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Mixed-methods approach to develop an agreed concept on patient relevance: study protocol for the 'PRO patients study'

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ABSTRACT

Introduction With respect to patient-centred care and shared decision-making, measuring care effects based on outcomes relevant to patients is becoming increasingly important. Recently, a scoping review of the international literature revealed a wide range of supposedly patient-relevant outcomes and found that there is neither a sound definition of patient relevance nor a consistent set of outcomes relevant to patients. To close this gap, this study aims to develop an agreed concept on patient relevance including a set of outcomes relevant to patients irrespective of diseases, which grades outcomes according to their importance.

Methods and analysis This prospective mixed-methods study will integrate the perspectives of patients across diseases, healthcare professionals and researchers. The consensus process will consist of four phases. Based on the results of the recent scoping review, a patient survey will be conducted first, followed by a multiprofessional group discussion. Finally, a two-round online Delphi approach based on data from the previous phases will be applied to agree on a concept.

Ethics and dissemination Ethics approval for the study was granted on 26 August 2020 by the Ethics Commission of Witten/Herdecke University (reference number: 156/2020). In the long run, the implementation of an agreed concept on patient relevance will help improve the comparability of study results regarding the patient benefit and thereby strengthen the role of patients in the decision-making process. Also, the experiences regarding grading outcomes according to importance will help to develop a method on how to individualise clinical trial outcomes according to each patient's individual specifics and priorities in order to more adequately represent the patient perspective in clinical research.

Trial registration number Core Outcome Measures in Effectiveness Trials Initiative (registration number: 1685).

Strengths and limitations of this study

- This study is based on the results of a scoping review of the international literature, which showed that there is neither a sound definition of patient relevance nor a consistent set of outcomes relevant to patients.
- An agreed concept on patient relevance including a generic outcome set will help adequately operationalise patient-centred care and increase the comparability of study results.
- The multiprofessional approach applied in this study will ensure that the concept considers aspects that are relevant to patients and is feasible in the scientific context as well.
- Due to the online approach used, it cannot be excluded that particular patient groups might be under-represented in the consensus process.
- Also, the consented concept might not be internationally valid as the study is conducted among German-speaking participants only.

INTRODUCTION

Patient-centred care means that patients, their values, preferences as well as their individual life and health goals are at the heart of care processes and that patients are involved in care decisions.[1] To achieve this, physician–patient communication is fundamental. Comparing different communication strategies, a study among German general practice patients indicated that most patients prefer shared decision-making, which is also considered the academic gold standard.[2] Being actively involved in care decisions implies that patients are adequately informed about existing care options and their potential effects, understand these options and are given the opportunity to explore what is most relevant to them in order to make a choice based on their personal preferences.[3] Against this background, studies need to examine the effects of care based on parameters that matter to patients, thereby enabling them to make an informed decision. However, recent systematic reviews conclude that patient-relevant outcomes are under-represented in clinical trials.[4–6] The authors assume different outcomes as relevant to patients, that is, survival, quality of life and functionality,[4–6] but do not explain why these outcomes are supposedly relevant.

A recent scoping review of the international literature aimed to assess which outcomes might be particularly relevant to patients.[7] Based on 44 studies published in the past 20 years, the review revealed neither a consistent terminology or sound definition nor a consistent set of outcomes relevant to patients across diseases.[7] Another recent review about the use of patient-reported outcomes in core outcome sets found that different outcome sets cover the same domains, but recommend different instruments.[8] Even though patient-reported outcomes and patient-relevant outcomes might not be necessarily the same, the findings of both reviews underline the need for harmonising the selection and measurement of outcomes as inconsistencies in their choice, and measurement limits the comparability of study results regarding the patient benefit. Due to this, a consensus on a consistent concept is needed to adequately operationalise patient-centred care.

Aim

We designed the 'PRO patients study' to achieve a consensus regarding a concept on patient relevance based on the recent literature that considers both the patients' perspective and usability in research. In detail, the finally achieved concept will (1) define which terminology is most suitable to describe patient-relevant outcomes, (2) provide insight which criteria are appropriate to characterise outcomes relevant to patients in the sense of a definition, (3) prioritise which parameters mostly represent generic patient relevance independently of diseases.

In addition, our long-term aim is to derive an empirically based methodological framework on how to select and weigh parameters relevant to patients when designing trials and in the process of shared decision-making. However, this manuscript focuses on the development of the consented concept.

Scope

The scope of an agreed concept on patient relevance and a framework facilitating the selection of outcomes is intended for standard use in (clinical) studies in order to improve the comparability of study results regarding the patient benefit, and thereby empowering patients to make informed choices in the context of shared decision-making.

Health condition

Generic, not disease specific.

Population

Adults ≥18 years of age.

METHOD

This study protocol is reported in accordance with the Core Outcome Set-STAndardised Protocol Items Statement.[9]

Study design

The study will be conducted as a prospective mixed-methods study that uses quantitative and qualitative methodologies. In order to achieve a consensus, a two-round online Delphi approach based on data from the previous scoping review,[7] a patient survey and an interactive group discussion will be carried out (figure 1).

Study participants

The study will consider the perspectives of patients, medical and therapeutic professionals, and researchers. This multiprofessional approach will ensure that the agreed concept fits the patient's perspective and is feasible for scientific purposes at the same time.

All participants are required to be at least 18 years old, to be sufficiently proficient in German and to provide written consent. For patients, no other inclusion and exclusion criteria are defined so that patients irrespective of age, gender and diseases will be included. Persons of the other two target groups are eligible for participation if they practise a medical or therapeutic profession or are researchers in the fields of medicine, public health, nursing or another related scientific field.

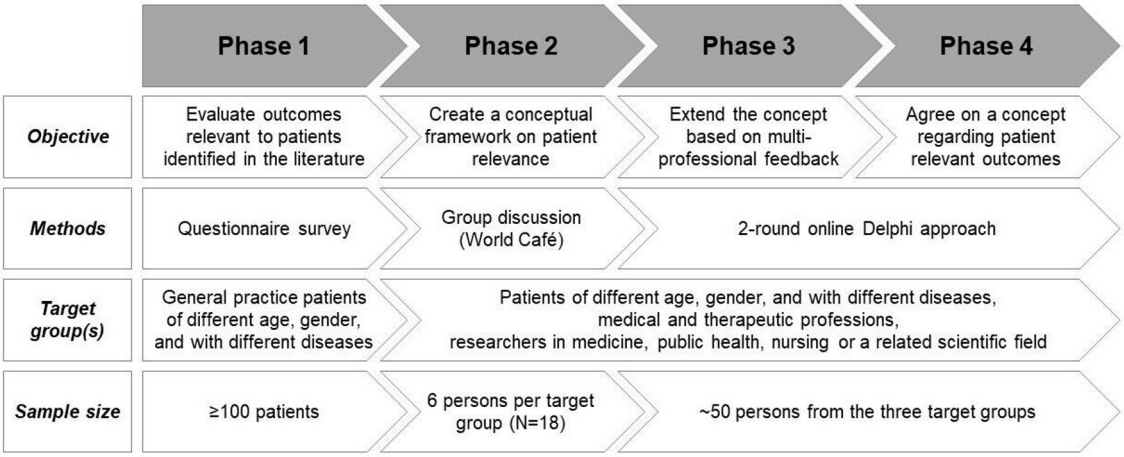

Figure 1 Flow chart of the study conduct.

Consensus process

Aiming to build a consensus regarding a concept on patient relevance, we designed a stepwise approach including four study phases. The results of each phase will provide the basis for the next one (figure 1):

Phase 1: evaluation of patient-relevant outcomes identified in the literature

Objective and method

In fall 2020, a cross-sectional questionnaire survey was conducted to assess patients' views and beliefs concerning supposedly patient-relevant outcomes identified in former studies. In detail, the survey aimed to gain insight into how patients evaluate and weigh different outcome dimensions in order to contribute to an empirically based methodological concept of patient relevance in medical decision-making in the future.

Participants and recruitment

The survey was conducted among general practice patients. Participants were recruited via teaching and research practices of the Chairs of General Practice, Witten/Herdecke University. All practices attending the chair's 2020 fall network meeting were invited to support the study and trained on how to conduct the survey in their practices. During a 2-week period in October 2020, one responsible healthcare assistant per practice asked every fifth patient aged ≥18 years coming in for a scheduled appointment to participate. In order to obtain meaningful results, the minimum sample size was defined as 100.

Data collection

Healthcare assistants documented the age, gender, chronic disease (yes/no) and occupational status of participants unwilling to participate. Patients willing to participate self-administered a three-page anonymous questionnaire. This questionnaire was developed by one of the authors (CK) based on the results of a previous scoping review on outcomes particularly relevant to patients.[7] The relevance of outcomes identified in the scoping review was assessed on a 5-point Likert scale ranging from not relevant to highly relevant. Additionally, an included free-text field allowed to add outcomes that are missing in the list of relevant outcomes identified so far. In order to ensure comprehensibility and completeness, the questionnaire was pretested and discussed by a group of nine researchers from Witten/Herdecke University. After revision, it was discussed with 19 general practitioners and healthcare assistants who attended the 2020 fall network meeting. The original German-language questionnaire and an English translation are provided as online supplemental files 1 and 2.

Data management and analysis

All data were entered manually into an access-restricted database located at the Chair of General Practice II and Patient-Centredness in Primary Care, Witten/Herdecke University. For analysis, descriptive statistics were performed using IBM SPSS Statistics for Windows, Version 26 (IBM Corp). New parameters mentioned as free text were added to the list of outcomes identified from the literature. Finally, a non-responder analysis was conducted to check whether participants and non-participants differed regarding demographic characteristics.

Synthesis

In preparation of phase 2, the parameters assessed in the cross-sectional survey will be sorted in accordance to their importance as defined by the data. The additional mentioned parameters will be listed without prioritisation.

Phase 2: creation of a conceptual framework on patient relevance

Objective and method

Based on the results of the scoping review[7] and the results of phase 1, a multiprofessional group discussion will be conducted to draft a first version of a framework for a concept on patient relevance. The discussion will be held in 2021 applying the World Café method.[10]

Participants and recruitment

A total of 18 persons (n=6 persons per target group) will be recruited for the group discussion. This sample size is reasonable due to the method applied.[10] Participants will be recruited using an invitation letter which will be disseminated via email and postal service using several distributors, for example, the mailing list of the teaching and research practices of the Chairs of General Practice, Witten/Herdecke University, mailing lists of research projects recently conducted at the chairs, a patient advisory council established at the chairs, and interprofessional linkages to other institutes of Witten/Herdecke and nearby universities. Persons interested in supporting the study will be asked to contact the study team.

Data collection

During a personal meeting, participants will discuss the following in small groups with a changing group composition at three round tables representing one topic each:

1. Terminology: What terminology from the literature is most suitable to describe outcomes relevant to patients? What German terms might be appropriate equivalents?
2. Criteria: What criteria characterise outcomes relevant to patients? Which of these criteria are mandatory for a definition?
3. Outcomes: Are there any relevant outcomes missing on the list of outcomes identified from the literature and the patient survey? Which of the outcomes mentioned are unclear and/or even dispensable? Which of the remaining outcomes are—in terms of gradation—more or less important than others and why? How do patients weigh these different outcomes against each other?

At the first table, participants will use post-it notes to mark their preferred terminology. All other discussion results will be documented immediately at each table using facilitation cards and/or flip charts.

Data management and analysis

The results of the discussions will be digitalised. For the analysis, the post-it notes assigned to the different terms will be counted, criteria mentioned as mandatory will be incorporated into one or more working definitions on patient-relevant outcomes, and all outcomes defined as suitable to represent patient relevance will be graded from most to least important.

Synthesis

In preparation of phase 3, the terms preferred in phase 2 will be selected and working definitions on patient relevance will be incorporated. Additionally, results regarding the importance of parameters derived from phase 1 and phase 2 will be compared and transferred to a common list, which will be sorted by importance.

Phase 3: extension of the concept based on multiprofessional feedback

Objective and method

The third phase aims to extend and further develop the framework created in phase 2. To consider various opinions and different perspectives, a Delphi process will be initiated.[11 12]

Participants and recruitment

As the Delphi process will be conducted electronically, the invitation to participate including the link to the survey will be sent to representatives via email. In addition to the distributors used for phase 2, the link will also be disseminated via mailing lists of patient organisations, professional societies and professional associations. Persons who are not willing or not able to participate electronically will have the opportunity to request a paper-based version of the Delphi questionnaire. According to practical advice on how to conduct Delphi approaches, the minimum sample size is defined as 50.[11 13]

Data collection

The various opinions will be assessed electronically using LimeSurvey (Hamburg, Germany: LimeSurvey). Participants will be asked to evaluate the framework of the concept with regard to the following aspects:

1. Terminology: individual assessment of which of the terms preferred in the previous phase is most suitable for describing outcomes relevant to patients.
2. Criteria: individual statement on which working definition is most appropriate and what revisions are required.
3. Outcomes: amendment of outcomes missing in the list and adjustment of the gradation of outcomes in terms of importance.

In addition, some key characteristics will be assessed to describe the study sample and to make sure that participants belong to one of the target groups. Besides age and gender, healthcare professionals and researchers will be asked to provide information about their profession, while patients will be asked to provide information about chronic conditions in a free-text field.

Data management and analysis

Before data collection, data will be exported from LimeSurvey to IBM SPSS for Windows, Version 26. Statements regarding the working definition will additionally be transferred to MAXQDA (Berlin, Germany: VERBI Software). As the analyses will consider only the opinions of participants representing one of the target groups, descriptive statistics on key characteristics will be conducted first. After that, simple frequency calculations will be performed with regard to the preferred terminology and the working definition. Supported by MAXQDA, proposed modifications for the preferred definition will be categorised inductively and then incorporated stepwise. In case of conflicting modification requests, those mentioned more frequently will be considered. Based on the rankings, mean values will be calculated for each outcome including those added by participants.

Synthesis

In preparation of phase 4, the terminology evaluated as most suitable and one final definition will be derived from the data of this phase. In addition, the outcomes will be summarised to a preliminary set grading outcomes from most to least important according to the ratings of this phase.

Phase 4: agreement on a concept regarding patient relevance

Objective and method

The last phase aims to reach a consensus regarding a concept on patient relevance. Based on the results of phase 3, a second Delphi survey will be conducted in order to consider various perspectives.[11 12]

Participants and recruitment

Similar to the recruitment procedures in phase 3, representatives of the three target groups will be invited by email. The dissemination of the invitation and the survey link will use the same distributors as in phase 2 and phase 3. The minimum sample size is again defined as 50.[11 13]

Data collection

The Delphi survey will be conducted electronically using LimeSurvey. Persons unwilling or not able to participate online can request the Delphi questionnaire as paper-based version. Participants will be asked to confirm the three aspects of the concept:

1. Terminology: agreement to the most frequently preferred terminology identified as suitable in the first Delphi survey or reasoned refusal providing a more suitable alternative.
2. Criteria: agreement to the developed definition on patient-relevant outcomes or reasoned refusal providing information on required revisions.
3. Outcomes: agreement to the outcome set including the gradation of outcomes according to importance or reasoned refusal providing information on required revisions.

As participation in the previous Delphi survey is not a prerequisite for participating in the second round,

participants will additionally provide information on some key characteristics. Like in the previous round, all participants will be asked to indicate their age and their gender. Additionally, for healthcare professionals' and researchers' information regarding their profession will be requested, while patients will be asked to provide information on chronic conditions.

Data management and analysis

The data collected via LimeSurvey will be exported to IBM SPSS for Windows, Version 26; free-text items resulting from reasoned refusals will be transferred to MAXQDA. As in the previous phase, descriptive statistics on key characteristics will be conducted first. Simple frequency calculation will then be performed for each of the three aspects of the concept (terminology, criteria, outcomes) to determine the proportion of participants that agrees to the concept. According to rules for the development of guidelines of the German Association of the Scientific Medical Professional Societies, consensus will be assumed when ≥75% of the participants agree to the concept.[13] In case of an agreement <75%, the reasoned refusals for the respective aspect will be analysed and incorporated into the concept. After that, the Delphi survey will be repeated as described until a consensus is reached for all three aspects of the concept.

Synthesis

In order to harmonise the operationalisation of the outcomes identified as particularly relevant to patients, these outcomes will be compared with domains covered in common instruments, that is, used to measure generic quality of life.[14] Additionally, the experiences and results on grading of outcomes according to their importance that will be made during the different phases of the consensus process will provide useful hints for developing a methodological framework on how to select outcomes according to patients' preferences in the long term.

Patient and public involvement

Patients and the public will be actively involved in each step of the study conduct as their opinions and perspectives are at the heart of this approach.

Ethics

The 'PRO patients study' obtained ethical approval from the Ethics Commission of Witten/Herdecke University (reference number: 156/2020, date of approval: 26 August 2020). Participants of all phases will receive written information and provide informed consent.

DISSEMINATION

The agreed concept will be presented at research conferences and published in peer-reviewed journals. Once disseminated in the scientific context, the implementation of an agreed concept and consistent understanding on patient relevance will have the potential to improve the comparability of study results regarding the patient benefit. As this will enable patients to make informed decisions based on outcomes that really matter to them, a stronger consideration of patient-relevant outcomes as intended by an agreed concept will also enhance the patients' role within the decision-making process. However, the consented concept might not be internationally valid due to the restriction to German-speaking participants only. Assuming that the topic of patient relevance is of international interest, an international adoption of the concept needs to be considered in the future. Also, it cannot be excluded that particular groups of patients (eg, at high age or with severe diseases) might be under-represented in this study so that the list of prioritised outcomes deriving from the consensus process might not be generically valid. Due to this, the need for developing supplemental generic outcome sets depending on—for example—age or gender will be evaluated during the study conduct.

Additionally, aspects of adapting the concept will be addressed when developing a supplemental methodological framework on how to individually prioritise clinical trial outcomes from the patient's perspective.[15 16] For this purpose, it will be important to understand how patients ultimately make decisions, how they weigh between different parameters and what factors shift their priorities. Besides the experiences and results in terms of grading of outcomes according to their importance emerging from the consensus process, preference exploration methods will be needed in order to understand the processes underlying patients' decision-making. In the long term, the framework should allow to distinguish between parameters that are of general importance irrespective of underlying diseases and parameters that need to be adapted individually or according to specific characteristics. In addition, the framework should also provide guidance on how to adapt these parameters to different scenarios.

In the future, a consented concept on patient relevance including a framework on selecting parameters relevant to patients will not only help to conduct more patient-centred clinical research, which adequately addresses different patients' characteristics and specifics, but also enhance the process of shared decision-making between patients and physicians.

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