Development of an innovative methodology to define patient-designed quality of life: a new version of a wellknown concept in healthcare

Barbara Bartolini, Serena Bertoldi, Laura Benedan, Carlotta Galeone, Paolo Mariani, Francesca Sofia, Mariangela Zenga

1 Introduction

Quality of life (QoL) is a concept embracing several aspects and functionalities of people's lives. Some of the areas affected are health, relationships, socializing, leisure (The International Society for Quality of Life Research). The achievement of a good QoL is recognized as an essential aim of health assistance, regardless of the pathology and the administered therapy (M. Asadi-Lari et al., 2004). QoL is therefore a pivotal parameter used by clinicians to evaluate how treatments and therapies influence patients' functionality and emotional state, aiming to ameliorate interventions and their outcomes.

QoL is determined by indices assessed by administering questionnaires that can be either generic or disease-specific (D. L. Patrick & Deyo, R. A., 1989; R. Rabin & de Charro, F., 2001; J. E. Ware, Jr. et al., 2016). Currently, the majority of the QoL questionnaires are designed with the main contribution of clinicians and, therefore, include items that are centered on the disease rather than on its multifaceted impact on people's life. These tools are useful for clinicians in determining the best clinical approach, but may fail to truly grasp the patients' perspective, needs, aspirations, perceptions and emotional state, resulting in a major drawback that sets medical care on clinical parameters alone. A proper tool defining the patient's perception of the pathology is missing.

To bridge this existing gap, the definition of a bottom-up patient-designed QoL index could provide a new, patient-centric, unbiased tool to evaluate the patients' perception of their own well-being. Here we describe the development of an innovative methodology to define patient-designed QoL, based predominantly on patients' contribution.

2 Working group and methodology

To define a patient-centric QoL tool, we used a consensus technique aiming to favor the expression of the major players involved in dealing with the pathology.

The Delphi method is currently widely used in academic research, industry, social sciences and healthcare to reach consensus (R. Boulkedid et al., 2011; I. R. Diamond et al., 2014; M. K. Murphy et al., 1998; Robinson N Trevelyan EG, 2015). The main goal is to collect different opinions to be evaluated by the panel, with the aim of reaching pluralistic evaluations of an issue. In the Delphi panel, the participants are either technical or non-technical experts (i.e., patient representatives) reporting their own point of view (G. Mazziotta Marbach, C. & Rizzi, A., 1991).

In our model, patients and healthcare professionals constitute the working group to build the settings and assertions of the questionnaire.

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FUP Best Practice in Scholarly Publishing (DOI 10.36253/fup_best_practice)

Barbara Bartolini, Serena Bertoldi, Laura Benedan, Carlotta Galeone, Paolo Mariani, Francesca Sofia, Mariangela Zenga, *Development of an innovative methodology to define patient-designed quality of life: a new version of a wellknown concept in healthcare*, pp. 155-159, © 2021 Author(s), CC BY 4.0 International, DOI 10.36253/978-88-5518-461-8.30, in Bruno Bertaccini, Luigi Fabbris, Alessandra Petrucci (edited by), *ASA 2021 Statistics and Information Systems for Policy Evaluation. Book of short papers of the on-site conference*, © 2021 Author(s), content CC BY 4.0 International, metadata CC0 1.0 Universal, published by Firenze University Press (www.fupress.com), ISSN 2704-5846 (online), ISBN 978-88-5518-461-8 (PDF), DOI 10.36253/978-88-5518-461-8

The items of the QoL questionnaire were defined during focus groups involving a panel of patients, two clinicians, one statistician and one facilitator.

Patients are the active players that identify the settings and the main items. According to the European Patients' Academy on Therapeutic Innovation (EUPATI) (K. Warner et al., 2018) definition, patients involved may be:

- Individual Patients, i.e., "persons with experience of living with a disease. Their main role is to contribute with their subjective experience";
- Patient Organization Representatives, i.e., "persons who are mandated to represent and express the collective views of a patient organization on a specific issue or disease area".

Clinicians offer the technical knowledge of the pathology, supporting the precise description of the items. Facilitators act as a guide for discussion among the parties, with the important role of harmonizing the contribution of all the participants to avoid a decisional bias due to the influential opinion of the clinicians on patients' decision.

The Pseudo-Delphi we propose is an iterative process with subsequent steps aiming to identify a shared solution. It is a flexible method, useful when the identification of the statistical model to be applied is uncertain and when the gist of the discussed problem is not completely known.

The Pseudo-Delphi steps are as follows:

- Problem definition and identification of the expert panel
- First round to collect the basic aspects of the research
- Definition of the first questionnaire with open questions
- Administration of the questionnaire to each expert, who respond anonymously
- Second round to discuss questionnaire's answers and definition of the Likert scale and the closed questions
- Definition of the second questionnaire, open questions and closed questions with the respective agreement scale
- Administration of the second questionnaire to each expert
- Third round, with discussion on the questionnaire's answers, identification of the scale of importance with the evaluation of the possible consequences of each decision and the feasibility of each defined option
- Moderated feedback, result aggregation and sharing with the experts. In this round, the new questionnaires resulting from the previous opinions are also shared
- Repetition of the questionnaire, if necessary, to reach the agreement.

Working anonymously allows to avoid the prevalence of a charismatic individual over the others, which can freely express their opinions without any social pressure.

Moreover, the feedback control allows the experts to be provided with all the information needed to reach the final agreement. With the Pseudo-Delphi method all the participants can analyze and re-consider a variety of aspects included in the questionnaire. This method entails a great effort for the methodologists in estimating and summarizing facts, quantitative data and subjective variables. This approach is of value in the analysis of real-world data, especially in QoL evaluation (S. Pietersma et al., 2014).

The workflow starts from the patients' evaluation of a list of settings identified by a literature search. Patients independently provide social, economic, and organizational information related to their pathology and their relationship with the healthcare system. This allows the identification of the settings of the questionnaire.

The setting evaluation is carried out by every single patient anonymously, to avoid any kind of psychological subjection from the healthcare professional's opinion. At the end of this process, the group meets in a roundtable session to openly discuss about the settings emerged

and to rank them according to the perceived order of importance. This step is crucial to skim the settings and find those to be included in the questionnaire, to make it usable in daily practice.

Within each setting, a series of assertions are generated individually and anonymously by each patient. Following similar steps, a set of assertions is identified and included in the questionnaire. Every assertion is then associated to a four-point Likert scale. On the basis of the score, a synthetic patient-centric QoL index is then defined (Figure 1).

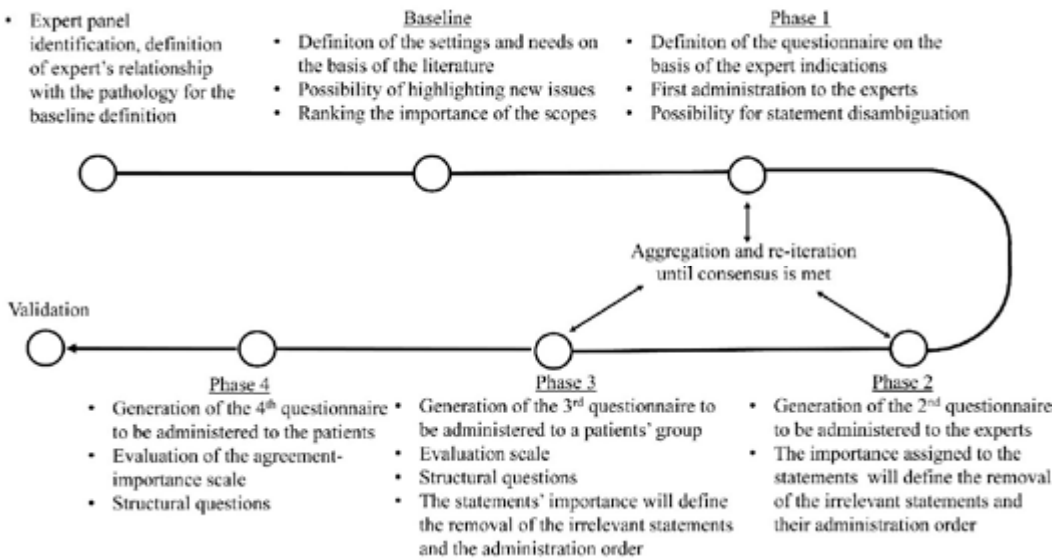

Figure 1. Flow chart showing the generation of the QoL questionnaire.

The final version of the questionnaire contains the scales for agreement and importance measures. They aim to link the agreement to one item with the importance for the patient in her/his life and they are built on the basis of the Customer Satisfaction Techniques. After the identification of the different settings (e.g., physical, emotional, social, functional and economic) the level of agreement and the level of importance of the statements within each setting are rated on a 4-points Likert scale (response categories: not at all; a little; quite a bit; very much) by the participants. The methodology allows the production of a composite index for "uneasiness", which will be then compared to the internal control –provided by the evaluation of each own QoL on a one to ten scale. The composite index is defined as follows.

Let x_{ijs} (i=1,…, n; j=1,…, k_s; s=1,...S) be the agreement of the i-*th* respondent on the j-*th* statement for the *s-th* setting. The categories on the agreement part for a statement are treated as numeric variable where "not at all" =0.001; "a little" =0.33; "quite a bit" =0.67; "very much" =1. In this case we transform the variable at 4 categories in 3 categories where the distance between each successive item category is equivalent and equal to 0.33. The agreement on *not at all* is treated as the lack respect to the statement. Moreover let w_{ijs} (i=1,…, n; j=1,…, k_s; s=1,...S) be the importance given by the *i-th* respondent to the *j-th* statement for the *s-th* setting. In this case the categories for the importance for a statement are "not at all" =0.25; "a little" =0.5; "quite a bit" =0.75; "very much" =1.

An indicator on the *j-th* statement for the *s-th* setting given by the *i-th* respondent is given by

$$u_{ijs} = x_{ijs} \cdot w_{ijs} \quad \text{(I)}$$

The u_{ijs} takes values in $[0.00025;\ 1]$. For each value of u_{ijs}, it is possible to find the correct combination of x_{ijs} and w_{ijs}.

The questionnaire includes a section with structural questions exploring the current state of the disease, personal evaluation about the psychological state and the type of assistance received, geographical and demographic characteristics. This information completes the patient profile and can be used for further analysis and stratification.

For the *i-th* respondent, it is possible to create an uneasiness score for the *s-th* setting as

$$U_{is} = \sum_{j=1}^{k_s} x_{ijs} \cdot w_{ijs} \quad \text{(II)}$$

In (II) the statements running in the opposite direction for the *s-th* setting are reversed for the score. The U_{is} could take values in $[k_s \cdot 0.00025;\ k_s]$.

For the *i-th* respondent, the total composite index is given by:

$$TU_i = \sum_{s=1}^{S} U_{is} \quad \text{(III)}$$

that takes values in $[0.00025 \sum_{s=1}^{S} k_s\ ;\ \sum_{s=1}^{S} k_s]$. The linear transformation of (III) in

$$TU_i^{10} = (10-1) \cdot \frac{TU_i - \sum_{s=1}^{S} k_s \cdot 0.00025}{\sum_{s=1}^{S} k_s (1-0.00025)} + 1 \quad \text{(IV)}$$

allows that $TU_i^{10} \in [1; 10]$. The TU_i^{10} represents the synthetic patient-centric QoL index. It is possible to compare TU_i^{10} respect to the *i-th* respondent to the score of the quality of life of the *i-th* respondent QoL_i.

3 Conclusions

With this pilot study we suggest a methodology to set up a questionnaire for the identification of a synthetic index that allows the evaluation of the overall QoL of patients, regardless of the clinical data. The index enhances the patients' awareness of their subjective experience with the disease and enables them to better present their situation to the clinicians. This methodology can be considered in light of the idea of improving patient engagement as highlighted by the EUPATI PARADIGM project (P. Spindler & Lima, B. S., 2018). This methodology needs to be further validated through administration to patients suffering from different pathologies, and compared to the methodologies already available from international sources. An index directly generated by the patients can provide a descriptive model helpful not only to patients, but also to clinicians and third parties, that can be further integrated with clinical details to obtain an overall view of the course of treatment for each patient.

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