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Development and Testing of a Tool to Assess Patient Preferences for Phase I Clinical Trial Participation

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Dear Editor

Shared-decision making entails that physicians assess patients' preferences. This assessment is crucial for advanced stage cancer patients(1). Yet, both system-related and communication barriers exist to this assessment. System-related barriers include lack of time and reimbursement practices that do not encourage patient-physician communication, even during critical periods of care such as deciding about early phase trial participation(1, 2). Assessing patient preferences has proven difficult as well. Barriers include the dual problem of physicians expecting patients to voice their preferences(3) and not readily recognizing indirect expressions of concern(4).

To date, interventions to improve patient understanding have had limited success (5, 6). Recognizing this challenge, we adopted a paradigm shift and focused on improving the physician's recommendation rather than on improving patients' understanding or facilitating communication. Numerous tools have been developed to improve patient-physician communication, but none focus on informing the physician's recommendation. We

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developed a tool for physicians to assess patients' preferences for participation in phase I clinical trials, the Patient Preference Assessment Tool (PPAT).

Methods

The development team was drawn from five IRB-approved sites: Emory School of Medicine, M.D. Anderson Cancer Center, Mayo Clinic, Penn State College of Medicine, and University of Alabama, Birmingham.

PPAT Development

An initial set of preferences was generated through qualitative interviews with 29 advanced stage cancer patients and a questionnaire completed by 36 oncologists. These items were reviewed and revised by a focus group comprised of 11 healthcare professionals involved in phase I oncology research and were cognitively tested by five phase I clinical trial participants.

The final set of 16 preferences and the stage at which they were identified are listed in Table I.

Testing of the PPAT

Concordance Testing—Forty physician-patient pairs (including 12 physicians) independently identified the patient's top four preferences.

Concordance was measured by the Surrogate Accuracy in Matching Patient Preferences Scale (SAMPPS)(7), which was evaluated for each physician participant by summing the total number of correct predictions of the patient's choices. The possible scores ranged from 0 to 4. When physicians evaluated multiple patients, the score was calculated as the total number of correct predictions for all patients divided by the number of patients.

Feasibility Testing—A second sample of 20 oncology and 9 amyotrophic lateral sclerosis patients completed the PPAT before meeting with their physicians (9 oncologists and 1 neurologist) to discuss phase I trial options. They provided their physician with the completed tool prior to the discussion. The phase I consent discussion was audio-recorded, transcribed and qualitatively coded to determine if at least one PPAT preference was discussed. The dual threshold for determining feasibility was 50% of patients identifying at least one preference on the PPAT, and 50% of phase I consent conversations including a discussion of at least one preference.

Item order effect testing—During feasibility testing, the order of preferences was randomly arranged for each patient to control for possible bias of choosing preferences that appeared earlier in the list. To test for an item-order-effect, we calculated the proportion of patients selecting each preference in concordance testing (items were not randomized) and in feasibility testing and then calculated the correlation coefficient between the selection rate of preferences in each test sample.

Patient and physician evaluation—The initial 36 physicians identified barriers and perceived need for the tool. During both concordance and feasibility testing, 69 patients and 10 physicians evaluated the tool for clarity, usefulness and for physicians, effect on recommendation using a 5 point Likert scale (1 strongly disagree, 5 strongly agree).

Results

Initial Physician Survey

The most frequently cited barriers to assessing patients' preferences during discussions of phase I clinical trials were: lack of time(72%); family members monopolize the conversation, so it is not possible to determine what is important to the patient(58%); patient reluctance to discuss their preferences(56%); lack of a convenient assessment tool(56%). Twenty-five(69%) physicians were interested in a tool or resource to inform them. Thirty-four(96%) stated they were aware/very aware of their patients' preferences and 19(53%) stated they were certain/very certain that their understanding of their patients' preferences was correct.

Concordance

The mean SAMPPS score was 1.66 correct matches out of 4 (41.5%, range 0-3.5).

Feasibility

All patients identified at least one preference, and 76% (22) gave their physician the completed tool. Physicians reviewed the tool in 21 of those 22 (95%) consent conversations and specifically discussed one or more of the preferences in 14(64%), meeting the threshold for feasibility.

Item order effect

There was a 93.8% agreement rate (15/16) on choice of preferences between feasibility and concordance. The correlation coefficient was 0.89 ($p<0.001$). Together, these data indicate that not randomizing the order of preferences does not significantly influence how patients choose preferences.

Patient and Physician Evaluation

Patients rated the tool as clear (4.0), easy to understand (4.2), easy to use (4.1) and useful with all patients offered a phase I trial (4.2). Physicians rated the tool clear (4.3); accurate (4.6); easy to use (4.2); providing helpful information (4.3), useful for all potential phase I patients (4.6), influencing their recommendation (4.0); but, not necessarily a time saver (3.4).

Discussion

The debate continues about the adequacy of physician communication regarding participation in phase I trials, with some contending that communication is inadequate(8), while others argue that participants understand well enough to provide valid informed consent(9). Attempts to improve communication have included the development of

numerous decision aids for patients and training activities and tools for physicians. In preference-sensitive decisions, like the choice to participate in a phase I clinical trial, it is important for physicians to assess patients' current preferences; yet, no such preference assessment tool existed prior to this study.

The need for PPAT is evident. The 36 physicians surveyed during tool development identified numerous barriers to assessing patients' preferences. Although over half were confident they knew their patients' preferences, and almost all stated they were aware of them, 12 physicians were unable to correctly identify their patients' preferences 59% of the time. Our concordance (41%) is comparable to or lower than levels reported in other concordance studies for treatment choices (48%–72%) (10) and would be considered inadequate based on the proposition by Sulmasy et al. who considered 70% agreement to be intermediate (7).

The intended use of the tool - the patient completing the PPAT before the clinical trial consent conversation, handing it to the physician who then discusses the top-ranked preferences - met the predefined threshold for feasibility of use in a phase I clinic. The major barriers were patients not providing the tool to their physicians (identified in 7 conversations), and physicians reviewing the tool but not discussing any of the patient's preferences (identified in 7 conversations).

The PPAT addresses an unmet need for a simple and easily implementable tool to assist physicians in assessing patient preferences regarding enrollment in early phase clinical trials. Further research is needed to test the PPAT's effectiveness in meeting the goal of communicating the patient's current preferences, and thereby informing and improving the physician's recommendation about trial participation.

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Key points

- Since improving communication and comprehension in the phase I setting have proven to be difficult, we adopted a paradigm shift to develop a tool to inform the physician's recommendation rather than focusing on the patient's understanding or on communication.
- The Patient Preference Assessment Tool was developed in four stages: patient interview, physician survey, focus group and cognitive testing.
- The need for the tool was evident in the physician questionnaire, in which many barriers to assessing patient preferences were identified.
- Concordance between physician and patient independent choice of preferences was low (41%), also establishing need.
- The tool proved feasible to use in busy phase I clinic settings.

Table I

Origin of Items	Final Order
Originated in Patient Interview and Physician Questionnaire	
1. Helping other cancer patients by participating in research	4
2. Getting the best possible care from expert doctors	6
3. Having access to the best cancer treatment	8
4. Having access to new treatments, even if they are not yet proven and are part of research	9
5. Avoiding as many procedures as possible (like biopsies and blood draws)	10
6. Avoiding as many trial or research side-effects as possible	13
Originated during Focus Group	
7. Attending an important event (like a wedding or a graduation) in the future	14
8. Making my family happy by continuing to fight my cancer	15
9. Avoiding spending lots of hours in the clinic	3
Modified during Focus Group (original wording in parentheses)	
10. Feeling as good as possible every day given my condition (feeling good now, normal, back to old self)	1
11. Fighting my cancer as much as possible (being cured)	2
12. Making it easier on my family so they don't have to drive me or come with me to clinic appointments (not being a burden to my family)	7
Originated during Cognitive Testing	
13. Taking part in a research study, if it is my only option	5
14. Following the recommendations of my doctor	12
"Spending more time at home than in the clinic" originated during interview and survey but was divided into two preferences during cognitive testing	
15. Avoiding missing fun events at home because I am in the clinic	11
16. Avoiding extra travel time and time away from home	16