Issues in the measurement of quality of life in hemophilia

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It is widely claimed that "you can't manage what you don't measure". Likely this quote is derived from statements made by Lord Kelvin to the Institution of Civil Engineers. In his 1883 address, he claimed:

…when you cannot measure it, when you cannot express it in numbers, your knowledge is of a meager and unsatisfactory kind… [1]

A number of tools have been developed to accurately assess our hemophilia patients' progress and response to changing treatments. These include radiographic scores (like the Pettersson (X-ray) score and the compatible magnetic resonance imaging score), musculoskeletal assessment tools (like the Hemophilia Joint Health Scale), functional assessment questionnaires (like the Haemo-philia Activities List) or observational tools (like the Functional Independence Scale for Haemophilia), as well as summary measures of health - which are often called quality of life (QoL) or health-related quality of life (HRQL) tools[2-15].

These tools have given clinicians and researchers powerful new ways to describe their patients' illnesses, but there are a number of considerations that should be addressed in future work. This commentary will address some of these issues.

The subjective nature of quality of life

Current questionnaires used in hemophilia do not appear to adequately address the personal and subjective nature of QoL.

Quality of life is a term that has been used over the last many decades, originally to describe material affluence. More recently, it has been used in the social sciences and medical literature, synonymously with the terms 'life satisfaction', 'self-esteem', 'well-being', 'happiness', 'health', 'the value and meaning of life', and 'functional status'[16].

In order to bring some standardization to the field, the World Health Organization (WHO) developed their definition of QoL as an:

...individuals' perceptions of their position in life in the context of the culture and value systems in which they live and in relation to their goals, expectations, standards and concerns[17].

This definition emphasizes the subjective and personal nature of QoL.

Our current hemophilia QoL questionnaires reflect the questions that are important to groups of persons with hemophilia (PWH), and the values assigned to the questions likewise reflect those held by groups of PWH. However, in choosing therapies, and managing patients, we are more often motivated by maximizing our individual patient's well-being.

A future challenge will be to incorporate personal and subjective valuation into measured QoL.

The inadequately defined concept of health-related quality of life

Researchers, recognizing that the medical system may not be able to address all the concerns that inform QoL (e.g. financial, social, spiritual, etc.), developed a new term, HRQL, in the early 1980s[18,19]. This has often been defined as the impact of health on QoL.

However, our current hemophilia QoL measures operationalize HRQL more as a standardized and scored history taking. That is, HRQL most often reflects patient-reported health states. Again, the questions and their associated values are determined by the developers, often taking into account the values of groups of PWH, rather than the individual being questioned.

If we accept the WHO definition of health as a complete biopsychosocial construct, rather than just the absence of disease, then it is unclear whether there are aspects of QoL that are outside the boundaries of health[20].

Future work might focus on defining what exactly is meant by HRQL in hemophilia, to better address the needs of patients, clinicians and researchers.

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Formative versus reflective measures

The standard psychometric approach to the development of a QoL questionnaire assumes that there is an underlying construct (concept) of QoL that we all carry around in our heads, and that measuring it is a matter of tapping into the questions that 'reflect' that underlying construct (so-called reflective measures). If this is correct, then all valid ways of measuring QoL should give the same results.

However, we (and many others) have found that using different questionnaires and methods to measure QoL gives us very different results[21-23]. This suggests that we do not carry around a concept of our QoL in our heads; rather we construct our concept of QoL based on what and how we are asked (philosophical constructivism).

It seems that we cannot assume that there is an underlying single construct of QoL, rather that we form our idea of QoL based on the questions we are being asked. If this is true, QoL questionnaires are formative measures[24]. An implication of trying to measure a formative construct is that a single, overall, number value may not be appropriate when expressing QoL. Rather the values of the individual components that form the particular concept of QoL being measured (e.g. questions, or domains of questions) should be reported separately. This approach might be explored in the future as we move ahead with refining QoL tool development.

Measurement issues that need further consideration

When looking at change in QoL, researchers often use percent change (relative change) to describe it. However, when using non-ratio scales (i.e. there is no true meaning to a zero score), relative change should not be used. For example, the change in temperature from 10C to 20C is exactly the same as the change from 50ºF to 68ºF; in the former case the relative change would be a 100% increase, whereas in the latter it would be a 36% increase. Future research looking at change should consider this.

Most of our QoL questionnaires consist of a number of questions answered on an ordinal categorical scale, however researchers often treat each question as yielding a continuous number. For example, the Canadian Haemophilia Outcomes - Kids Life Assessment Tool (CHO-KLAT) QoL questionnaire has 35 questions, each with 5 response options (never, seldom, sometimes, often, always)[25]. Because of the way it is scored (by averaging), a patient who always has problems with one important item in their life, will get the same QoL score as another patient who seldom has problems in 4 items. However, it is unlikely that these 2 situations are equivalent. Rather, the CHO-KLAT has 2.91 x 1024 unique combinations of possible answers, each with a unique value. In the future researchers should consider multi-attribute utility methods to derive more reasonable scores from our ordinal questionnaires, rather than treating them as continuous numerical measures.

Clearly the field of research in hemophilia has benefitted tremendously by the excellent work done by researchers, developing accurate measurements of disease and QoL. However, there continues to be much work to be done as we refine and improve our measurement techniques. This paper has listed just a few of the considerations the next generation of re-searchers might consider as the field moves forward.

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