Quality of life in Health Care: focus on patients

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1. Introduction

Health-Related Quality of Life (HRQoL) is a well-known concept collecting aspects of overall quality of life related to physical or mental health (Centers for Disease Control and Prevention, 2000; Selim et al., 2009). HRQoL can be defined as "an individual's or group's perceived physical and mental health over time" (Centers for Disease Control and Prevention, 2000). On the individual level, HRQoL includes physical and mental health perceptions and their correlates—including health risks and conditions, functional status, social support, and socioeconomic status. On the community level, HRQoL includes community-level resources, conditions, policies, and practices that influence a population's health perceptions and functional status.

The achievement of a good HRQoL is recognised as an essential aim of health assistance, regardless of the pathology and the administered therapy (Asadi-Lari *et al.,* 2004). HRQoL is 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. HRQoL is determined by indices assessed by administering questionnaires that can be either generic or disease-specific (Patrick & Deyo, 1989; Rabin & de Charro, 2001; Ware, *et al.,* 2016). These questionnaires have become an important component of public health surveillance and are generally considered valid indicators of unmet needs and intervention outcomes. Currently, the majority of the HRQoL questionnaires are designed with the main contribution of clinicians and, therefore, include items that are focused 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. The patient's self-assessed health status may be a more powerful predictor than many objective health measures. Unfortunately, a proper tool defining HRQoL from the patient's perspective is missing.

The present paper aims to propose a methodology to define a bottom-up patient-designed HRQoL questionnaire.

2. Methodology

The demand to create an HRQoL questionnaire stemmed from the request of a rare disease patients' association. The project's first step consisted of examining the existing scientific literature to understand what was already known and what instruments are used nationally and internationally. After that, a pseudo-Delphi study was carried out.

The Delphi method, a flexible and iterative process, helps collect experts' opinions in health research (Trevelyan & Robinson, 2015). It was chosen to ensure patient participation and foster the convergence of opinions through the iterative structure, i.e. the collection of experts' opinions through multiple iterations, to allow the participants to review their evaluations at least once after a comparison with the response of the group (Pacinelli, 2008). However, in a traditional Delphi study,

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participants are polled individually, generally via self-administered questionnaires over two (or more) rounds, and no face-to-face meeting is scheduled (Boulkedid *et al.,* 2011). In the present study, the connotation "Pseudo-Delphi" should be applied because complete anonymity of participants could not be granted as all the group discussions were organised via "face-to-face" virtual meetings. Hence, all the recruited experts could participate and contribute to the group discussion. Nonetheless, a private (and completely anonymous) evaluation of all the questionnaire's items was granted after each meeting so that every person could critically analyse, re-consider, make suggestions, express comments and provide individual responses without any social pressure or compliance effect that may conversely arise during the group discussions. For more details on the overall study procedure, see Bartolini *et al.,* 2021, and Benedan *et al.,* 2021.

The multidisciplinary panel of experts comprised a Delphi master, six patients or patients' caregivers, two clinicians recognised as international key opinion leaders for their disease-specific expertise, a psychologist, and a statistician.

A first group meeting was organised to discuss every step of the project, the main topics to cover, and the primary aim to be achieved. Successively, the patients and clinicians were asked to provide a list of spontaneously generated items to describe different areas of the patient's HRQoL. The results were presented in the first roundtable session to discuss all the implications of daily living with the disease openly. On this occasion, great care was taken to ensure a comprehensive and accurate understanding of the experts' points of view.

Seven domains were identified and endorsed by the group (see Table 1 for a description of each domain).

Table 1: Questionnaire domains

Domain	Description
Physical	It includes the most relevant aspects in terms of health and physical well-being.
Functioning and autonomy	It refers to self-sufficiency and includes statements about the ability to perform common routine actions.
Psycho-emotional	It refers to psycho-emotional well-being, including emotions, thoughts and feelings.
Family	It refers to the relationships with parents, siblings, or other family members such as partners and children if it applies.
Relational	It includes statements about relationships and frequent interactions with people who do not belong to the family (e.g., friends, classmates, colleagues, strangers on the street, etc.).
Work and economic	It includes statements referring to the work context and the financial implications of the disease.
Medical care and assistance	It refers to disease-related healthcare, including medical and nursing assistance.

After defining the domains and examining the main topics, a first questionnaire (Q1) was created. Respondents were required to rank them within each domain according to their importance.

Therefore, for every domain, the rating may range from a minimum of 0 to a maximum equal to the number of items in that domain (Physical = 14; Functioning and autonomy = 15; Psycho-emotional = 13; Family = 12; Relational = 9; Work and economic = 11, Medical care and assistance = 6). They were also required to comment on the clarity and specificity of each item, to write any potential new item, and to report any missing information that might have been included. The main aim of this phase was to exclude any irrelevant items to shorten the entire set of questions and have a more manageable questionnaire. Each expert responded anonymously to the questionnaire and returned it to be discussed in the second Delphi round. All the answers were carefully examined, and a ranking was created for every item within each domain according to the degree of importance indicated by the participants. The results of this analysis were discussed in the group, and further questionnaire refinement was made. Some items were changed or rephrased for greater clarity; others were merged or removed because of their lesser importance.

A new questionnaire (Q2) was defined, considering all suggestions from the group meeting. The previously identified core domains remained unchanged, but some new items were suggested and inserted. At this stage, each participant was asked to rate both the degree of agreement and the degree of importance of each item on a four-point Likert scale ("Not at all", "A little", "Quite a lot", "Very much"). This step is necessary to remove some irrelevant statements and evaluate the order in which the items are presented. In addition to the abovementioned seven domains, some specific questions were inserted about the type of the rare disease diagnosed and some socio-demographic information. Finally, an overall Quality of Life satisfaction question was asked.

The results of this phase were presented to the group to define the questionnaire structure further and prepare the new version (Q3) that each participant anonymously filled in.

Figure 1 illustrates the flow of the project from the beginning to the validation phase of the final questionnaire. For the purposes of the present study, we will focus on the Delphi rounds involving the development and refinement of the questionnaire from Q1 to Q3. The following section will provide a thorough description of how the questionnaires changed through the iterative process.

Figure 1: Flow chart of the project
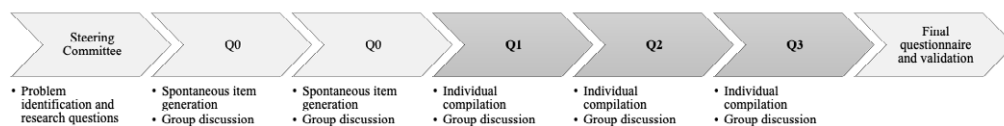

3. Results and Discussion

The first questionnaire (Q1) contained 80 items grouped into the seven previously identified core domains. This first version was carefully reviewed, and several changes were suggested by the panellists. After an in-depth examination of all the items, through private compilation and group discussion, many adjustments were made. From the original list of statements, 54 (68%) items remained unchanged, 19 (24%) were rephrased (e.g. "I might have children" was changed to "I can have children"), and 7 (9%) were eliminated - some were merged into one for the sake of synthesis: for instance, "I feel frustrated", "I feel helpless", and "I feel demoralised" were merged into a single one ("I feel helpless, demoralised/or frustrated/or").

It should be noted that the changes concerned not only the questionnaire as a whole but also the individual domains. In fact, two items were moved from one domain to another: for instance, "I feel I'm self-reliant" was moved from the functioning and autonomy domain to the psycho-emotional domain. In addition, 13 new statements were inserted in the following version of the questionnaire. Considering all these changes, Q2 was composed of 86 items. The order in which the items were presented changed according to the importance of each statement within the domain so that the

more important items were the first, as established in the previous round. The same private examination and group discussion process aimed at reviewing the items was applied to Q2. Again, several changes were suggested, examined and, whenever approved by the group, introduced in the new version of the questionnaire. Forty-six (54%) items remained unchanged, while 39 (45%) were rephrased to be more easily understandable and clear. One of these items was also moved from the functioning and autonomy domain to the psycho-emotional domain ("I can have children", which was also rewritten as "I worry about being able to have children"). Only one sentence was removed, and no new items were suggested.

The new version of the questionnaire (Q3) comprised 85 items. As in the previous rounds, each participant anonymously filled in the questionnaire and then the results were discussed in the group. Figure 2 shows the comparison between Q1 and Q2, and between Q3 and Q2. It can be noticed that a process of progressive refinement and definition was carried out from one iteration to the next, affecting all the domains.

Figure 2: Comparison between Q2 vs. Q1 (n=80) and Q3 vs Q2.
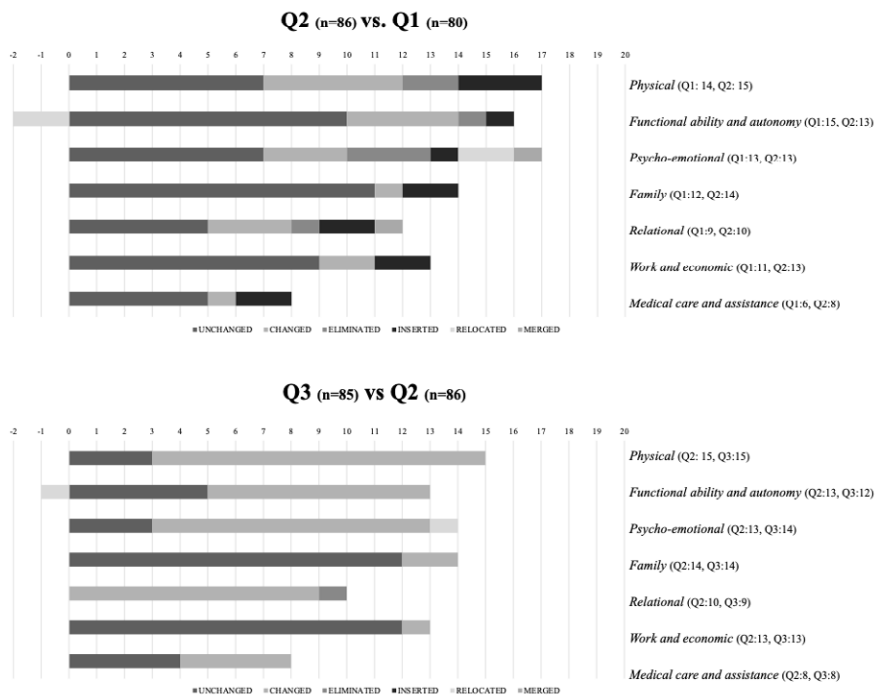
Source: elaboration of research data, collected from June to August 2021

4. Conclusions

The present study is part of a more extensive research project to develop a valid and reliable questionnaire to assess the HRQoL of patients affected by a rare disease. In order to grasp the point of view and the patient's subjective experience beyond clinical symptoms, a pseudo-Delphi study was carried out. The questionnaire's items were progressively created, elaborated and refined through the iterations, round after round. The changes made in the wording of the items from the first version of the questionnaire to the third one were described. The result is an HRQoL

questionnaire that goes beyond the physical symptoms and the clinical evolution of the disease, encompassing functional autonomy, psycho-emotional well-being, social relations inside and outside the family context, the working field and several aspects of the medical care and assistance. The methodology proposed here may help improve patient engagement in line with the EUPATI project (Warner *et al.*, 2018) and allow the analysis of real-world data related to HRQoL, especially when the number of participants is reduced.

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