Pediatric Liver Transplant Quality of Life (PeLTQL©) Questionnaire

An instrument to assess quality of life in pediatric liver transplant recipients

Manual and Interpretation Guide
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Preface

This manual describes the Pediatric Liver Transplant Quality of Life (PeLTQL©) questionnaire. PeLTQL© measures the health related quality of life in pediatric liver transplant recipients from both the patient’s and parent’s perspective. It was developed and validated to measure quality of life of children who have received a liver transplant with the intention of identifying and improving areas of poor quality of life.

The PeLTQL© Manual & Interpretation Guide contains information on the background, keypoints about the development and validation of the PeLTQL© questionnaire, as well as information on how to administer and score the questionnaire.

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1. Introduction

1.1 Pediatric Liver Transplantation
Liver transplantation (LT) is a necessary and life-saving treatment for a number of pediatric conditions such as biliary atresia, hepatoblastoma, metabolic disorders, and acute liver failure. One-year and five-year patient survival rates are well over 90% and 85% respectively at experienced centers and long-term survival following LT is now the rule rather than the exception for an ever growing population of children. Due to excellent survival rates, the ensuing course of treatment for these children post-LT presents multiple stressors, such as lingering medical and psychological fears and uncertainties of potential immediate and long-term adverse medical complications which may impact on their lives.

1.2 Outcomes that matter
The World Health Organization (WHO) defines health as “a state of complete physical, mental and social well-being, not merely the absence of disease or infirmity”. The evolution of the broader approach to health has been accompanied by changes in the way health and disease are measured and evaluated. The concept of patient-centered care has developed over the years and aims to incorporate patient perspectives and values into medical decisions. Patient-centered care has led to the development of instruments that measure a wide range of health-related phenomena, including physical and psychosocial function, patient satisfaction and quality of life.

1.3 Health Related Quality of Life
While it is difficult to objectively measure complete wellbeing, health related quality of life (HRQOL) comprises the health related aspects of life that contribute to the wellness and meaning of life, including one’s perception of wellbeing, and one’s perceived ability to fulfill certain life roles. HRQOL is a multidimensional construct that incorporates health-related factors (such as physical well-being, social and emotional functioning, and mental and psychological status) and by non-health-related elements (such as family, friends, and life circumstances). HRQOL measures are believed to provide a more complete picture of an individual and are complementary to traditional biomedical measures and functional assessments.

1.4 Importance of Measuring HRQOL in Pediatric Liver Transplantation
Early reports of “good” health-related quality of life (HRQOL) after LT in children relied on the presence or absence of conventional outcomes such as growth, number of hospitalizations, and disease symptoms, without direct measurements of social, education and emotional impairments experienced as a result of undergoing and living with a LT. Although LT is a life-saving procedure, it is not curative and recipients continue to live with a chronic condition that requires immunosuppressive medications and ongoing medical monitoring. As most pediatric liver recipients now live long lives it is imperative to move beyond biomedical outcomes and assess
quality of life. Assessing HRQOL permits us to hear the voice and perspective of the patient\(^6\) and has been found to predict, often better than physiologic measures, future health status, mortality, and resource utilization\(^7\).

**1.5 Challenges in Measuring HRQOL in Pediatric Liver Transplantation**

Perspective can affect measurement of HRQOL, as medical providers may have different perspectives than a parent proxy or the patient themselves. HRQOL assessment in pediatrics is challenging because it must take into account the varying developmental level and age of each child\(^8,9\). Direct assessment of a child’s HRQOL may be limited by age, medical condition and developmental ability for comprehension, and hence may require a parent/caregiver to act as a proxy for the child\(^10\). Moreover, quality of life is a dynamic phenomenon, evolving and changing as priorities and health status change. Furthermore, LT introduces unique concerns to a patient’s life, and thus the ability to evaluate small but meaningful changes needs to be evaluated using a disease-specific tool.

**1.6 Overview of established measures**

There are currently no established disease-specific HRQOL measures for use in the pediatric liver transplant population. To date, the published literature is primarily limited to studies utilizing generic (although validated) HRQOL tools, including a recent multi-centre study comparing pediatric liver transplant recipients to other chronic disease groups. Both groups were found to have impairments in all domains\(^11\).
2. Development of PeLTQL©
The PeLTQL© was developed and validated using published well-established tool development methodology. The following section outlines the initial development and validation of the PeLTQL©.

2.1 Development of PeLTQL©
The first phase of development involved generating items relevant to HRQOL in children after liver transplantation through healthcare providers’ focus groups, parent and patient interviews, as well as a thorough review of the published English literature. A list of items generated was compiled and subsequently reduced based on frequency and pertinence using an expert-based approach. A pilot questionnaire was then developed and administered to pediatric liver transplant recipients and their parents followed by pediatric liver transplant programs in Canada, the UK, Australia and the United States. Participants rated items based on importance and frequency of concern and items were selected to be included in the final PeLTQL© based on a high impact score derived from the sum of the frequency and importance for each item.

2.2. Validation of PeLTQL©
The final 26-item PeLTQL© was then assessed for validity after development. Pediatric liver transplant recipients and their parents were administered the PeLTQL© in order to assess reliability, validity and sensibility. Reliability was assessed using test-retest reliability and internal consistency. Test-retest reliability yielded an intraclass correlation coefficient of 0.85 and internal consistency yielded a Cronbach’s $\alpha$ of 0.86. Construct validity was established using a priori hypotheses using already established quality of life instruments measured concurrently with the PeLTQL©. Furthermore, sensibility was established to be excellent: only 0.5% of questions were left blank and most participants could complete the questionnaire in less than 5 minutes with no assistance.
3. Properties of the PeLTQL©
The PeLTQL© is a 26-item self-administered questionnaire with both a patient and a parent version. The measure has a Flesch-Kincaid grade 5 reading level.

3.1 Domains
The PeLTQL© questionnaire assesses the following domains:
- Future Health (9 questions: #5, 8, 9, 11, 13, 14, 17, 19, 20)
- Coping & Adjustment (8 questions: #1, 3, 4, 6, 10, 15, 24, 25)
- Social-Emotional (9 questions: #2, 7, 12, 16, 18, 21, 22, 23, 26)

3.2 Scoring
The direction of scoring for the 5-point Likert scale used in the questionnaire is reversed, so that a higher score indicates better quality of life. The final score is scaled so that possible final scores range from 0 to 100. Please see “Scoring Sheet” for more information.

4. Does the PeLTQL© measure HRQOL?
The PeLTQL© has been demonstrated to have excellent reliability and validity as a measure of HRQOL in pediatric liver transplant recipients. A strength of this questionnaire is that it assesses the patient’s HRQOL from both the patient and parent proxy perspective.
5. Using the PeLTQL©

5.1 Administration

What does the PeLTQL© measure?
The PeLTQL© questionnaire measures both the patient’s and parent’s perspective about the pediatric liver transplant recipient’s health-related quality of life with regard to emotional and psychological functioning, social functioning, physical health, current treatments and interventions, and future health status. It is both a useful proxy and self-report measure of health related quality of life of children who have received a liver transplant.

Who is the PeLTQL© designed for?
The PeLTQL© was designed to measure both patient and parent perceptions of HRQOL in pediatric liver transplant recipients. The PeLTQL© has been validated for use in patients between 8 and 17 years old who are at least one year post-transplant AND their parents.

How is the PeLTQL© administered?
The PeLTQL© is intended to be self-administered. Patients complete the PeLTQL© Patient Version and parents complete the PeLTQL© Parent Version. Patients and parents are encouraged to complete the questionnaire at a clinical visit in case they have any questions or concerns, however they can complete it at home and return it by mail. Patients and parents should complete the questionnaire separately so as not to influence each other’s responses. Patients who are unable to read the questionnaire by themselves can have the questions read to them but should mark their own answers. Questions should not be interpreted for the patients.

How long does the PeLTQL© take to complete?
The questionnaire typically takes less than 5 minutes to complete.

5.1.1 Administration Instructions

Patient Version

- Explain to the patient that he/she will be completing a questionnaire asking about his/her life because he/she had a liver transplant. Even if he/she had a liver transplant as an infant, it is likely that there are still things that must be done as a result of the transplant that have an impact.
- Explain that the 26 questions on the PeLTQL© came from children around the world who have had a liver transplant. Not every question asked will have to do with him/her. Just because an item was brought up by one child does not mean that it is something that affects all children. Tell them that everyone is different, so what is important to someone else may not be important for them and that is perfectly ok!
- Mention that he/she should feel free to ask you questions at any time while completing the questionnaires.
  - For children who have reading difficulties, it is acceptable for you to read each question to him/her. But please read off one copy, and have the patient fill in the answers on their own copy; please be careful to not interpret an answer for the patient.
• After the PeLTQL© is completed, feel free to ask him/her if there were any questions or points that he/she would like to talk about. If the participant appears upset, we have provided “Dealing with Distressing Issues” in Appendix A

**Parent Version**

• Explain that the parent will be completing a questionnaire asking about their child’s liver transplant
• Explain that it is very important that the parent answer the questions from their child’s perspective and NOT their own perspective.

### 5.2 Scoring

Raw item scores should be reverse scored using the *Scoring Sheet* that comes with the questionnaire. The reverse scored items are summed to create a total score that is then scaled by dividing by 26 to obtain the total final score. Domain scores can also be calculated using the *Scoring Sheet*.

**Missing values**

A reliable PeLTQL© score can be obtained if there are 3 or less missing item scores. If the patient or the parent left more than 3 items blank then the final score may not be reliable and should be interpreted with caution. For more information, consult Appendix B “Dealing with Missing Values”.

### 5.3 Interpretation of Scores

Good clinical judgment of should be used when interpreting scores. There are currently no established norms for PeLTQL© scores, but they will become available as the tool begins to be used clinically. However, a higher final score indicates a better quality of life, with possible scores ranging from 0 to 100.

The PeLTQL© is meant to be used as a starting point or tool in the continuous process of evaluating quality of life, rather than an isolated measure of quality of life.
Dealing with Distressing Issues

Some Suggestions for the Clinician

In the unlikely event that a patient/parent finds a question/statement in the questionnaire upsetting, it is important that the appropriate support be available.

Possible approach for dealing with distress:

- Prior to administering the PeLTQL®, it is important to emphasize that not every issue is relevant to each individual patient. Let the patient/parent know that the concerns were brought up by other children who have had a liver transplant, and their parents. However, each child’s circumstances may have been very different. Just because it is on the questionnaire, does not mean that it applies to everyone and is something they should be worried about.

- After the patient/parent completes the PeLTQL®, ask them if there were any questions that they wanted to talk about. If they mention a specific issue that they found upsetting, talk to them about it. Reaffirm that just because something is on the PeLTQL®, it does not mean it is something that they need to be worried about.

- Tell them that 150 people were interviewed about every possible thing that worries or concerns them after having a liver transplant. 150 is a lot of people! What is true for one person does not have to be true for someone else… just like some people might be afraid of the dark, someone else might be afraid of spiders, and someone else might be afraid of both of these things.

- It is important to emphasize that everyone is different.

- If the patient/parent is still upset, ask them if they would like to talk to a clinical team member. If they do, proceed to get in contact with the appropriate person.
Dealing with Missing Values

General issues to consider:

There is some literature which has examined methods of dealing with missing values in quality of life scales\textsuperscript{13,14}. One of the first issues is to try and determine whether it is missing for reasons unrelated to the patient’s quality of life. Examples of this may include an administrative error in which the participant did not receive all pages of the questionnaire, or in a longitudinal, repeated measures study, where a questionnaire was not completed because patient had moved away, or it had been completed at an incorrect time (too early or late for a particular end point). In these instances the data could be considered to be “missing at random\textsuperscript{13}.”

If data is missing for non-random reasons, for example, patient death or progression of disease, then censoring their information may result in bias of the results. There are a number of ways which have been used to deal with missing data in the context of multi-item assessments such as quality of life measures. Deleting the entire case when there are missing items is possible, but this can result in a loss of power and may lead to bias if the subjects with poorer quality of life are preferentially skipping or failing to complete certain items\textsuperscript{14}. If a significant number of items are missing (>50%) this may need to be dealt with on a case-by-case basis. If there are less significant numbers of missing items then developing a method for imputing missing items for an individual is desirable, rather than losing the entire case\textsuperscript{14}. The methods can be divided into those which use the subject’s own data versus those in which the missing data is imputed from other subject’s data. Using the subject’s own data, one can take the mean of all non-missing items for the entire measure or a specific domain. Using data from other subjects, it is possible to take the mean of a particular item for those subjects who did respond. More sophisticated methods have also been proposed\textsuperscript{15}.

PeLTQL\textsuperscript{©}:

- If >50% of response are missing, the total summed score or domain scores cannot be reliably obtained.
- If there are ≤3 missing responses, calculate the mean overall score and domain scores by dividing the sum of the items over the number of items answered.
References


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