been better informed and more interested than others in patient preferences and clinical research. We aimed to recruit a range of patient participants with different levels of experience in research to mitigate this, but patients experienced in research were more likely to be affiliated with patient organizations and thus more likely to have seen the opportunity to participate.

Implications for future research

This study allowed us to begin understanding the required elements for including patient preferences in trial design within rheumatology. First, many participants spoke to the need for comprehensive guidance. Though there may be interest in incorporating preferences, there is a lack of guidance on how to operationalize preferences (through preference elicitation methods) in a trial design setting. Comprehensive guidance on best practices should be developed and tailored to researchers conducting trials. This could help facilitate the inclusion of preferences in clinical trial design as measuring preferences can be complex, particularly for rheumatic diseases which are chronic, as preferences can change over time. Not all researchers are experienced with the use of preference elicitation methods, but that should not be a deterrent, and seminal guidance could help researchers begin that process. Participants emphasized that the process requires meaningful patient engagement to be successful. Thus, any efforts to include patient preferences in clinical trial design must involve patients in the process.

Participants also recognized the need to account for diversity of patients when including preferences in trial design. Identifying the right sample of patients in trials is paramount to ensuring preferences can accurately represent the broader patient population. However, conducting large-scale preference studies to include many different patients could help provide key insights on patient preferences, which could then be leveraged to inform trial design. While some participants considered the diversity of preferences as a barrier, preference elicitation methods could allow trialists to measure this diversity and understand whether it would impact the design of the trial. Some clinicians/researchers spoke to the potential challenge of conceptualizing preferences and felt concerned that patients would have difficulty understanding these concepts. In our study, however, we found that providing everyone with the same educational material, including case examples and a short video, facilitated a common level of understanding, which was also credited by participants. This is supported in the literature, with others suggesting that a similar approach could be used when including preferences in clinical trials [16].

Conclusion

Our findings suggest that further research is required to understand where and when including patient preferences in rheumatology clinical trials is of the most value, and to develop guidance for researchers who want to incorporate preferences via preference elicitation methods in clinical trial design. There may also be a need to increase awareness and understanding on preference elicitation methods as an appropriate research strategy. This work will continue through our OMERACT SIG. It will be important to foster strong patient partnerships for future research, and to understand the role of preference data for designing clinical trials to potentially benefit treatment decision-making downstream for people living with rheumatic diseases.

Declarations

Specific author contributions

Planning and conducting the study (all authors), data collection (MT), data interpretation (MT, DM, ALS, SB, GH), drafting the manuscript (all authors).

Guarantor of the article

Dr. Glen Hazlewood

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Ethics approval

This study received institutional ethics approval.

Informed consent

All study participants provided informed consent for participation.

Declaration of Competing Interest

None.

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Supplementary materials

Supplementary material associated with this article can be found, in the online version, at doi:10.1016/j.semarthrit.2022.152112.

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