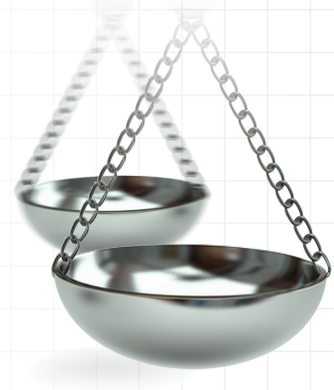
Methodology



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Treatment goals for rheumatoid arthritis: patient engagement and goal collection

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Aim: We developed the Patient-Engaged Health Technology Assessment strategy for survey-based goal collection from patients to yield patient-important outcomes suitable for use in multi-criteria decision analysis. **Methods:** Rheumatoid arthritis patients were recruited from online patient networks for proof-of-concept testing of goal collection and prioritization using a survey. A Project Steering Committee and Expert Panel rated the feasibility of scaling to larger samples. **Results:** Survey respondents (n = 47) completed the goal collection exercise. Finding effective treatments was rated by respondents as the most important goal, and reducing stiffness was rated as the least important. Feedback from our steering committee and expert panel support the approach's feasibility for goal identification and ranking. **Conclusion:** Goals relevant for treatment evaluation can be identified and rated for importance by patients to permit wide input from patients with lived experience of disease.

Plain language summary: Methods for collecting patient input on valuation of healthcare interventions are in wide use, but the extent to which existing methods capture the range of outcomes important to patients is not known. There is not yet a standard approach to identify and quantify patient-important outcomes for use in deliberative decision-making processes. We worked with a steering committee and surveyed patients with rheumatoid arthritis to test a new method for engaging patients in healthcare valuation using patient goals. We found this method to be feasible for wider use. Deliberative methods of valuation can include outcomes based on goals collected directly from patients. Patient input through goal framing provides a way for patients to be actively involved in valuation methods.

Tweetable abstract: Patient goals can be collected and rated by online patient panels for inclusion in multicriteria decision analyses for health technology assessments.

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Commonly used approaches to assessing the value of health technologies, such as the cost per quality-adjusted life year (QALY), often fail to capture a comprehensive set of clinical and economic outcomes that matter to patients and their caregivers [1,2]. However, incorporating patient-important outcomes into value assessments in healthcare is a core pillar of patient-centered care [3,4]. The Professional Society for Health Economics and Outcomes Research (ISPOR) has led the development of the 'value flower', a framework that suggests additional elements of value important to patients that are not traditionally captured in value assessments, and subsequent research has explored specific elements identified in this framework [5,6]. Though several efforts to define these patient-important

outcomes are underway [7–9], we lack a standard approach to identify and quantify patient-important outcomes in a way that would make the measures appropriate for use in deliberative decision-making processes such as through multi-criteria decision analysis (MCDA) [10,11].

In this paper, we describe a method to fill this gap, the Patient-Engaged Health Technology Assessment Strategy. We report the results of a proof-of-concept application of this method to elicit treatment goals for people with rheumatoid arthritis (RA): a survey instrument for ascertaining and prioritizing goals for people living with RA, the output of which could be suitable for deliberative assessment of both individual treatments and sequences of treatments.

The Patient-Engaged Health Technology Assessment Strategy [12] draws on methods of goal attainment scaling (GAS), a method validated across multiple disease states, to identify and quantify patient-important outcomes as one way to capture patient perspectives for use in subsequent valuation methods [13–15]. GAS was developed to guide clinical decision-making through discussion and completion of treatment goals by the patient along with a clinician [15]. Using a survey method to collect goals directly from patients can enable scaling within a patient population, yielding inputs for MCDA [16] or other deliberative methods. Goals can be efficiently 'crowd sourced' from a larger patient population in this way, with the option for open-ended contribution of goals in addition to importance rating of a defined set of goals. The patient goals can be collected by patient representatives from different subgroups within a given patient community and incorporated with the help of the patient representatives in the next steps of deliberation. By collecting patient-generated goals and understanding their relative importance, the inputs for MCDA (i.e., criteria and weights) can be informed directly by input from a large number of patients, increasing the representativeness of those inputs.

Obtaining patient input is particularly relevant for a disease state like RA where there is discrepancy between the outcomes included in clinical trials and value assessments, and the outcomes that patients most care about [17,18]. While some patient-important treatment goals are commonly captured (e.g., slowed or stopped disease progression), others are not well understood. For example, routine blood monitoring is widely used for assessment of disease activity in RA, but many patients report symptomology, such as fatigue, and functional limits that are not captured in lab results – so called 'subclinical' manifestations of disease. To date, the best way to understand and evaluate what is most important to patients is to ask patients directly.

RA was chosen as a disease state for proof-of-concept testing of the proposed strategy, as patient-important outcomes have a relatively long history in RA research and care, and patient-reported outcomes (PROs) are increasingly becoming a part of core outcome sets recommended for both clinical trials and routine documentation [19]. The European League Against Rheumatism (EULAR) and the Outcome Measures in Rheumatology (OMERACT) group are driving adoption of patient-important outcomes in clinical trials, by developing and improving consensus for research priorities and outcome measures with direct participation from patient stakeholders [20–22], and through direct research support via funding. PROMIS® short forms, which are sets of PRO items that capture important health domains, have been found to capture meaningful improvement or worsening in key symptom features of RA [23], supporting the usefulness of direct patient report in RA. Engaging patients in their care by asking them to rate symptoms using PRO tools has also been found to improve the patient experience, including confidence in treatment decisions [24]. Additionally, tools for collecting preferences of RA patients such as the McMaster Toronto Arthritis patient preference questionnaire (MACTAR) have been used to assess improvement in functional status in RA clinical trials [25,26] and the International Classification of Functioning, Disability and Health (ICF) has developed a core set of concepts and problems in people with RA [27]. This research builds on those existing efforts by operationalizing these concepts into patient-centered goals that can be used in a standardized way across patient populations.

In the current research, we build on what is known about disease burden and treatment attributes that are important to people with RA by developing a set of goals from which people can select the most important goals to them in managing their disease condition, eliciting the relative importance of each goal based on direct input via a survey of people with RA, and assessing the feasibility of this approach by having our expert panel review these findings. Using this standardized approach and list of goals, a set of important patient goals for treatment can be developed and potentially incorporated into value assessment.

Methods

This work involved four main elements: collection of input from the project Steering Committee (SC) to address strengths and weaknesses of a plan for patient-engagement in GAS and use of results in MCDA; development of

a survey instrument and cognitive interviews with patients; collection of survey data from pilot respondents, with survey item revision based on first round of data collection and fielding a second round survey with additional respondents; and review of the data obtained to determine feasibility of use in health technology assessment (HTA). The SC was again consulted following completion of data collection to aid with practical steps needed to scale the method up for wider use.

Collection of input from the Steering Committee

We sought to incorporate input from multiple stakeholders throughout this project to ensure the feasibility and scientific rigor of the approach and its relevance to informing HTA. A project Steering Committee (SC) was formed with patients, clinicians, and researchers and practitioners with expertise in GAS, MCDA, and HTA broadly. Two meetings were held with the SC to discuss the approach and provide input on the development of the Patient-Engaged Health Technology Assessment Strategy. The project plan was refined based on collective input. Members of the SC are listed in Appendix 1.

Three members of the SC – two clinician researchers whose work focuses on measurement of outcomes important to RA patients (SJB, COB) and one person with RA with expertise in research engagement by the RA community (SS) – continued to engage as project advisors during the subsequent phases of the project: development of the survey and interpretation of results.

Development of survey instrument & cognitive interviews

To identify high-level potential goals and specific domains to include as goals in the patient survey, we conducted a targeted search of the peer-reviewed literature via MedLine/PubMed. We first searched for review articles on RA from 2010–2018 that had the keyword 'rheumatoid arthritis' (MeSH term) related to patient outcomes, goals, or preferences. We then searched all published articles from 2018 to 2020 (not limited to review articles) again identifying articles on RA that had keyword 'rheumatoid arthritis' related to patient outcomes, goals, or preferences.

The research team reviewed these articles and extracted the domains or particular outcomes that the authors identified as important to people with RA. We maintained a list of distinct concepts, combining potential goals that were conceptually similar. We then used an inductive and iterative approach to group each outcome or topic into broad domains, such as 'Activity Limitations', 'Sleep', and 'Overall Health'. We chose to use this model to keep patients at the center, as well as to test whether this method would work in the future for other disease area without as well-defined of a set of outcomes as RA. We also consulted the Innovation and Value Initiative (IVI) value model for RA [28]. The IVI value model worked with patient groups to identify patient views on health benefits as well as risks and costs associated with disease-modifying anti-rheumatic drugs (DMARDs) to address the value of different DMARD sequences.

After sorting goal topics into higher-level domains, we created one phrase that described each topic. Each phrase was drafted to complete the stem, "*I want treatments that will support my goals for my RA. One of these goals is to. . .* " We attempted to keep the phrasing and vocabulary simple and direct. We solicited input from a patient consultant advisor, who recommended how to combine symptoms and concepts to condense the list and allow us to focus on the goals that have the most impact and broadest relevance to the RA population. This resulted in 26 goal statements across 4 domains that we moved forward to cognitive testing. In addition to the pre-defined survey items, we decided to include an option for 'write in' responses for each domain in the survey.

We reviewed the draft survey first with the patient advisor and then with three people with RA through formal cognitive interviews in which they completed the survey and explained their reasoning with the interviewer. The patient advocate SC member identified respondents for cognitive interviews. A trained interviewer from the RAND Corporation conducted the interviews by phone using a structured interview protocol. Respondents were emailed a draft survey protocol in advance of the interview. Respondents were first asked a set of introductory questions, including what the interviewee understood 'goals' to mean in the context of RA, how clear the survey instructions were, and what topics they assumed the survey would cover. The interviewer then asked about each candidate goal and the respondent rated each on a 4-point scale of Not Important, Somewhat Important, Important, and Very Important and gave feedback on the wording. Finally, the interviewer asked about overall impressions of the survey, the appropriateness of the rating scale, and whether 'goals for living with RA' accurately described the items. Respondents were compensated $125 for their participation. The interviewer took detailed notes in each interview.

The final set of patient goals included in the survey are shown in the first column of Table 2. All goal statements were phrased to complete the stem "*My goals for living with RA are to. . .* ". Response options were on a 4-point

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Table 1. Respondent characteristics.

	Round 1 (n = 20)	Round 2 (n = 26)[†]	Combined (n = 46)
Age	55.0 (range 28–72, sd 12.0)	50.0 (range 28–69, sd 13.3)	52.2 (range 28–72, sd 12.9)
Women	20 (100%)	23 (88%)	43 (93%)
Ethnicity			
Hispanic or Latino	3 (15%)	0 (0%)	3 (7%)
Race			
White	18 (90%)	23 (88%)	41 (87%)
Black or African–American	1 (5%)	4 (15%)	5 (11%)
Asian	0 (0%)	0 (0%)	0 (0%)
Native Hawaiian or Other Pacific Islander	0 (0%)	0 (0%)	0 (0%)
American–Indian or Alaska Native	0 (0%)	0 (0%)	0 (0%)
Other	1 (5%)	0 (0%)	1 (2%)
Highest grade or level of school			
Did not graduate high school	0 (0%)	0 (0%)	0 (0%)
High school graduate or GED	1 (5%)	3 (12%)	4 (9%)
Some college or 2-year degree	6 (30%)	3 (12%)	9 (20%)
4-year college graduate	6 (30%)	10 (38%)	16 (35%)
More than 4-year college degree	7 (35%)	10 (38%)	17 (37%)
Income (round 2 only)			
Less than $30,000	–	2 (8%)	–
Between $30,000 and $50,000	–	5 (19%)	–
Between $50,001 and $75,000	–	6 (23%)	–
Between $75,001 and $100,000		7 (27%)	
Over $100,000	–	6 (23%)	–
Insurance coverage – check all that apply (round 2 only)			
Employer		18 (69%)	–
Exchange		3 (12%)	–
Medicare		7 (27%)	–
Medicaid	–	4 (15%)	–
Other	–	1 (4%)	–
Years with RA diagnosis	17.2 (range: 0–54; SD 15.7)	13.8 (range: 0–39; SD 11.4)	15.3 (range: 0–54; SD 13.4)
Severity of RA now			
Mild	5 (25%)	6 (23%)	11 (24%)
Moderate	6 (30%)	16 (62%)	22 (48%)
Severe	9 (45%)	4 (15%)	13 (28%)
How do you feel your arthritis is today?			
Excellent	1 (5%)	1 (4%)	2 (4%)
Very good	3 (15%)	5 (19%)	8 (17%)
Good	3 (15%)	12 (46%)	15 (33%)
Fair	12 (60%)	6 (23%)	18 (39%)
Poor	1 (5%)	2 (8%)	3 (7%)
How much pain did you have due to your arthritis in the last week?			
No pain (1)	0 (0%)	2 (8%)	2 (4%)
(2)	1 (5%)	4 (15%)	5 (11%)
(3)	2 (10%)	1 (4%)	3 (7%)
(4)	3 (15%)	7 (27%)	10 (22%)
(5)	2 (10%)	2 (8%)	4 (9%)
(6)	3 (15%)	1 (4%)	4 (9%)
(7)	2 (10%)	5 (19%)	7 (15%)
(8)	6 (30%)	0 (0%)	6 (13%)
(9)	0 (0%)	2 (8%)	2 (4%)
Worst pain (10)	1 (5%)	2 (8%)	3 (7%)

[†]Note: 27 respondents completed the rating tasks in Round 2, but only 26 completed demographic questions.
RA: Rheumatoid arthritis; SD: Standard deviation.

Table 2. Ratings for each goal across both rounds of the survey.

Goals[†]	Not important	Somewhat important	Important	Very important
Symptom management				
Improve the quality of my life with RA	0% (0)	0% (0)	23% (11)	77% (36)
Manage my RA pain	0% (0)	2% (1)	11% (5)	87% (41)
Reduce my tiredness or fatigue	4% (2)	2% (1)	30% (14)	64% (30)
Reduce my morning stiffness	6% (3)	13% (6)	55% (26)	26% (12)
Improve my ability to do things like dress, eat, or walk	2% (1)	13% (6)	34% (16)	51% (24)
Improve the quality of my sleep	2% (1)	28% (13)	19% (9)	51% (24)
Reduce how my RA pain interferes with my life	0% (0)	9% (4)	17% (8)	74% (35)
Limit surprises in how my RA symptoms affect me	2% (1)	13% (6)	34% (16)	51% (24)
Life impact				
Do things that I enjoy doing	0% (0)	0% (0)	34% (16)	66% (31)
Reduce the ways that RA interferes with my ability to work or take care of my life	0% (0)	6% (3)	26% (12)	68% (32)
Reduce the impact that RA symptoms have on my life	0% (0)	2% (1)	32% (15)	66% (31)
Be the kind of friend or loved one that I want to be despite my RA symptoms	0% (0)	11% (5)	32% (15)	57% (27)
Reduce how much RA interferes with social time or my ability to connect with family and friends	2% (1)	13% (6)	47% (22)	38% (18)
Reduce the ways in which RA interferes with my life	0% (0)	2% (1)	21% (10)	77% (36)
Be independent in my daily functioning	0% (0)	4% (1)	15% (4)	81% (22)
Managing my RA				
Feel like I can manage my RA	0% (0)	2% (1)	26% (12)	72% (34)
Reduce my worry or anxiety about my RA	4% (2)	15% (7)	43% (20)	38% (18)
Reduce my sadness about my RA	11% (5)	26% (12)	26% (12)	38% (18)
Feel more accepting of my RA	11% (3)	30% (8)	26% (7)	33% (9)
Treatment features				
Understand my RA treatment options	0% (0)	4% (2)	21% (10)	74% (35)
Have the information I need to make treatment decisions	0% (0)	0% (0)	19% (9)	81% (38)
Reduce the impact that RA treatment has on my life	0% (0)	4% (2)	26% (12)	70% (33)
Know what to expect with my RA treatment	0% (0)	2% (1)	23% (11)	74% (35)
Find treatments that are effective	0% (0)	0% (0)	6% (3)	94% (44)
Find treatments that don't cost too much	2% (1)	26% (12)	23% (11)	49% (23)
Find treatments that have very few side effects	0% (0)	11% (5)	30% (14)	60% (28)
Find treatments that have manageable side effects	0% (0)	4% (2)	51% (24)	45% (21)
Reduce the time I spend getting treatment for RA	9% (4)	30% (14)	34% (16)	28% (13)
Reduce the burden my treatment puts on my family	15% (7)	15% (7)	34% (16)	36% (17)
Find treatments that allow me to keep working	11% (3)	15% (4)	15% (4)	59% (16)
Limit the financial impacts that treatments have on me and my family	7% (2)	15% (4)	26% (7)	52% (14)

[†]Prompt before each goal was "*My goals for living with RA are to…*"
Underlined text indicates change to item wording or new item for round 2.
RA: Rheumatoid arthritis.

scale of: 'Not Important', 'Somewhat Important', 'Important', and 'Very Important'. Goal statements were listed within four domains: 'Symptom Management', 'Life Impact', 'Managing My RA', and 'Treatment Features'. A final section for 'Other Goals for Living with RA' allowed respondents to write in a response. The write-in option was included to capture potential goals that were not included in the pre-defined list. The survey was fielded in two rounds, described in step 3. We analyzed the write-in responses from the first round of the survey. To conduct the qualitative analysis, a member of the research team read the free-text goals and open-ended comments and inductively developed a set of preliminary potential goals. Others from the research team reviewed the comments, iterated on these potential goals, and developed a final set of potential goals. Based on write-in responses from round

Table 3. Write-in responses and resulting changes between round 1 and round 2.

Example write-in responses	New or modified goal text [survey domain]
The isolation of a RA patient as I find friends don't 'get it' or else disappear. I know this leads to anxiety, loneliness, and sadness.	reduce how much RA interferes with social time or my ability to connect with family and friends [life impact]
Travelling Physical independence	be independent in my daily functioning [life impact]
Work on my emotions	feel more accepting of my RA [managing my RA]
Disability assistance The one thing that I have trouble with is when the pain occurs during an activity. The pain is severe enough to make stop what I am doing…except when at work. Working… with the pain is very difficult.	find treatments that allow me to keep working [treatment features]
There must be a way that RA patients can participate in the copay programs. Most of us cannot because we have federal benefits	limit the financial impacts that treatments have on me and my family [treatment features]

Underlined text indicates change to item wording or new item addition.
RA: Rheumatoid arthritis.

1, we edited the phrasing of one goal to make it more inclusive and added four new goals within the Treatment Features domain (Table 3). Write-in responses in the second round of the survey did not contain any new goals.

In the flow of the survey, after rating goals, respondents were asked about their sociodemographic characteristics and health status: age, gender, ethnicity, race, state of residence, educational attainment, age when diagnosed with RA, self-rated severity of RA now ("*How would you describe the severity of your RA now?*", response options: Mild/Moderate/Severe), self-rated RA status ("*Considering all the ways your arthritis has affected you, how do you feel your arthritis is today?*", response options: Excellent/Very Good/Good/Fair/Poor), and pain in last week ("*How much pain did you have due to your arthritis in the last week?*", 10-point scale with 0 being No Pain and 10 being Worst Pain). In the second round of the survey, to better assess the representativeness of the sample, we added items on total household income and current health insurance coverage. The survey is available by request from the corresponding author.

Collection of survey data from pilot respondents

We partnered with AiArthritis (The International Foundation for Autoimmune and Autoinflammatory Arthritis, an international non-profit organization that advocates for patient participation in education, advocacy, and research) to disseminate a call for survey participants. The organization posted a solicitation for survey respondents on their websites and conducted additional recruitment via individualized email outreach. Additional participant recruitment was conducted by the Arthritis Foundation (a nonprofit in the USA that works to improve the lives of people with arthritis) during round 2 of the survey; the Arthritis Foundation shared information about the study with their members. The advertisement that was posted and circulated via email briefly described the project, time commitment (15–20 minutes), and incentive (a $75 Amazon credit). Interested individuals were directed to contact the RAND Corporation via an email alias. RAND staff followed-up with screening questions on age, race, ethnicity, and education. Study staff reviewed the demographics of potential respondents and invited a purposive sample to participate in each round of the interview. Invited individuals were selected to maximize heterogeneity of respondents with regard to gender, race, ethnicity, and educational attainment.

Data collection was planned in two rounds, with the first round intended to inform final items and response choices to be used in the second round, and the second round of data collection providing additional information on feasibility. After reviewing first-round responses, the research team made slight modifications to the survey instrument based on the write-in responses (e.g., we modified the goal "*reduce how much RA interferes with social time with family and friends*" to "*reduce how much RA interferes with social time or my ability to connect with family and friends*" to reflect that people thought of all types of meaningful connections with family and friends, not just the time spent socializing). Survey results were totaled and summarized quantitatively using several different approaches, including the percentage of respondents who selected each rating for each goal. To calculate average rating, we assigned a value of 0 to 'Not important', 1 to 'Somewhat important', 2, to 'Important', and 3 to 'Very important'.

Steering Committee assessment of feasibility

Upon completion of the data collection in step 3, a second convening was held with the SC members to review the results from pilot testing. The SC members subsequently completed a survey to rate the feasibility of the proposed methods and then asked to review feasibility again following results from pilot testing. (See Online Content for feasibility items.)

We also convened a meeting of an expert panel to review the results of the survey and assess the feasibility of this method for collecting goals and using the results in HTA. Five of the nine members of this panel were on the SC that was convened earlier; the four new members consisted of two more patient representatives and two methodologists with expertise in value assessment. We recorded this meeting, took detailed notes, and qualitatively analyzed the comments and feedback from panel members to identify opinions and major concerns regarding the feasibility and limitations of this approach and its usefulness for HTA.

Results

Collection of input from the Steering Committee

Nine of the 11 members of the SC rated agreement with questions regarding the feasibility of the method before we began the pilot study. All members rated agreement at 'somewhat agree' or completely agree for all items, with four exceptions. Two members rated the following item as 'somewhat disagree': "*Goals collected from patients using an online format can be aggregated or combined into criteria useful for MCDA*". One member rated the following item as 'somewhat disagree': "*Existing RA networks could support recruitment of patients for goal collection*" and one member rated the following item as 'somewhat disagree': "*Patients can be trained to help translate patient goals into criteria for MCDA*". The SC generally was supportive of the method, with some noting that there would be challenges with selecting a diverse group of patients, training them, and supporting them throughout this method, especially given the desire to incorporate perspectives of heterogenous patient populations with different levels of health literacy.

Development of survey instrument & cognitive interviews

All respondents in the cognitive interviews felt that the survey instructions were clear, the survey was easy to complete, and 'goals' was the correct term to describe the rating tasks. Two interviewees felt that this scale was a good way to assess the importance of these goals, while one thought that most people were likely to select '3 – Most Important' for almost all goals. Based on feedback from the cognitive interview participants we made several changes to the goal statements included in the survey (changes described in Table 2).

Several responses related to aspects of the patient experience beyond clinical goals that were the focus of this work were excluded from consideration in item revision, for example "*Teaching others about RA,*" and "*Literature available to help my family understand*". The remaining clinical goal-focused write-in responses were reviewed and considered for inclusion.

Collection of survey data from pilot respondents

The first round of the survey was conducted in December 2020. Twenty of 22 respondents contacted completed the survey. The second round of the survey was then completed by 27 individuals in February and March 2021. Because of the slight differences in the survey instrument and available demographic data between the two rounds, we discuss results for all respondents when possible, noting differences between the two rounds of data collection when relevant.

Respondent characteristics

Of the 47 respondents, majority were women (93%), White (87%), and college-educated (72%; see Table 1). Respondents were most in their 40s or 50s (range: 28–72 years). In the second round of the survey, two respondents (8%) reported incomes of less than $30,000, while the remainder of respondents were spread evenly across the other income categories. Source of health insurance was a mix of employer-based coverage, Medicare, and Medicaid. Respondents reported a median time of about 15 years since diagnosis. Respondents reported fairly high levels of disease activity and symptom burden: about three-quarters rated the current severity of their RA as moderate or severe; when asked "*How you feel your arthritis is today?*", nearly half said they feel their arthritis is fair or poor; and about 40 percent reported a pain rating of seven or higher for pain due to arthritis in the past week.

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Table 4. Percentage of goals rated "very important" by sociodemographic characteristics.

Variable	Average % 'very important'	Minimum	Maximum
Age range, years			
18–34 (n = 6)	58%	22%	100%
35–44 (n = 10)	67%	29%	94%
45–54 (n = 7)	53%	29%	67%
55–64 (n = 13)	58%	19%	96%
65+ (n = 10)	56%	16%	84%
Education			
High school graduate or GED (n = 4)	74%	68%	84%
Some college or 2-year degree (n = 9)	58%	19%	96%
4-year college graduate (n = 16)	66%	29%	100%
More than 4-year college degree (n = 17)	49%	16%	87%
Disease severity			
Mild (n = 11)	53%	23%	90%
Moderate (n = 22)	58%	16%	87%
Severe (n = 13)	65%	19%	100%
Self-reported pain			
Lower half of scale [1–5] (n = 24)	54%	16%	100%
Upper half of scale [6–10] (n = 22)	64%	19%	96%

Importance ratings by item

Table 2 shows the proportion of respondents who rated each goal as not important, somewhat important, important, and very important. A large share of respondents rated every goal as either important or very important, with a range from 26% saying that it was very important to "*reduce my morning stiffness*" and 94% saying it was very important to "*find treatments that are effective*". This pattern of results confirms that these goals reflect what people with RA believe is important, and variation in use of the response options captures the heterogeneity in patient-important goals, even among patients with the same disease. Only two participants rated every goal as 'Very Important'.

Importance ratings by sociodemographic groups

The importance ratings were examined by level of education, self-reported disease severity, and self-ratings of pain. For analysis by education level, we collapsed responses to two categories (less than college, college degree or more) but observed no major difference in importance ratings across all items. For both disease severity and pain, the largest differences by level were observed for importance ratings of morning stiffness, anxiety, and sadness. For the three levels of disease severity examined, most items showed a somewhat graded response, with respondents with higher levels of severity indicating higher item importance. No other items demonstrated a notable difference by these variables. (See online appendix Table 1.)

Table 4 shows percentage ratings of very important (top of scale) for all goals, by different sociodemographic characteristics. The importance of goals varied by respondent, reflecting individual differences in important goals. The percentage endorsing very important across all goals suggests that this set of domains matches with key goals for this sample of RA patients.

Write-in responses

Most of the survey respondents (36 of 47) wrote in at least one free-text goal or provided a comment. There were several goals mentioned by multiple respondents. Nearly half of respondents reported a goal of trying to find the right specialist, one who would listen to their symptoms and concerns. About half also discussed goals related to their emotional health, including the loneliness or isolation they had experienced, and wanted to find support from others with RA. Some described their goal of wanting to help others with RA. Some respondents wrote in goals that specified activities they wanted to be able to do, including lifting weights, practicing yoga, or running. One discussed the additional time required for daily tasks while living with RA. Others reiterated that dealing with their pain was their most important goal. Several respondents hypothesized that goals would vary with age or time with the disease.

Steering Committee assessment of feasibility

Steering Committee feasibility rating following data collection

Nine of the 11 members of the SC rated agreement with the same set of questions regarding the feasibility of the method, following review of the results of this study. All members rated agreement at 'somewhat agree' or completely agree for all items, with two exceptions. One member rated the following item as 'somewhat disagree': "*Goals collected from patients using an online format can be aggregated or combined into criteria useful for MCDA*". One member rated the following item as 'somewhat disagree': "*Existing RA networks could support recruitment of patients for goal collection*". This member shared that the reason for the disagreement with that item is that online networks are "*almost all white women under 50*".

Expert panel meeting feasibility results

The expert panel generally thought the method for collecting patient goals was feasible and could be adapted for incorporation into value assessment processes like MCDA. Panelists felt that the goals identified as important through this process could be used as criteria within MCDA, though additional work would be needed to weight these criteria for incorporation into MCDA. The goals in this process were specific to RA, but panelists felt that a broad library of goals that spanned different disease areas could be developed. As part of the MCDA process, patient advocates could select the subset of goals most important for a disease area based on the decision scenario and survey an online panel of patients to determine what matters most to these patients. The impact of different technologies on these goals would then be assessed, and then these impacts could be incorporated into MCDA. Further discussion of these feasibility findings can be found in our Practical Guide [29].

Patient advocates did note that many patient communities are not familiar with deliberative methods commonly used in value assessment, so additional care must be taken to explain to patients the objectives, specific actions patients are being asked to complete, and results and implications of the MCDA valuation exercise.

Conclusion

This study provides evidence of the feasibility of generating a set of patient-important outcomes from reviewing the literature and engaging with patient and expert stakeholders, and of validating outcomes through survey testing. Results of the pilot survey demonstrate that people with RA find a range of diverse outcomes important; every goal on the survey was rated as 'Very Important' by at least 25% of participants.

This work follows on a substantial history of outcomes assessment, including patient-engaged methods, in RA [19,20,22,23]. Unique to this method is the focus on care goals and the representation of goals that can be used in a GAS framework as well as for collection from large samples. Results support the importance of this goal set to these patients. SC and expert panel members generally agreed with statements about the feasibility of proceeding with this method, with two important caveats. Some members noted the current limitations of existing online patient networks in terms of gender, age, and race and ethnicity. Ensuring representative patient samples is likely to require effort beyond outreach to a single patient network.

To ensure the goal set continues to reflect the patient community goals accurately, continued open-ended goal elicitation is warranted. Periodic checks of goals using patient/clinician GAS discussions can further enhance the fidelity of the goal set to the patient community of interest.

Limitations

While we find this evidence adequate for support of this 'proof of concept' study, we note that our sample is not representative of people with RA as our respondents were more likely to be female, White, and non-Hispanic. Future research on patient goals should seek input from larger, representative samples for the target disease condition. Our respondents also reported a higher level of disease severity than may be typical in many clinic-based populations, with 76 percent saying that their arthritis was moderate or severe. Higher level of disease activity and more severe disease can be expected to translate to greater symptom burden, which could affect how respondents rated the importance of goals. While collecting goals relevant to patients across the spectrum of disease severity is of interest, the goals of patients at the moderate to severe level are likely to be most relevant for specific HTA decision-making as these are the patients who are likely in the need of the most expensive healthcare treatments. We compared across the disease severity continuum but recognize that the online panel of patients represent a different, and likely higher, severity range than the full range of patients seen for clinical care in practice.

Implications for future work

Further data collection would permit identification of goals that are RA-disease specific and those that are generic or applicable to other patient communities. Maintaining connection to disease-specific goals is a key contribution of this method, given the intended use as inputs for MCDA, but identifying which goals are relevant across diseases would be helpful for establishing a foundational or core set of goals from which different therapeutic areas can build.

We established the feasibility of collection of a set of goals important to people living with RA across the disease severity spectrum using a GAS-based survey instrument. The method for goals collection used partnerships with patient organizations for online-only contact and data collection of treatment goals, expanding the goals-based approach from an in-clinic discussion between patient and clinician. With wider "crowd-sourced" data collection using various platforms (e.g., web-based interface, mobile applications), broader input from patient communities is possible to include in HTA, including conducting HTA of specific interventions or treatment sequences. This goal inventory and process build on existing efforts to collect patient input on RA treatment and outcomes and highlight how goals can vary by patient and can be captured for use in HTA.

Capturing the patient perspective in HTA has expanded, but the patient-importance of outcomes assessed remains limited. There is a need to coalesce around a common definition of healthcare value that is rooted in outcomes that matter to patients [30]. This proof of concept for eliciting patient-important goals and their relative importance has applicability for deliberative methods used in HTA, including being considered for criteria and weighting in MCDA. Other efforts to incorporate broader value elements into value assessment have found that these elements can greatly influence assessment outcomes [31], suggesting that this work has the potential to influence value assessment of RA treatments. By expanding the numbers and representativeness of patients directly involved in the process of valuation and using a careful, accessible, and scalable method for goal collection, this approach offers a method to ensure the accurate representation of heterogenous patient perspectives, enhance patient-centricity, and improve the relevance of healthcare valuation to inform healthcare resource allocation by taking patient goals into account.

Summary points

- Methods for collecting patient input on valuation of healthcare interventions are in wide use, but the extent to which existing methods capture the range of outcomes important to patients has not been established.
- There is not yet a standard approach to identify and quantify patient-important outcomes for use in deliberative decision-making processes. This new method for engaging patients in healthcare valuation, using patient goals, is feasible for wider use.
- Deliberative methods of valuation can include outcomes based on goals collected directly from patients. Patient input through goal framing provides a way for patients to be actively involved in valuation methods.

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Ethical conduct of research

The authors state that they have obtained appropriate institutional review board approval for all human investigations. In addition, informed consent has been obtained from the participants involved.

Data sharing statement

The data that support the findings of this study are available from the corresponding author upon reasonable request.

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